

# AMICUS THERAPEUTICS INC

## FORM 10-K (Annual Report)

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UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION  
Washington, D.C. 20549

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FORM 10-K

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ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE  
SECURITIES EXCHANGE ACT OF 1934

For the fiscal year ended December 31, 2010

Commission File Number 001-33497

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**Amicus Therapeutics, Inc.**

(Exact name of registrant as specified in its charter)

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Delaware

(State or other jurisdiction of  
incorporation or organization)

71-0869350

(IRS Employer  
Identification No.)

6 Cedar Brook Drive, Cranbury, NJ 08512  
(Address of principal executive offices)  
Telephone: (609) 662-2000

Securities registered pursuant to Section 12(b) of the Act:

Title of each class  
Common Stock, par value \$0.01 per share

Name of each exchange on which registered  
The NASDAQ Stock Market LLC

Securities registered pursuant to Section 12(g) of the Act: None

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Indicate by check mark if the registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act.  
Yes  No

Indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or Section 15(d) of the Act.  
Yes  No

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes  No

Indicate by check mark whether the registrant has submitted electronically and posted on its corporate Web site, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). Yes  No

Indicate by check mark if disclosure of delinquent filers pursuant to Item 405 of Regulation S-K is not contained herein, and will not be contained, to the best of the registrant's knowledge, in definitive proxy or information statements incorporated by reference in Part III of this Form 10-K or any amendment to this Form 10-K.

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, non-accelerated filer or a smaller reporting company. See definition of "large accelerated filer," "accelerated filer" and "smaller reporting company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer

Accelerated filer

Non-accelerated filer

Smaller Reporting Company

Indicate by check mark if the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes  No

The aggregate market value of the 7,242,725 shares of voting common equity held by non-affiliates of the registrant, computed by reference to the closing price as reported on the NASDAQ, as of the last business day of the registrant's most recently completed second fiscal quarter (June 30, 2010) was approximately \$16,223,704. Shares of voting and non-voting stock held by executive officers, directors and holders of more than 10% of the outstanding stock have been excluded from this calculation because such persons or institutions may be deemed affiliates. This determination of affiliate status is not a conclusive determination for other purposes.

As of February 18, 2011, there were 34,508,932 shares of common stock outstanding.

**DOCUMENTS INCORPORATED BY REFERENCE:** Portions of the Proxy Statement for the registrant's 2011 Annual Meeting of Stockholders which is to be filed subsequent to the date hereof are incorporated by reference into Part III of this Annual Report on Form 10-K.

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## SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This annual report on Form 10-K contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts, included in this annual report on Form 10-K regarding our strategy, future operations, future financial position, future revenues, projected costs, prospects, plans and objectives of management are forward-looking statements. The words “anticipate,” “believe,” “estimate,” “expect,” “intend,” “may,” “plan,” “predict,” “project,” “will,” “would” and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.

The forward-looking statements in this annual report on Form 10-K include, among other things, statements about:

- the progress and results of our clinical trials of our drug candidates, including Amigal;
- our ability to achieve development and commercialization milestone payments and sales royalties under our collaboration with GlaxoSmithKline PLC;
- the scope, progress, results and costs of preclinical development, laboratory testing and clinical trials for our product candidates including those testing the use of pharmacological chaperones co-administered with ERT and for the treatment of diseases of neurodegeneration;
- the costs, timing and outcome of regulatory review of our product candidates;
- the number and development requirements of other product candidates that we pursue;
- the costs of commercialization activities, including product marketing, sales and distribution;
- the emergence of competing technologies and other adverse market developments;
- the costs of preparing, filing and prosecuting patent applications and maintaining, enforcing and defending intellectual property related claims;
- the extent to which we acquire or invest in businesses, products and technologies; and
- our ability to establish collaborations and obtain milestone, royalty or other payments from any such collaborators.

We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make. We have included important factors in the cautionary statements included in this annual report on Form 10-K, particularly in Part I, Item 1A “Risk Factors” that we believe could cause actual results or events to differ materially from the forward-looking statements that we make. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, joint ventures, collaborations or investments we may make.

You should read this annual report on Form 10-K and the documents that we incorporate by reference in this annual report on Form 10-K completely and with the understanding that our actual future results may be materially different from what we expect. We do not assume any obligation to update any forward-looking statements.

**PART I**

**Item 1. BUSINESS.**

**Overview**

We are a biopharmaceutical company focused on the discovery, development and commercialization of orally-administered, small molecule drugs known as pharmacological chaperones for the treatment of rare diseases. Pharmacological chaperones are a novel, first-in-class approach to treating a broad range of diseases including lysosomal storage disorders and diseases of neurodegeneration. We believe that our pharmacological chaperone technology, our advanced product pipeline, especially our lead product candidate, Amigal, and our strategic collaboration with GlaxoSmithKline uniquely position us as a leader in the development of treatments for rare diseases.

Our current areas of focus include the following:

- Phase 3 development of our lead product candidate, Amigal for Fabry disease;
- preclinical and clinical development of pharmacological chaperones co-administered with enzyme replacement therapy; and
- preclinical evaluation of the use of pharmacological chaperones for diseases of neurodegeneration.

Fabry and other lysosomal storage disorders such as Gaucher and Pompe diseases are among certain human diseases that are caused by mutations in specific genes that, in many cases, lead to the production of proteins with reduced stability. Proteins with such mutations may not fold into their correct three-dimensional shape and are generally referred to as misfolded or unstable proteins. Misfolded or unstable proteins are often recognized by cells as having defects and, as a result, may be eliminated prior to reaching their intended location in the cell. The reduced biological activity of these proteins leads to impaired cellular function and ultimately to disease.

Our novel approach to the treatment of human genetic diseases consists of using pharmacological chaperones that selectively bind to the target protein, increasing the stability of the protein and helping it fold into the correct three-dimensional shape. This allows proper trafficking of the protein within the cell, thereby increasing protein activity, improving cellular function and potentially reducing cell stress. We have also demonstrated in preclinical studies that pharmacological chaperones can further stabilize normal, or “wild-type” proteins. This stabilization could lead to a higher percentage of the target proteins folding correctly and more stably, which can increase cellular levels of that target protein and improve cellular function, making chaperones potentially applicable to a wide range of diseases.

Our lead product candidate, Amigal (migalastat hydrochloride) for Fabry disease, is in Phase 3 development. We are developing and commercializing Amigal with an affiliate of GlaxoSmithKline PLC (GSK) pursuant to a License and Collaboration Agreement entered into in October 2010. Our partnership with GSK allows us to utilize GSK’s significant expertise in clinical, regulatory, commercial and manufacturing matters in the development in Amigal. In addition, the cost-sharing arrangements and potential milestone and royalty payments under the License and Collaboration Agreement provide us with financial strength and allow us to continue the development of Amigal while also advancing our other programs. We also believe this collaboration is important in validating our status as a leader in the development of treatments for rare diseases given the increasing focus placed on the rare disease field.

Our Phase 3 clinical development program for the use of Amigal as monotherapy in Fabry disease includes two clinical trials: Study 011 and Study 012. We have enrolled a majority of the planned 60 patients for Study 011, and intend to commence an additional Phase 3 study (Study 012) in the first half of 2011. We plan to use the data from Study 011 to support the filing of a New Drug Application, or NDA, for marketing approval in the United States and the data from Study 012 to support the filing of an application for marketing authorization in Europe. We believe Amigal may have advantages over the current standard of treatment for Fabry disease, enzyme replacement therapy, or ERT. While ERT compensates for the reduced level of activity of specific enzymes through regular infusions of recombinant forms of the enzyme, our approach uses orally-administered small molecule pharmacological chaperones to improve the function of the enzyme that is made by the patient’s own body. We believe this approach to treating these diseases could provide benefits to patients through better bio-distribution and ease of use.

In addition to potential benefits pharmacological chaperones may provide as a monotherapy, we also believe the use of pharmacological chaperones co-administered with ERT may address certain key limitations of ERT. The use of pharmacological chaperones co-administered with ERT may significantly enhance the safety and efficacy of ERT by, among other effects, prolonging the half-life of infused enzymes in the circulation, increasing uptake of the infused enzymes into cells and tissues, and increasing enzyme activity and substrate reduction in target tissues compared to that observed with ERT alone. We are evaluating the use of pharmacological chaperones co-administered with ERT in preclinical studies in Gaucher and Pompe disease and in a Phase 2 clinical study being conducted with GSK evaluating the use of Amigal co-administered with ERT.



Although Fabry, Gaucher and Pompe are relatively rare diseases, they represent substantial commercial markets due to the severity of the symptoms and the chronic nature of the diseases. The publicly-reported worldwide net product sales for the six currently approved therapeutics to treat Fabry, Gaucher and Pompe disease were approximately \$1.9 billion in 2010.

While our initial clinical efforts have focused on the use of pharmacological chaperones to treat lysosomal storage diseases, we believe that our technology may be applicable to the treatment of certain diseases of neurodegeneration. Our lead preclinical program in this area is focused on Parkinson's disease, where we expect to complete late-stage preclinical proof of concept studies, including IND-enabling activities, for our pharmacological chaperone molecule AT3375 during 2011. Our second preclinical program in this area is focused on Alzheimer's disease. Our preclinical work in both Parkinson's and Alzheimer's disease is presently focused on genetically-defined subpopulations of Parkinson's and Alzheimer's patients and leverages our expertise and knowledge in the rare disease field.

**Our Pharmacological Chaperone Technology**

In the human body, proteins are involved in almost every aspect of cellular function. Proteins are linear strings of amino acids that fold and twist into specific three-dimensional shapes in order to function properly. Certain human diseases result from mutations that cause changes in the amino acid sequence of a protein, and these changes often reduce protein stability and may prevent them from folding properly. The majority of genetic mutations that lead to the production of less stable or misfolded proteins are called missense mutations. These mutations result in the substitution of a single amino acid for another in the protein. Because of this type of error, missense mutations often result in proteins that have a reduced level of biological activity.

Proteins generally fold in a specific region of the cell known as the endoplasmic reticulum (ER). The cell has quality control mechanisms that ensure that proteins are folded into their correct three-dimensional shape before they can move from the ER to the appropriate destination in the cell, a process generally referred to as protein trafficking. Misfolded proteins are often eliminated by the quality control mechanisms after initially being retained in the ER. In certain instances, misfolded or unstable proteins can accumulate in the ER before being eliminated.

The retention of misfolded proteins in the ER interrupts their proper trafficking, and the resulting reduced biological activity can lead to impaired cellular function and ultimately to disease. In addition, the accumulation of misfolded proteins in the ER may lead to various types of stress on cells, which may also contribute to cellular dysfunction and disease.

We use pharmacological chaperones to increase the stability of target proteins and help them fold into their correct three-dimensional shape. This allows proper trafficking of the protein within the cell, thereby increasing protein activity, improving cellular function and potentially reducing cell stress.

**Potential Advantages of Pharmacological Chaperones for the Treatment of Lysosomal Storage Disorders**

To date, we have mainly focused on developing pharmacological chaperones for the treatment of lysosomal storage disorders. Lysosomal storage disorders are a type of metabolic disorder characterized by mutations in lysosomal enzymes, which are specialized proteins that break down cellular substrates in a part of the cell called the lysosome. We believe that pharmacological chaperone therapy may have advantages relative to the current therapeutic standard of care for these disorders, ERT, which involves regular infusions of recombinant human enzyme to compensate for the deficient lysosomal enzyme. The following table compares some features of enzyme replacement therapy to pharmacological chaperone therapy.

<u>Product Characteristic</u>	<u>Enzyme Replacement Therapy</u>	<u>Pharmacological Chaperone Therapy</u>
<i>Biodistribution</i>	Variable tissue distribution	Broad tissue distribution, including brain
<i>Ease of Use</i>	Weekly or every other week intravenous infusion	Oral administration
<i>Manufacturing</i>	Recombinant protein manufacturing	Chemical synthesis

An additional therapeutic approach to the treatment of certain lysosomal storage disorders is substrate reduction therapy. We believe our pharmacological chaperone therapies may have advantages relative to substrate reduction therapy as well. Like pharmacological chaperone therapies, substrate reduction therapy uses orally-administered small molecules; however, the underlying mechanism of action is very different. Substrate reduction therapies are designed to prevent the production of the substrate that accumulates in disease by inhibiting an enzyme required to make the substrate in cells, which is not the same enzyme that is deficient in the disease. Importantly, if synthesis of the substrate is inhibited it cannot perform its normal biological functions. Additionally, the enzyme that is inhibited is needed to make other molecules that are used in other biological processes. As a result, inhibiting this enzyme may have adverse effects that are difficult to predict. By contrast, our pharmacological chaperones are designed to bind directly to the enzyme deficient in the disease, increasing its stability and helping it fold into its correct three-dimensional shape. This in turn enables proper trafficking to the lysosome where the enzyme can directly decrease substrate accumulation. To date, one substrate reduction therapy product has received regulatory approval in the U.S. and the European Union (EU) for the treatment of one lysosomal storage disorder. Zavesca<sup>®</sup>, a substrate reduction therapy product commercialized by Actelion, Ltd., is approved for the treatment of Gaucher disease in the U.S., the EU and other countries. Genzyme Corporation is currently developing a substrate reduction therapy product which is in Phase 3 development for the treatment of Gaucher Disease.

### **Amigal for Fabry Disease**

#### *Overview*

Our most advanced product candidate, Amigal, is an orally-administered, small molecule pharmacological chaperone for the treatment of Fabry disease. In October, 2010, we entered into a License and Collaboration Agreement with GSK to develop and commercialize Amigal. Under the terms of the License and Collaboration Agreement, GSK received an exclusive worldwide license to develop, manufacture and commercialize Amigal. In consideration of the license grant, we received an upfront license payment of \$30 million and are eligible to receive further payments of approximately \$170 million upon the successful achievement of development and commercialization milestones, as well as tiered double-digit royalties on global sales of Amigal. We will jointly fund development costs with GSK in accordance with an agreed upon development plan, which provides that we will fund 50% of the development costs for 2011 and 25% of the development costs in 2012 and beyond, subject to annual and aggregate caps.

We commenced Study 011 in 2009 and expect to complete enrollment in the first half of 2011. We have enrolled a majority of the planned patients and expect to report top line results in the second half of the year. Study 011 is a 6-month, randomized, double-blind trial comparing Amigal to placebo in approximately 60 subjects. The surrogate primary endpoint is the change in the amount of kidney interstitial capillary GL-3. Subjects being enrolled are Fabry patients who have never received ERT, or who have not received ERT for at least 6 months, and who have a mutation responsive to Amigal. In the United States, we intend to seek Accelerated Approval for Amigal according to Subpart H regulations as an orphan drug.

In addition, we expect to commence Study 012 in the first half of 2011. Study 012 will be an 18-month, randomized, open-label study comparing Amigal to ERT in approximately 60 subjects. The primary outcome of efficacy will be renal function as measured by glomerular filtration rate (GFR).

We completed four Phase 2 clinical trials of Amigal in 2007 and our Phase 2 extension study is ongoing. The extension study is designed to evaluate the long-term safety and efficacy of Amigal. Among the endpoints being evaluated are two measures of renal function, estimated glomerular filtration rate (eGFR) and 24-hour urine protein.

The key findings from the Phase 2 studies and long term extension study to date include the following:

- Amigal has been generally safe and well-tolerated at all doses evaluated and no drug-related serious adverse events have been reported.
- Amigal increased the level of the enzyme deficient in 24 of the 26 original Phase 2 study subjects
- Amigal was shown to reduce the accumulated substrate in a majority of study subjects.
- eGFR has remained stable out to 3-4 years for all subjects in the extension study.
- Trends of reduced 24-hour urine protein continued to be observed in subjects identified as responders to Amigal; seventeen subjects continue to receive treatment in the Phase 2 extension study
- Responses in patients with different Fabry mutations were consistent with the results of in vitro testing, thus confirming the ability to use pharmacogenetics to select likely responders for future studies.

In February 2004, the FDA granted orphan drug designation to Amigal for the treatment of Fabry disease and in May 2006, the EMEA granted orphan medicinal product designation for Amigal.



***Causes of Fabry Disease and Rationale for Use of Amigal***

Fabry disease is a lysosomal storage disorder resulting from a deficiency in  $\alpha$ -GAL. Symptoms can be severe and debilitating, including kidney failure and increased risk of heart attack and stroke. The deficiency of  $\alpha$ -Gal A in Fabry patients is caused by inherited genetic mutations. Certain of these mutations cause changes in the amino acid sequence of  $\alpha$ -Gal A that may result in the production of  $\alpha$ -Gal A with reduced stability that does not fold into its correct three-dimensional shape. Although  $\alpha$ -Gal A produced in patient cells often retains the potential for some level of biological activity, the cell's quality control mechanisms recognize and retain misfolded  $\alpha$ -Gal A in the ER, until it is ultimately moved to another part of the cell for degradation and elimination. Consequently, little or no  $\alpha$ -Gal A moves to the lysosome, where it normally breaks down GL-3. This leads to accumulation of GL-3 in cells, which is believed to be the cause of the symptoms of Fabry disease. In addition, accumulation of the misfolded  $\alpha$ -Gal A enzyme in the ER may lead to stress on cells and inflammatory-like responses, which may contribute to cellular dysfunction and disease.

Amigal is designed to act as a pharmacological chaperone for  $\alpha$ -Gal A by selectively binding to the enzyme, which increases its stability and helps the enzyme fold into its correct three-dimensional shape. This stabilization of  $\alpha$ -Gal A allows the cell's quality control mechanisms to recognize the enzyme as properly folded so that trafficking of the enzyme to the lysosome is increased, enabling it to carry out its intended biological function, the metabolism of GL-3. As a result of restoring the proper trafficking of  $\alpha$ -Gal A from the ER to the lysosome, Amigal also reduces the accumulation of misfolded protein in the ER, which may alleviate stress on cells and some inflammatory-like responses that may be contributing factors in Fabry disease.

Because Amigal increases levels of a patient's naturally produced  $\alpha$ -GAL, those Fabry disease patients with a missense mutation or other genetic mutations that result in production of  $\alpha$ -Gal A that is less stable but with some residual enzyme activity are the ones most likely to respond to treatment with Amigal. We estimate that approximately fifty percent of patients with Fabry disease may respond to pharmacological chaperone therapy. Patients with genetic mutations leading to a partially made  $\alpha$ -Gal A enzyme or  $\alpha$ -Gal A enzyme with an irreversible loss of activity are less likely to respond to treatment with Amigal.

***Fabry Disease Background***

The clinical manifestations of Fabry disease span a broad spectrum of severity and roughly correlate with a patient's residual  $\alpha$ -Gal A levels. The majority of currently treated patients are referred to as classic Fabry disease patients, most of whom are males. These patients experience disease of various organs, including the kidneys, heart and brain, with disease symptoms first appearing in adolescence and typically progressing in severity until death in the fourth or fifth decade of life. A number of studies suggest that there are a large number of undiagnosed males and females that have a range of Fabry disease symptoms, such as impaired cardiac or renal function and strokes, that usually first appear in adulthood.

Individuals with this type of Fabry disease, referred to as later-onset Fabry disease, tend to have higher residual  $\alpha$ -Gal A levels than classic Fabry disease patients. Although the symptoms of Fabry disease span a spectrum of severity, it is useful to classify patients as having classic or later-onset Fabry disease when discussing the disease and the associated treatable population.

***Classic Fabry Disease***

Individuals with classic Fabry disease are in most instances males. They have little or no detectable  $\alpha$ -Gal A levels and are the most severely affected. These patients first experience disease symptoms in adolescence, including pain and tingling in the extremities, skin lesions, a decreased ability to sweat and clouded eye lenses. If these patients are not treated, their life expectancy is reduced and death usually occurs in the fourth or fifth decade of life from renal failure, cardiac dysfunction or stroke. Studies reported in the *Journal of the American Medical Association* (January 1999) and *The Metabolic and Molecular Bases of Inherited Disease* (8th edition 2001) suggest the annual incidence of Fabry disease in newborn males is 1:40,000-1:60,000. Current estimates from the University of Iowa and the National Kidney Foundation suggest that there are a total of approximately 5,000 classic Fabry disease patients worldwide.

***Later-onset Fabry Disease***

Individuals with later-onset Fabry disease can be male or female. They typically first experience disease symptoms in adulthood, and often have disease symptoms focused on a single organ. For example, many males and females with later-onset Fabry disease have enlargement of the left ventricle of the heart. As the patients advance in age, the cardiac complications of the disease progress and can lead to death. Studies reported in *Circulation* and *Journal of the American Heart Association* (March 2002 and August 2004), estimated that 6-12% of patients between 40 and 60 years of age with an unexplained enlargement of the left ventricle of the heart, a condition referred to as left ventricular hypertrophy, have Fabry disease.

A number of males and females also have later-onset Fabry disease with disease symptoms focused on the kidney that progress to end stage renal failure and eventually death. Studies reported in Nephrology Dialysis Transplant (2003), Clinical and Experimental Nephrology (2005) and Nephrology Clinical Practice (2005) estimate that 0.20% to 0.94% of patients on dialysis have Fabry disease.

In addition, later-onset Fabry disease may also present in the form of strokes of unknown cause. A recent study reported in The Lancet (November 2005) found that approximately 4% of 721 male and female patients in Germany between the ages of 18 to 55 with stroke of unknown cause have Fabry disease.

It was previously believed to be rare for female Fabry disease patients to develop overt clinical manifestations of Fabry disease. Fabry disease is known as an X-linked disease because the inherited  $\alpha$ -Gal A gene mutation is located only on the X chromosome. Females inherit an X chromosome from each parent and therefore can inherit a Fabry mutation from either parent. By contrast, males inherit an X chromosome (and potentially a Fabry mutation) only from their mothers. For this reason, there are expected to be roughly twice as many females as males that have Fabry disease mutations. Several studies reported in the Journal of Medical Genetics (2001), the Internal Medicine Journal (2002) and the Journal of Inherited Metabolic Disease (2001) report that, while the majority of females with Fabry disease mutations have mild symptoms, many have severe symptoms, including enlargement of the left ventricle of the heart and/or renal failure.

In a recent study reported in the American Journal of Human Genetics, more than thirty-seven thousand newborn males in Italy were screened for  $\alpha$ -Gal A activity and mutations. The incidence of Fabry mutations in this study was 1:3,100, over ten times higher than previous estimates. This high incidence was attributed to a large number of newborn males with  $\alpha$ -Gal A mutations often associated with later-onset Fabry disease, which may not have been identified in previous screening studies that relied on diagnosis based on development of symptoms of classic Fabry disease.

### ***Fabry Disease Market Opportunity***

Fabry disease is a relatively rare disorder. The current estimates of approximately 5,000 patients worldwide are generally based on a small number of studies in single ethnic populations in which people were screened for classic Fabry disease. The results of these studies were subsequently extrapolated to the broader world population assuming similar prevalence rates across populations. We believe these previously reported studies did not account for the prevalence of later-onset Fabry disease and, as described above, a number of recent studies suggest that the prevalence of Fabry disease could be many times higher than previously reported.

We expect that as awareness of later-onset Fabry disease grows, the number of patients diagnosed with the disease will increase. Increased awareness of all forms of Fabry disease, particularly for specialists not accustomed to treating Fabry disease patients, may lead to increased testing and diagnosis of patients with the disease. We intend to develop and launch educational and awareness campaigns targeting cardiologists, nephrologists and neurologists regarding Fabry disease and its diagnosis. Assuming we receive regulatory approval, we expect these educational and awareness campaigns would continue as a part of the marketing of Amigal.

Based on published data from the Human Gene Mutation Database and our experience in the field, we believe the majority of the known genetic mutations that cause Fabry disease are missense mutations. There are few widely-occurring genetic mutations reported for Fabry disease, suggesting that the frequency of a specific genetic mutation reported in the Human Gene Mutation Database reflects the approximate frequency of that mutation in the general Fabry patient population. In addition, data presented at the 11th International Conference on Health Problems Related to the Chinese (2002) suggest that the vast majority of newly diagnosed patients with later-onset Fabry disease also have missense mutations. Because missense mutations often result in less stable, misfolded  $\alpha$ -Gal A with some residual enzyme activity, we believe patients with these mutations may benefit from treatment with Amigal. We also believe that other types of genetic mutations may result in misfolded  $\alpha$ -Gal A and therefore may respond to treatment with Amigal. Based on this, we believe that approximately fifty percent of the Fabry disease patient population may benefit from treatment with Amigal.

### ***Existing Products for the Treatment of Fabry Disease and Potential Advantages of Amigal***

The current standard of treatment for Fabry disease is enzyme replacement therapy. Currently, two products are approved for the treatment of Fabry disease: Fabrazyme<sup>®</sup> and Replagal<sup>®</sup>. Fabrazyme<sup>®</sup> is approved globally and commercialized by Genzyme Corporation. Fabrazyme<sup>®</sup> was approved in the U.S. in 2003 and in the EU in 2001. Orphan drug exclusivity for Fabrazyme<sup>®</sup> expired in the U.S. in 2010 and will expire in EU in 2011. Replagal<sup>®</sup> is commercialized by Shire and approved in the EU and other countries but not in the U.S., Replagal<sup>®</sup> was approved in the EU in August 2001 and has orphan drug exclusivity in the EU until 2011. The net product sales of Fabrazyme<sup>®</sup> and Replagal<sup>®</sup> for 2010 were approximately \$188 million as publicly reported by Genzyme Corporation and \$351 million as publicly reported by Shire, respectively.

Prior to the availability of ERT, treatments for Fabry disease were directed at ameliorating symptoms without treating the underlying disease. Some of these treatments include opiates, anticonvulsants, antipsychotics and antidepressants to control pain and other symptoms, and beta-blockers, calcium channel blockers, ACE inhibitors, angiotensin receptor antagonists and other agents to treat blood pressure and vascular disease.

For Fabry disease patients who respond to Amigal, we believe that the use of Amigal may have advantages relative to the use of Fabrazyme<sup>®</sup> and Replagal<sup>®</sup>. Published data for patients treated with Fabrazyme<sup>®</sup> and Replagal<sup>®</sup> for periods of up to five years demonstrate that these drugs can lead to the reduction of GL-3 in multiple cell types in the skin, heart and kidney. However, because they are large protein molecules, Fabrazyme<sup>®</sup> and Replagal<sup>®</sup> are believed to have difficulty penetrating some tissues and cell types. In particular, it is widely believed that Fabrazyme<sup>®</sup> and Replagal<sup>®</sup> are unable to cross the blood-brain barrier and thus are unlikely to address the neurological symptoms of Fabry disease. As a small molecule therapy that has demonstrated high oral bioavailability and good biodistribution properties in preclinical testing, Amigal has the potential to reach cells of all the target tissues of Fabry disease. Furthermore, treatment with Fabrazyme<sup>®</sup> and Replagal<sup>®</sup> requires intravenous infusions every other week, frequently on-site at health care facilities, presenting an inconvenience to Fabry patients. Oral treatment with Amigal may be much more convenient for patients and may not have the safety risks associated with intravenous infusions.

In February 2004, Amigal was granted orphan drug designation by the FDA for the treatment of Fabry disease and in March 2006 the EMEA recommended orphan medicinal product designation for Amigal. We believe that orphan drug designation of Fabrazyme<sup>®</sup> in the U.S. and of Fabrazyme<sup>®</sup> and Replagal<sup>®</sup> in the EU will not prevent us from obtaining marketing approval of Amigal in either geography. See “Government Regulation.”

### **Chaperone-ERT Co-administration Therapy**

We are currently conducting clinical and preclinical studies on the use of pharmacological chaperones co-administered with ERT. Pharmacological chaperones are designed to selectively bind to target enzymes in patient cells, thereby increasing protein stability and allowing for increased transport to lysosomes and degradation of substrate by the enzyme. When used in combination with ERT, we believe that these binding and stabilization properties may improve key characteristics of the infused enzymes used in ERT by allowing for increased transport of enzymes to the lysosomes and degradation of substrate, thereby increasing ERT's safety and efficacy. At several scientific conferences, we have presented data which demonstrated that the addition of a pharmacological chaperone to ERT has the potential to address key limitations of ERT, such as a lack of stability in circulation which can reduce safety and efficacy. In particular, in February 2010, we presented data from preclinical studies that evaluated the combination of Amigal and an ERT, and another pharmacological chaperone, AT2220 (1-deoxynojirimycin HC1) and a different ERT, in mouse models of Fabry and Pompe disease, respectively. Studies of both combinations demonstrated that co-administration of the chaperone with ERT resulted in prolonged half-life of the administered enzyme in the circulation, increased enzyme activity in cells and greater substrate reduction in target tissues compared to that seen with ERT alone.

In February 2011, along with our partner GSK, we initiated a Phase 2 study designed to evaluate the co-administration of Amigal with ERT for Fabry disease. This study is investigating drug-drug interactions between Amigal and the ERTs Fabrazyme<sup>®</sup> and Replagal<sup>®</sup> in 18 male patients with Fabry disease, ages 18-65, who have been receiving ERT for at least one month before entry into the study. Patients, who need not have a genetic mutation responsive to Amigal as a monotherapy, will receive ERT alone and then ERT after administration of a single oral dose of Amigal. We expect to have results from this study in the second half of 2011.

Additionally, we expect to initiate a Phase 2 study with our pharmacological chaperone AT2220 co-administered with ERT for Pompe disease in the first half of 2011 with results expected in the second half of the year. We will seek FDA approval to lift the clinical hold on the AT2220 program in order to conduct this study.

### **Diseases of Neurodegeneration**

We are also conducting preclinical studies on the use of our pharmacological chaperone technology to treat diseases of neurodegeneration, with an initial focus on genetically-identified subpopulations within Parkinson's and Alzheimer's disease patients. We believe the knowledge we have gained from exploring the use of pharmacological chaperones in rare genetic diseases can be applied to these non-lysosomal storage disease applications, especially in light of recent studies linking Parkinson's disease to Gaucher disease and Alzheimer's disease with lysosomal storage diseases. We believe that pharmacological chaperones may be used to stabilize mutated proteins and further stabilize normal or “wild-type” proteins, and may therefore increase the cellular amounts and activities of specifically chosen target proteins that may be important for the treatment of neurodegenerative diseases. While our initial efforts are focused on subpopulations of Parkinson's and Alzheimer's patients, we believe the characteristics of chaperones may make treatment of broader populations within these diseases possible.

Recent population genetics studies have established a link between being a Gaucher carrier and developing Parkinson's disease. In particular, these studies demonstrate that Gaucher carriers have an estimated five-fold increased risk for Parkinson's disease and Gaucher patients an estimated twenty-fold increase risk, and that both tend to develop Parkinson's at an earlier age. We previously presented data evaluating a pharmacological chaperone in relevant mouse models. These studies, funded in part by a grant from the Michael J. Fox Foundation, demonstrated that treatment with the chaperone increased the activity of  $\beta$ -glucocerebrosidase (GCase), prevented accumulation of  $\alpha$ -synuclein in the brain and improved motor function as assessed in various behavioral tests. As previously reported, we have developed new compounds that improve on the properties of this chaperone and expand the range of doses and regimens that show motor improvement in mouse models of the disease. We expect to complete late-stage preclinical proof-of-concept studies, including IND-enabling activities, for our pharmacological chaperone AT3375 for the treatment of Parkinson's disease during 2011.

Our second preclinical neurodegenerative disease program is for Alzheimer's disease. We are currently researching novel approaches to treating patients with Genetic (Familial) Alzheimer's through a Presenilin-1 target and those with Sporadic Alzheimer's, with a focus on a lysosomal enzyme target. Our work in Alzheimer's also builds on the understanding of pharmacological chaperones we have developed over the past several years examining treatment of lysosomal storage disorders and our work in Parkinson's disease. We expect to continue preclinical proof-of-concept studies in Alzheimer's disease during 2011.

### **Parkinson's Disease Background**

Parkinson's disease is a chronic, degenerative neurological disorder of the central nervous system that results from the loss of cells in various parts of the brain, including a region called the substantia nigra. The substantia nigra cells produce dopamine, a chemical messenger responsible for transmitting signals within the brain that allow for coordination of movement. Loss of dopamine causes neurons to fire without normal control, leaving patients less able to direct or control their movement. The key signs of Parkinson's disease are resting tremor, slowness of movement (bradykinesia), postural instability (balance problems) and rigidity. Other symptoms include stiff facial expression, shuffling walk, muffled speech and depression.

Parkinson's disease affects both men and women in almost equal numbers and shows no social, ethnic, economic or geographic boundaries. While the condition usually develops after the age of 65, 15% of those diagnosed are under 50. It is estimated that approximately 1 million people in the United States suffer from Parkinson's disease.

### **Alzheimer's Disease Background**

Alzheimer's disease is an irreversible, progressive and fatal brain disease that slowly destroys memory and thinking skills, and eventually the ability to perform even simple tasks. It is the most common form of dementia. Although the cause of Alzheimer's disease is unknown, two abnormal structures in the brain called plaques and tangles are believed to play a significant role in the manifestation of the disease. Tangles, which are twisted fibers of the protein tau, begin to develop deep in the brain, in an area called the entorhinal cortex, and plaques, which contain deposits of a protein fragment called beta-amyloid, form in other areas. As more and more plaques and tangles form in particular brain areas, healthy neurons begin to work less efficiently, lose their ability to function and communicate with each other, and eventually die. As the death of neurons increases, affected brain regions begin to shrink. By the final stage of Alzheimer's, damage is widespread and brain tissue has shrunk significantly.

In most people, Alzheimer's symptoms first appear after age 60. It is estimated that approximately 5.1 million people in the United States suffer from Alzheimer's disease.

### **Other Product Candidates**

In addition to Amigal, we have two other pharmacological chaperone product candidates in clinical development, AT2220 (1-deoxynojirimycin HCl) for Pompe disease and Plicera (afegostat tartrate) for Gaucher disease. We are also evaluating additional pharmacological chaperones for our Gaucher program. The FDA previously granted orphan drug designation for the active ingredient in Plicera for the treatment of Gaucher disease in the United States as well as for the active ingredient in AT2220.

#### ***AT2220 for Pompe Disease***

Results of our preclinical studies evaluating AT2220 co-administered with ERT have been encouraging. We therefore intend to seek FDA approval to lift the clinical hold on our AT2220 program to permit us to conduct the Phase 2 study of AT2220 co-administered with ERT discussed above. As previously reported, we suspended enrollment for the Phase 2 clinical trial of our investigational drug AT2220 in adults with Pompe disease and received notice from the FDA that the AT2220 Investigational New Drug application (IND) was placed on clinical hold following two separate self-reported adverse events by patients in the trial, which were categorized by the site investigator as serious and probably related to treatment with AT2220. We conducted a Phase 1 study of AT2220 to evaluate the pharmacokinetics of AT2220 in muscle tissue in healthy adult subjects and announced results from the trial in 2010.



### *Plicera for Gaucher Disease*

Based on the preliminary results from our Phase 2 study of Plicera in treatment-naive adult patients with type 1 Gaucher disease, we decided not to advance Plicera into Phase 3 development. However, we remain encouraged by the results of preclinical studies designed to evaluate the use of Plicera co-administered with ERT, and will continue to evaluate the Plicera program. In addition, we are developing additional compounds that we believe have potential as Gaucher product candidates, both as monotherapies and co-administered with ERT.

### *Pompe and Gaucher Disease Background*

Like Fabry disease, Pompe and Gaucher disease are lysosomal storage disorders resulting from a deficiency in an enzyme,  $\alpha$ -glucosidase (GAA) for Pompe and GCCase for Gaucher. Signs and symptoms of both diseases can be severe and debilitating. For Pompe, they include progressive muscle weakness throughout the body, particularly the heart and skeletal muscles; while patients suffering from Gaucher may experience an enlarged liver and spleen, abnormally low levels of red blood cells and platelets, and skeletal complications. In some forms of Gaucher disease, there is also significant impairment of the central nervous system. The enzyme deficiencies in Pompe and Gaucher patients are caused by inherited genetic mutations. Certain of these mutations cause changes in the amino acid sequence of the enzyme that may result in the production of an enzyme with reduced stability that does not fold into its correct three-dimensional shape. Although the enzymes produced in patient cells often retain the potential for some level of biological activity, the cell's quality control mechanisms recognize and retain the misfolded enzyme in the ER until it is ultimately moved to another part of the cell for degradation and elimination. Consequently, little or no GAA in Pompe patients or GCCase in Gaucher patients moves to the lysosome, where it normally breaks down its substrate, a complex lipid called glycogen in Pompe patients and glucocerebroside in Gaucher patients. This leads to accumulation of glycogen or glucocerebroside in cells, which is believed to result in the clinical manifestations of Pompe and Gaucher disease, respectively. In addition, the accumulation of the misfolded enzyme in the ER may lead to cellular stress and inflammatory-like responses, which may contribute to cellular dysfunction and disease.

### **Strategic Alliances and Arrangements**

On October 28, 2010, the Company entered into the License and Collaboration Agreement with Glaxo Group Limited, an affiliate of GSK, to develop and commercialize Amigal. Under the terms of the License and Collaboration Agreement, GSK received an exclusive worldwide license to develop, manufacture and commercialize Amigal. In consideration of the license grant, the Company received an upfront, license payment of \$30 million from GSK and is eligible to receive further payments of approximately \$170 million upon the successful achievement of development and commercialization milestones, as well as tiered double-digit royalties on global sales of Amigal. GSK and the Company will jointly fund development costs in accordance with an agreed upon development plan. This plan provides that the Company will fund 50% of the development costs for 2011 and 25% of the development costs in 2012 and beyond. The Company's development costs are subject to annual and aggregate caps. Additionally, GSK purchased approximately 6.9 million shares of the Company's common stock at a price of \$4.56 per share. The total value of this equity investment to the Company is approximately \$31 million and represents a 19.9% ownership position in the Company.

Under the terms of the Agreement, while we will collaborate with GSK, GSK will have decision-making authority over clinical, regulatory and commercial matters. Additionally, GSK will have primary responsibility for interactions with regulatory agencies and prosecuting applications for marketing and reimbursement approvals worldwide.

We will continue to evaluate other business development opportunities as appropriate that build shareholder value and provide us with access to the financial, technical, clinical and commercial resources necessary to develop and market pharmacological chaperone therapeutics. We are exploring potential collaborations, alliances and other business development opportunities on a regular basis.

### **Intellectual Property**

#### *Patents and Trade Secrets*

Our success depends in part on our ability to maintain proprietary protection surrounding our product candidates, technology and know-how, to operate without infringing the proprietary rights of others, and to prevent others from infringing our proprietary rights. Our policy is to seek to protect our proprietary position by filing U.S. and foreign patent applications related to our proprietary technology, including both new inventions and improvements of existing technology, that are important to the development of our business, unless this proprietary position would be better protected using trade secrets. Our patent strategy includes obtaining patent protection, where possible, on compositions of matter, methods of manufacture, methods of use, combination therapies, dosing and administration regimens, formulations, therapeutic monitoring, screening methods and assays. We also rely on trade secrets, know-how, continuing technological innovation, in-licensing and partnership opportunities to develop and maintain our proprietary position. Lastly, we monitor third parties for activities that may infringe our proprietary rights, as well as the progression of third party patent applications that may have the potential to create blocks to our products or otherwise interfere with the development of our business. We are aware, for example, of U.S. patents, and corresponding international counterparts, owned by third parties that contain claims related to treating protein misfolding. If any of these patents were to be asserted against us we do not believe that our proposed products would be found to infringe any valid claim of these patents. There is no assurance that a court would find in our favor or that, if we choose or are required to seek a license, a license to any of these patents would be available to us on acceptable terms or at all.



We own or license rights to several issued patents in the U.S., current member states of the European Patent Convention and numerous pending foreign applications, which are foreign counterparts of many of our U.S. patents. We also own or license rights to several pending U.S. applications. Our patent portfolio includes patents and patent applications with claims relating to methods of increasing deficient enzyme activity to treat genetic diseases. The patent positions for Amigal (migalastat HCl), our most advanced product candidate for Fabry disease, pharmacological chaperone and ERT combination therapy, diseases of neurodegeneration, Plicera (afegostat tartrate) for Gaucher disease and AT2220 (1-deoxynojirimycin HCl) for Pompe disease are described below and include both patents and patent applications we own or exclusively license:

- We have an exclusive license to six issued U.S. patents and two pending U.S. applications that cover use of Amigal to treat Fabry disease, as well as corresponding European and Japanese patents, and a pending application in Canada. These exclusively licensed U.S. patents relating to Amigal expire in 2018 (not including the Hatch-Waxman statutory extension, which is described below), while the European and Japanese patents and foreign counterpart patent application in Canada, if granted, will expire in 2019 (not including the Supplemental Protection Certificates or SPC extensions, which are described below). The patents and the pending applications include claims covering methods of increasing the activity of and preventing the degradation of  $\alpha$ -GAL, and methods for the treatment of Fabry disease using Amigal. In addition, we own pending U.S. applications directed to specific treatment and monitoring regimens with Amigal as well as to dosing regimens with Amigal, which, if granted, may result in patents that expire in 2028. Further, we have a pending U.S. application directed to synthetic steps related to the commercial process for preparing Amigal, which may result in a patent that expires in 2026. Lastly, we jointly own one pending U.S. application and another pending international stage application covering methods of diagnosing Fabry disease and determining whether Fabry patients will respond to treatment with Amigal, which, if granted, will expire in 2027 and 2029, respectively. We have filed, or plan to file, U.S. and foreign counterparts of these applications, where appropriate, by the applicable deadlines.
- We have an exclusive license to pending patent applications covering the co-administration of Amigal with ERT (recombinant  $\alpha$ -galactosidase A), Plicera with ERT (recombinant glucocerebrosidase) and AT2220 wither (recombinant acid  $\alpha$ -glucosidase). These applications are pending in the U.S., Europe, Canada, Brazil, China, Israel, India, Japan and Mexico. If patents issue from these applications, expiration will be in 2024.
- As part of our License and Collaboration Agreement with GSK, we have licensed or sub-licensed to GSK all of our worldwide rights in our patents and applications to the extent that said patents and applications claim the use of Amigal as a monotherapy or co-administered with ERT.
- We own several US and foreign pending patent applications which cover the use of pharmacological chaperones to treat diseases of neurodegeneration. In particular we own two issued patents and one patent application that cover the use of isofagomine and/or its derivatives to treat Parkinson's disease and one patent application covering novel compounds for the treatment of Parkinson's disease. Further, we own two patent applications that cover the use of pharmacological chaperones to treat Alzheimer's disease. If patents issue from these applications expiration dates range from 2026 to 2030.
- We have an exclusive license to several U.S. patents and one pending U.S. application covering the use of Plicera to treat Gaucher disease. These patents expire in 2018 (not including the Hatch-Waxman statutory extension, which is described below). We also have an exclusive license to two U.S. patents claiming isofagomine, the active chemical moiety in Plicera, which expire in 2015 and 2016 (not including the Hatch-Waxman statutory extension, which is described below); and corresponding patents in the UK, France, Sweden, Germany, Switzerland and Japan all of which expire in 2015 (not including the SPC extensions, which are described below). We own a U.S. patent and its corresponding foreign applications covering isofagomine tartrate, which is the specific salt form or the active pharmaceutical ingredient in Plicera, which expires in 2027. We own several other pending U.S. applications directed to the synthesis of Plicera, dosing regimens of Plicera as well as specific treatment and monitoring regimens with Plicera which, if granted, will expire in 2028. We have filed, or plan to file, foreign counterparts of these applications, where appropriate, by the applicable deadlines.
- We have an exclusive license to several U.S. patents that cover the use of AT2220 to treat Pompe disease. These U.S. patents will expire in 2018 (not including the Hatch-Waxman statutory extension, which is described below). We own a U.S. patent application that covers dosing regimens of AT2220 to treat Pompe disease. We have filed, or plan to file, foreign counterparts of these applications, where appropriate, by the applicable deadlines.

Individual patents extend for varying periods depending on the effective date of filing of the patent application or the date of patent issuance, and the legal term of the patents in the countries in which they are obtained. Generally, patents issued in the U.S. are effective for:

- the longer of 17 years from the issue date or 20 years from the earliest effective filing date, if the patent application was filed prior to June 8, 1995; and
- 20 years from the earliest effective filing date, if the patent application was filed on or after June 8, 1995.

The term of foreign patents varies in accordance with provisions of applicable local law, but typically is 20 years from the earliest effective filing date.

The U.S. Drug Price Competition and Patent Term Restoration Act of 1984, more commonly known as the Hatch-Waxman Act, provides for an extension of one patent, known as a Hatch-Waxman statutory extension, for each NCE to compensate for a portion of the time spent in clinical development and regulatory review. However, the maximum extension is five years and the extension cannot extend the patent beyond 14 years from New Drug Application (NDA) approval. Similar extensions are available in European countries, known as SPC extensions, Japan and other countries. However, we will not know what, if any, extensions are available until a drug is approved. In addition, in the U.S., under provisions of the Best Pharmaceuticals for Children's Act, we may be entitled to an additional six month period of patent protection Market Exclusivity and Orphan Drug Exclusivity, for completing pediatric clinical studies in response to a FDA issued Pediatric Written Request before said exclusivities expire.

The patent positions of companies like ours are generally uncertain and involve complex legal, technical, scientific and factual questions. Our ability to maintain and solidify our proprietary position for our technology will depend on our success in promptly filing patent applications on new discoveries, and in obtaining effective claims and enforcing those claims once granted. We focus special attention on filing patent applications for formulations and delivery regimens for our products in development to further enhance our patent exclusivity for those products. We seek to protect our proprietary technology and processes, in part, by contracting with our employees, collaborators, scientific advisors and our commercial consultants to ensure that any inventions resulting from the relationship are disclosed promptly, maintained in confidence until a patent application is filed and preferably until publication of the patent application, and assigned to us or subject to a right to obtain a license. We do not know whether any of our own patent applications or those patent applications that are licensed to us will result in the issuance of any patents. Our issued patents and those that may issue in the future, or those licensed to us, may be challenged, narrowed, invalidated or circumvented or be found to be invalid or unenforceable, which could limit our ability to stop competitors from marketing related products and reduce the term of patent protection that we may have for our products. Neither we nor our licensors can be certain that we were the first to invent the inventions claimed in our owned or licensed patents or patent applications. In addition, our competitors may independently develop similar technologies or duplicate any technology developed by us and the rights granted under any issued patents may not provide us with any meaningful competitive advantages against these competitors. Furthermore, because of the extensive time required for development, testing and regulatory review of a potential product, it is possible that any related patent may expire prior to or shortly after commencing commercialization, thereby reducing the advantage of the patent to our business and products.

We may rely, in some circumstances, on trade secrets to protect our technology. However, trade secrets are difficult to protect. We seek to protect our trade secret technology and processes, in part, by entering into confidentiality agreements with commercial partners, collaborators, employees, consultants, scientific advisors and other contractors, and by contracting with our employees and some of our commercial consultants to ensure that any trade secrets resulting from such employment or consulting are owned by us. We also seek to preserve the integrity and confidentiality of our data and trade secrets by maintaining physical security of our premises and physical and electronic security of our information technology systems. While we have confidence in these individuals, organizations and systems, agreements or security measures may be breached, and we may not have adequate remedies for any breach. In addition, our trade secrets may otherwise become known or be discovered independently by others. To the extent that our consultants, contractors or collaborators use intellectual property owned by others in their work for us, disputes may arise as to the rights in related or resulting know-how and inventions.

### License Agreements

We have acquired rights to develop and commercialize our product candidates through licenses granted by various parties. The following summarizes our material rights and obligations under those licenses:

- *Mt. Sinai School of Medicine* — We have acquired exclusive worldwide patent rights to develop and commercialize Amigal, Plicera and AT2220 and other pharmacological chaperones for the prevention or treatment of human diseases or clinical conditions by increasing the activity of wild-type and mutant enzymes pursuant to a license agreement with Mt. Sinai School of Medicine (MSSM) of New York University. In connection with this agreement, we issued 232,266 shares of our common stock to MSSM in April 2002. In October 2006 we issued MSSM an additional 133,333 shares of common stock and made a payment of \$1.0 million in consideration of an expanded field of use under that license. Under this agreement, to date we have paid no upfront or annual license fees and we have no milestone or future payments other than royalties on net sales. However, on October 31, 2008, we amended and restated this license agreement to, among other items, provide us with the sole right to control the prosecution of patent rights under such agreement and to clarify the portion of royalties and milestone payments we received from Shire that were payable to MSSM. In connection therewith, we agreed to pay MSSM \$2.6 million in connection with the \$50 million upfront payment that we received in November 2007 from Shire, which was already accrued for at year-end 2007, and an additional \$2.6 million for the sole right to and control over the prosecution of patent rights. In addition, we paid MSSM \$3 million of the \$30 million upfront payment received from GSK in the fourth quarter of 2010. This agreement expires upon expiration of the last of the licensed patent rights, which will be in 2019, subject to any patent term extension that may be granted, or 2024 if we develop a product for combination therapy (pharmacological chaperone plus ERT) and a patent issues from the pending application covering the combination therapy, subject to any patent term extension that may be granted.
- *University of Maryland, Baltimore County* — We have acquired exclusive U.S. patent rights to develop and commercialize Plicera for the treatment of Gaucher disease from the University of Maryland, Baltimore County. Under this agreement, to date we have paid aggregate upfront and annual license fees of \$45 thousand. We are required to make a milestone payment upon the demonstration of safety and efficacy of Plicera for the treatment of Gaucher disease in a Phase 2 study, and another payment upon receiving FDA approval for Plicera for the treatment of Gaucher disease. We are also required to pay royalties on net sales. Upon satisfaction of both milestones, we could be required to make up to \$0.2 million in aggregate payments. This agreement expires upon expiration of the last of the licensed patent rights in 2015.
- *Novo Nordisk A/S* — We have acquired exclusive patent rights to develop and commercialize Plicera for all human indications. Under this agreement, to date we have paid an aggregate of \$0.4 million in license fees. We are also required to make milestone payments based on clinical progress of Plicera, with a payment due after initiation of a Phase 3 clinical trial for Plicera for the treatment of Gaucher disease and a payment due upon each filing for regulatory approval of Plicera for the treatment of Gaucher disease in any of the U.S., Europe or Japan. An additional payment is due upon approval of Plicera for the treatment of Gaucher disease in the U.S. and a payment is also due upon each approval of Plicera for the treatment of Gaucher disease in either of Europe or Japan. Assuming successful development of Plicera for the treatment of Gaucher disease in the U.S., Europe and Japan, total milestone payments would be \$7.8 million. We are also required to pay royalties on net sales. This license will terminate in 2016.

Under our license agreements, if we owe royalties on net sales for one of our products to more than one of the above licensors, then we have the right to reduce the royalties owed to one licensor for royalties paid to another. The amount of royalties to be offset is generally limited in each license and can vary under each agreement. For Amigal and AT2220, we will owe royalties only to MSSM and will owe no milestone payments. We would expect to pay royalties to all three licensors with respect to Plicera.

Our rights with respect to these agreements to develop and commercialize Amigal, Plicera and AT2220 may terminate, in whole or in part, if we fail to meet certain development or commercialization requirements or if we do not meet our obligations to make royalty payments.

### Trademarks

In addition to our patents and trade secrets, we have filed applications to register certain trademarks in the U.S. and/or abroad, including AMICUS, AMICUS THERAPEUTICS (and design), AMIGAL and PLICERA. At present, all of the U.S. trademark applications for these marks, which are based on an intention to use these marks, have been either registered or approved by the U.S. Patent and Trademark Office and Notices of Allowances and have been issued. We have also received foreign allowances or issued foreign registrations for certain of these marks. Our ability to obtain and maintain trademark registrations will in certain instances depend on making use of the mark in commerce on or in connection with our products. For the allowed marks for our candidate products, it may be necessary to re-apply for registration if it becomes apparent that we will not use the mark in commerce within the prescribed time period. As part of our License and Collaboration Agreement with GSK, we have licensed our worldwide rights in the Amigal trademark to GSK.

### Manufacturing

We continue to rely on contract manufacturers to supply the active pharmaceutical ingredients and gelatin capsules for Amigal and our other product candidates. The active pharmaceutical ingredients for these products are manufactured under current good

manufacturing practices (cGMP), at kilogram scale initiated with commercially available starting materials. The components in the final formulation for each product are commonly used in other encapsulated products and are well characterized ingredients. We have implemented appropriate controls for assuring the quality of both active pharmaceutical ingredients and capsules. Product specifications will be established in concurrence with regulatory bodies at the time of product registration.

## **Competition**

### ***Overview***

The biotechnology and pharmaceutical industries are characterized by rapidly advancing technologies, intense competition and a strong emphasis on proprietary products. In addition, several large pharmaceutical companies are increasingly focused on developing therapies for the treatment of rare diseases, both through organic growth and acquisitions and partnerships. While we believe that our technologies, knowledge, experience and scientific resources, along with our collaboration with GSK, provide us with competitive advantages, we face potential competition from many different sources, including commercial enterprises, academic institutions, government agencies and private and public research institutions. Any product candidates that we successfully develop and commercialize will compete with both existing and new therapies that may become available in the future.

Many of our competitors may have significantly greater financial resources and expertise associated with research and development, regulatory approvals and marketing approved products. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel, as well as in acquiring technologies complementary to, or necessary for, our programs. Smaller or early stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies.

Our commercial opportunities could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer side effects, are more convenient or are less expensive than products that we may develop. In addition, our ability to compete may be affected because in some cases insurers or other third party payors seek to encourage the use of generic products. This may have the effect of making branded products less attractive to buyers.

### ***Major Competitors***

Our major competitors include pharmaceutical and biotechnology companies in the U.S. and abroad that have approved therapies or therapies in development for lysosomal storage disorders within our core programs. Other competitors are pharmaceutical and biotechnology companies that have approved therapies or therapies in development for genetic diseases for which pharmacological chaperone technology may be applicable. Additionally, we are aware of several early-stage, niche pharmaceutical and biotechnology companies whose core business revolves around protein misfolding; however, we are not aware that any of these companies is currently working to develop products that would directly compete with ours. The key competitive factors affecting the success of our product candidates are likely to be their efficacy, safety, convenience and price.

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Any product candidates that we successfully develop and commercialize will compete with existing therapies and new therapies that may become available in the future. The following table lists our principal competitors and publicly available information on the status of their product offerings (U.S. dollars in millions):

<u>Competitor</u>	<u>Indication</u>	<u>Product</u>	<u>Class of Product</u>	<u>Status</u>	<u>2010 Sales</u> (in millions)
Genzyme Corporation	Fabry disease	Fabrazyme <sup>®</sup>	Enzyme Replacement Therapy	Marketed	\$ 188
	Gaucher disease	Cerezyme <sup>®</sup>	Enzyme Replacement Therapy	Marketed	\$ 720
	Pompe disease	Myozyme <sup>®</sup>	Enzyme Replacement Therapy	Marketed	\$ 412
	Gaucher disease	Eliglustat tartrate	Substrate Reduction Therapy	Phase 3	N/A
Shire	Fabry disease	Replagal <sup>®</sup>	Enzyme Replacement Therapy	Marketed	\$ 351
	Gaucher disease	VPRIV <sup>®</sup>	Enzyme Replacement Therapy	Marketed	\$ 143
Actelion, Ltd.	Gaucher disease	Zavesca <sup>®</sup>	Substrate Reduction Therapy	Marketed	\$ 66
Protalix Biotherapeutics	Gaucher disease	Taliglucerase alfa D	Enzyme Replacement Therapy	NDA filed December 2009	N/A

## Government Regulation

### *FDA Approval Process*

In the U.S., pharmaceutical products are subject to extensive regulation by the FDA. The Federal Food, Drug, and Cosmetic Act and other federal and state statutes and regulations, govern, among other things, the research, development, testing, manufacture, storage, recordkeeping, approval, labeling, promotion and marketing, distribution, post-approval monitoring and reporting, sampling, and import and export of pharmaceutical products. Failure to comply with applicable U.S. requirements may subject a company to a variety of administrative or judicial sanctions, such as FDA refusal to approve pending new drug applications (NDAs), warning letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, civil penalties, and criminal prosecution.

Pharmaceutical product development in the U.S. typically involves nonclinical laboratory and animal tests, the submission to the FDA of an investigational new drug application (IND), which must become effective before clinical testing may commence, and adequate and well-controlled clinical trials to establish the safety and effectiveness of the drug for each indication for which FDA approval is sought. Satisfaction of FDA pre-market approval requirements typically takes many years and the actual time required may vary substantially based upon the type, complexity and novelty of the product or disease. Preclinical tests include laboratory evaluation of product chemistry, formulation and toxicity, as well as animal trials to assess the characteristics and potential safety and efficacy of the product. The conduct of the preclinical tests must comply with federal regulations and requirements including good laboratory practices. The results of preclinical testing are submitted to the FDA as part of an IND along with other information including information about product chemistry, manufacturing and controls and a proposed clinical trial protocol. Long-term preclinical tests, such as animal tests of reproductive toxicity and carcinogenicity, may continue after the IND is submitted.

A 30-day waiting period after the submission of an IND is required prior to the commencement of clinical testing in humans. The IND becomes effective 30 days after its receipt by the FDA, and trials may begin at that point unless the FDA notifies the sponsor that the investigations are subject to a clinical hold.

Clinical trials involve the administration of the investigational new drug to healthy volunteers or patients under the supervision of a qualified investigator. Clinical trials must be conducted in compliance with applicable government regulations, good clinical practices (GCP), as well as under protocols detailing the objectives of the trial, the parameters to be used in monitoring safety and the effectiveness criteria to be evaluated. Each protocol involving testing on U.S. patients and subsequent protocol amendments must be submitted to the FDA as part of the IND.

The FDA may order the temporary or permanent discontinuation of a clinical trial at any time or impose other sanctions if it believes that the clinical trial is not being conducted in accordance with FDA requirements or presents an unacceptable risk to the clinical trial patients. The study protocol and informed consent information for patients in clinical trials must also be submitted to an institutional review board (IRB), for approval. An IRB may also require the clinical trial at the site to be halted, either temporarily or permanently, for failure to comply with the IRB's requirements, or may impose other conditions.

Clinical trials to support a new drug application (NDA) for marketing approval are typically conducted in three sequential phases, but the phases may overlap. In Phase 1, the initial introduction of the drug into healthy human subjects or patients, the drug is tested to assess metabolism, pharmacokinetics, pharmacological actions, side effects associated with increasing doses and, if possible, early evidence on effectiveness. Phase 2 usually involves trials in a limited patient population, to determine the effectiveness of the drug for a particular indication or indications, dosage tolerance and optimum dosage, and identify common adverse effects and safety risks. If a compound demonstrates evidence of effectiveness and an acceptable safety profile in Phase 2 evaluations, Phase 3 trials are undertaken to obtain the additional information about clinical efficacy and safety in a larger number of patients, typically at geographically dispersed clinical trial sites, to permit FDA to evaluate the overall benefit-risk relationship of the drug and to provide adequate information for the labeling of the drug.

After completion of the required clinical testing, an NDA is prepared and submitted to the FDA. FDA approval of the NDA is required before marketing of the product may begin in the U.S. The NDA must include the results of all preclinical, clinical and other testing and a compilation of data relating to the product's pharmacology, chemistry, manufacture, and controls. The cost of preparing and submitting an NDA is substantial. Under federal law, the submission of most NDAs is additionally subject to a substantial application user fee, and the holder of an approved NDA is also subject to annual product and establishment user fees. These fees are typically increased annually.

The FDA has 60 days from its receipt of a NDA to determine whether the application will be accepted for filing based on the agency's threshold determination that it is sufficiently complete to permit substantive review. Once the submission is accepted for filing, the FDA begins an in-depth review. The FDA has agreed to certain performance goals in the review of new drug applications. Most such applications for non-priority drug products are reviewed within ten months. The review process may be extended by FDA for three additional months to evaluate major amendments to information already provided in the initial submission. The FDA may also refer applications for novel drug products or drug products which present difficult questions of safety or efficacy to an advisory committee, typically a panel that includes clinicians and other experts, for review, evaluation and a recommendation as to whether the application should be approved. The FDA is not bound by the recommendation of an advisory committee, but it generally follows such recommendations. Before approving an NDA, the FDA will typically inspect one or more clinical sites to assure compliance with GCP. Additionally, the FDA will inspect the facility or the facilities at which the drug is manufactured. FDA will not approve the product unless compliance with current good manufacturing practices is satisfactory and the NDA contains data that provide substantial evidence that the drug is safe and effective in the indication studied.

After FDA evaluates the NDA and the manufacturing facilities, it issues an approval letter or a complete response letter. Complete response letters outline the deficiencies in the submission and may require substantial additional testing or information in order for the FDA to reconsider the application. If and when those deficiencies have been addressed to the FDA's satisfaction in an amendment submitted to the NDA, the FDA will issue an approval letter. FDA has committed to reviewing such resubmissions in 2 or 6 months depending on the type of information included.

An approval letter authorizes commercial marketing of the drug with specific prescribing information for specific indications. As a condition of NDA approval, the FDA may require substantial post-approval testing and surveillance to monitor the drug's safety or efficacy and may impose other conditions, including labeling restrictions which can materially affect the potential market and profitability of the drug. Once granted, product approvals may be withdrawn if compliance with regulatory standards is not maintained or problems are identified following initial marketing.

### ***The Hatch-Waxman Act***

In seeking approval for a drug through an NDA, applicants are required to list with the FDA each patent with claims that cover the applicant's product. Upon approval of a drug, each of the patents listed in the application for the drug is then published in the FDA's Approved Drug Products with Therapeutic Equivalence Evaluations, commonly known as the Orange Book. Drugs listed in the Orange Book can, in turn, be cited by potential competitors in support of approval of an abbreviated new drug application (ANDA). An ANDA provides for marketing of a drug product that has the same active ingredients in the same strengths and dosage form as the listed drug and has been shown through bioequivalence testing to be therapeutically equivalent to the listed drug. ANDA applicants are not required to conduct or submit results of preclinical or clinical tests to prove the safety or effectiveness of their drug product, other than the requirement for bioequivalence testing. Drugs approved in this way are commonly referred to as "generic equivalents" to the listed drug, and can often be substituted by pharmacists under prescriptions written for the original listed drug.



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The ANDA applicant is required to certify to the FDA concerning any patents listed for the approved product in the FDA's Orange Book. Specifically, the applicant must certify that: (i) the required patent information has not been filed; (ii) the listed patent has expired; (iii) the listed patent has not expired, but will expire on a particular date and approval is sought after patent expiration; or (iv) the listed patent is invalid or will not be infringed by the new product. A certification that the new product will not infringe the already approved product's listed patents or that such patents are invalid is called a Paragraph 4 certification. If the applicant does not challenge the listed patents, the ANDA application will not be approved until all the listed patents claiming the referenced product have expired.

If the ANDA applicant has provided a Paragraph 4 certification to the FDA, the applicant must also send notice of the Paragraph 4 certification to the NDA and patent holders once the ANDA has been accepted for filing by the FDA. The NDA and patent holders may then initiate a patent infringement lawsuit in response to the notice of the Paragraph 4 certification. The filing of a patent infringement lawsuit within 45 days of the receipt of a Paragraph 4 certification automatically prevents the FDA from approving the ANDA until the earlier of 30 months, expiration of the patent, settlement of the lawsuit or a decision in the infringement case that is favorable to the ANDA applicant.

The ANDA application also will not be approved until any non-patent exclusivity, such as exclusivity for obtaining approval of a new chemical entity, listed in the Orange Book for the referenced product has expired (New Chemical Entity Market Exclusivity). Federal law provides a period of five years following approval of a drug containing no previously approved active ingredients, during which ANDAs for generic versions of those drugs cannot be submitted unless the submission contains a Paragraph 4 challenge to a listed patent, in which case the submission may be made four years following the original product approval. Federal law provides for a period of three years of exclusivity following approval of a listed drug that contains previously approved active ingredients but is approved in a new dosage form, route of administration or combination, or for a new use, the approval of which was required to be supported by new clinical trials conducted by or for the sponsor, during which FDA cannot grant effective approval of an ANDA based on that listed drug for the same new dosage form, route of administration or combination, or new use.

### ***Other Regulatory Requirements***

Once an NDA is approved, a product will be subject to certain post-approval requirements. For instance, FDA closely regulates the post-approval marketing and promotion of drugs, including standards and regulations for direct-to-consumer advertising, off-label promotion, industry-sponsored scientific and educational activities and promotional activities involving the internet.

Drugs may be marketed only for the approved indications and in accordance with the provisions of the approved labeling. Changes to some of the conditions established in an approved application, including changes in indications, labeling, or manufacturing processes or facilities, require submission and FDA approval of a new NDA or NDA supplement before the change can be implemented. An NDA supplement for a new indication typically requires clinical data similar to that in the original application, and the FDA uses the same procedures and actions in reviewing NDA supplements as it does in reviewing NDAs.

Adverse event reporting and submission of periodic reports is required following FDA approval of an NDA. The FDA also may require post-marketing testing, known as Phase 4 testing, risk evaluation and mitigation strategies and surveillance to monitor the effects of an approved product, or place conditions on an approval that could restrict the distribution or use of the product. In addition, quality control as well as drug manufacture, packaging, and labeling procedures must continue to conform to current good manufacturing practices, or cGMPs, after approval. Drug manufacturers and certain of their subcontractors are required to register their establishments with FDA and certain state agencies, and are subject to routine inspections by the FDA during which the agency inspects manufacturing facilities to access compliance with cGMPs. Accordingly, manufacturers must continue to expend time, money and effort in the areas of production and quality control to maintain compliance with cGMPs. Regulatory authorities may withdraw product approvals or request product recalls if a company fails to comply with regulatory standards, if it encounters problems following initial marketing, or if previously unrecognized problems are subsequently discovered.

### ***Orphan Drugs***

Under the Orphan Drug Act, the FDA may grant orphan drug designation to drugs intended to treat a rare disease or condition, which is generally a disease or condition that affects fewer than 200,000 individuals in the U.S. Orphan drug designation must be requested before submitting an NDA. After the FDA grants orphan drug designation, the generic identity of the drug and its potential orphan use are disclosed publicly by the FDA. Orphan drug designation does not convey any advantage in or shorten the duration of the regulatory review and approval process. The first NDA applicant with FDA orphan drug designation for a particular active ingredient to receive FDA approval of the designated drug for the disease indication for which it has such designation, is entitled to a seven-year exclusive marketing period (Orphan Drug Exclusivity) in the U.S. for that product, for that indication. During the seven-year period, the FDA may not finally approve any other applications to market the same drug for the same disease, except in limited circumstances, such as a showing of clinical superiority to the product with orphan drug exclusivity or if the license holder cannot supply sufficient quantities of the product. Orphan drug exclusivity does not prevent FDA from approving a different drug for the same disease or condition, or the same drug for a different disease or condition. Among the other benefits of orphan drug designation are tax credits for certain research and a waiver of the NDA application user fee.



***Pediatric Information***

Under the Pediatric Research Equity Act of 2007 (PREA), NDAs or supplements to NDAs must contain data to assess the safety and effectiveness of the drug for the claimed indications in all relevant pediatric subpopulations and to support dosing and administration for each pediatric subpopulation for which the drug is safe and effective. The FDA may grant deferrals for submission of data or full or partial waivers. Unless otherwise required by regulation, PREA does not apply to any drug for an indication for which orphan designation has been granted.

***Fast Track Designation***

Under the fast track program, the sponsor of an IND may request FDA to designate the drug candidate as a fast track drug if it is intended to treat a serious condition and fulfill an unmet medical need. FDA must determine if the drug candidate qualifies for fast track designation within 60 days of receipt of the sponsor's request. Once FDA designates a drug as a fast track candidate, it is required to facilitate the development and expedite the review of that drug by providing more frequent communication with and guidance to the sponsor.

In addition to other benefits such as the ability to use surrogate endpoints and have greater interactions with FDA, FDA may initiate review of sections of a fast track drug's NDA before the application is complete. This rolling review is available if the applicant provides and FDA approves a schedule for the submission of the remaining information and the applicant pays applicable user fees. However, FDA's time period goal for reviewing an application does not begin until the last section of the NDA is submitted. Additionally, the fast track designation may be withdrawn by FDA if FDA believes that the designation is no longer supported by data emerging in the clinical trial process.

***Priority Review***

Under FDA policies, a drug candidate is eligible for priority review, or review within a six-month time frame from the time a complete NDA is accepted for filing, if the drug candidate provides a significant improvement compared to marketed drugs in the treatment, diagnosis or prevention of a disease. A fast track designated drug candidate would ordinarily meet FDA's criteria for priority review.

***Accelerated Approval***

Under FDA's accelerated approval regulations, FDA may approve a drug for a serious or life-threatening illness that provides meaningful therapeutic benefit to patients over existing treatments based upon a surrogate endpoint that is reasonably likely to predict clinical benefit. In clinical trials, a surrogate endpoint is a measurement of laboratory or clinical signs of a disease or condition that substitutes for a direct measurement of how a patient feels, functions, or survives. Surrogate endpoints can often be measured more easily or more rapidly than clinical endpoints. A drug candidate approved on this basis is subject to rigorous post-marketing compliance requirements, including the completion of Phase 4 or post-approval clinical trials to confirm the effect on the clinical endpoint. Failure to conduct required post-approval studies, or confirm a clinical benefit during post-marketing studies, will allow FDA to withdraw the drug from the market on an expedited basis. All promotional materials for drug candidates approved under accelerated regulations are subject to prior review by FDA.

***Section 505(b)(2) New Drug Applications***

Most drug products obtain FDA marketing approval pursuant to an NDA or an ANDA. A third alternative is a special type of NDA, commonly referred to as a Section 505(b)(2) NDA, which enables the applicant to rely, in part, on the safety and efficacy data of an existing product, or published literature, in support of its application.

505(b)(2) NDAs often provide an alternate path to FDA approval for new or improved formulations or new uses of previously approved products. Section 505(b)(2) permits the submission of a NDA where at least some of the information required for approval comes from studies not conducted by or for the applicant and for which the applicant has not obtained a right of reference. The applicant may rely upon certain preclinical or clinical studies conducted for an approved product. The FDA may also require companies to perform additional studies or measurements to support the change from the approved product. The FDA may then approve the new product candidate for all or some of the label indications for which the referenced product has been approved, as well as for any new indication sought by the Section 505(b)(2) applicant.

To the extent that the Section 505(b)(2) applicant is relying on studies conducted for an already approved product, the applicant is required to certify to the FDA concerning any patents listed for the approved product in the Orange Book to the same extent that an ANDA applicant would. Thus approval of a 505(b)(2) NDA can be stalled until all the listed patents claiming the referenced product have expired, until any non-patent exclusivity, such as exclusivity for obtaining approval of a new chemical entity, listed in the Orange Book for the referenced product has expired, and, in the case of a Paragraph 4 certification and subsequent patent infringement suit, until the earlier of 30 months, settlement of the lawsuit or a decision in the infringement case that is favorable to the Section 505 (b)(2) applicant.

### ***Anti-Kickback, False Claims Laws & The Prescription Drug Marketing Act***

In addition to FDA restrictions on marketing of pharmaceutical products, several other types of state and federal laws have been applied to restrict certain marketing practices in the pharmaceutical industry in recent years. These laws include anti-kickback statutes and false claims statutes. The federal healthcare program anti-kickback statute prohibits, among other things, knowingly and willfully offering, paying, soliciting or receiving remuneration to induce or in return for purchasing, leasing, ordering or arranging for the purchase, lease or order of any healthcare item or service reimbursable under Medicare, Medicaid or other federally financed healthcare programs. This statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on the one hand and prescribers, purchasers and formulary managers on the other. Violations of the anti-kickback statute are punishable by imprisonment, criminal fines, civil monetary penalties and exclusion from participation in federal healthcare programs. Although there are a number of statutory exemptions and regulatory safe harbors protecting certain common activities from prosecution or other regulatory sanctions, the exemptions and safe harbors are drawn narrowly, and practices that involve remuneration intended to induce prescribing, purchases or recommendations may be subject to scrutiny if they do not qualify for an exemption or safe harbor.

Federal false claims laws prohibit any person from knowingly presenting, or causing to be presented, a false claim for payment to the federal government, or knowingly making, or causing to be made, a false statement to have a false claim paid. Recently, several pharmaceutical and other healthcare companies have been prosecuted under these laws for allegedly inflating drug prices they report to pricing services, which in turn were used by the government to set Medicare and Medicaid reimbursement rates, and for allegedly providing free product to customers with the expectation that the customers would bill federal programs for the product. In addition, certain marketing practices, including off-label promotion, may also violate false claims laws. The majority of states also have statutes or regulations similar to the federal anti-kickback law and false claims laws, which apply to items and services reimbursed under Medicaid and other state programs, or, in several states, apply regardless of the payor.

### ***Physician Drug Samples***

As part of the sales and marketing process, pharmaceutical companies frequently provide samples of approved drugs to physicians. The Prescription Drug Marketing Act (the PDMA) imposes requirements and limitations upon the provision of drug samples to physicians, as well as prohibits states from licensing distributors of prescription drugs unless the state licensing program meets certain federal guidelines that include minimum standards for storage, handling and record keeping. In addition, the PDMA sets forth civil and criminal penalties for violations.

### ***Regulation Outside the U.S.***

In addition to regulations in the U.S., we will be subject to a variety of regulations in other jurisdictions governing clinical studies and commercial sales and distribution of our products. Most countries outside the U.S. require that clinical trial applications be submitted to and approved by the local regulatory authority for each clinical study. In addition, whether or not we obtain FDA approval for a product, we must obtain approval of a product by the comparable regulatory authorities of countries outside the U.S. before we can commence clinical studies or marketing of the product in those countries. The approval process varies from country to country, and the time may be longer or shorter than that required for FDA approval.

To obtain regulatory approval of a drug under EU regulatory systems, we may submit marketing authorizations either under a centralized or decentralized procedure. The centralized procedure, which is compulsory for medicines produced by certain biotechnological processes and optional for those which are highly innovative, provides for the grant of a single marketing authorization that is valid for all EU member states. The decentralized procedure provides for approval by one or more other, or concerned, member states of an assessment of an application performed by one member state, known as the reference member state. Under this procedure, an applicant submits an application, or dossier, and related materials including a draft summary of product characteristics, and draft labeling and package leaflet, to the reference member state and concerned member states. The reference member state prepares a draft assessment and drafts of the related materials within 120 days after receipt of a valid application. Within 90 days of receiving the reference member state's assessment report, each concerned member state must decide whether to approve the assessment report and related materials. If a member state cannot approve the assessment report and related materials on the grounds of potential serious risk to the public health, the disputed points may eventually be referred to the European Commission, whose decision is binding on all member states.



We have obtained an orphan medicinal product designation in the EU from the EMEA for Amigal for the treatment of Fabry disease and for Plicera for the treatment of Gaucher disease. We anticipate filing for orphan medicinal product designation from the EMEA for AT2220 for the treatment of Pompe disease. The EMEA grants orphan drug designation to promote the development of products that may offer therapeutic benefits for life-threatening or chronically debilitating conditions affecting not more than five in 10,000 people in the EU. In addition, orphan drug designation can be granted if the drug is intended for a life threatening, seriously debilitating or serious and chronic condition in the EU and that without incentives it is unlikely that sales of the drug in the EU would be sufficient to justify developing the drug. Orphan drug designation is only available if there is no other satisfactory method approved in the EU of diagnosing, preventing or treating the condition, or if such a method exists, the proposed orphan drug will be of significant benefit to patients.

Orphan drug designation provides opportunities for free protocol assistance and fee reductions for access to the centralized regulatory procedures before and during the first year after marketing approval, which reductions are not limited to the first year after marketing approval for small and medium enterprises. In addition, if a product which has an orphan drug designation subsequently receives EMEA marketing approval for the indication for which it has such designation, the product is entitled to orphan drug exclusivity, which means the EMEA may not approve any other application to market the same drug for the same indication for a period of ten years. The exclusivity period may be reduced to six years if the designation criteria are no longer met, including where it is shown that the product is sufficiently profitable not to justify maintenance of market exclusivity. Competitors may receive marketing approval of different drugs or biologics for the indications for which the orphan product has exclusivity. In order to do so, however, they must demonstrate that the new drugs or biologics provide a significant benefit over the existing orphan product. This demonstration of significant benefit may be done at the time of initial approval or in post-approval studies, depending on the type of marketing authorization granted.

As described in the section entitled “Amigal for Fabry Disease — Existing Products for the Treatment of Fabry Disease and Potential Advantages of Amigal,” we believe that the orphan designation of Fabrazyme<sup>®</sup> and Replagal<sup>®</sup> in the EU will not prevent us from obtaining marketing approval of Amigal in the EU for the treatment of Fabry disease because Amigal will provide significant benefits over Fabrazyme<sup>®</sup> and Replagal<sup>®</sup>.

### **Pharmaceutical Pricing and Reimbursement**

In the U.S. and markets in other countries, sales of any products for which we receive regulatory approval for commercial sale will depend in part on the availability of reimbursement from third party payors. Third party payors include government health administrative authorities, managed care providers, private health insurers and other organizations. These third party payors are increasingly challenging the price and examining the cost-effectiveness of medical products and services. In addition, significant uncertainty exists as to the reimbursement status of newly approved healthcare product candidates. We may need to conduct expensive pharmacoeconomic studies in order to demonstrate the cost-effectiveness of our products. Our product candidates may not be considered cost-effective. Adequate third party reimbursement may not be available to enable us to maintain price levels sufficient to realize an appropriate return on our investment in product development.

In 2003, the U.S. government enacted legislation providing a partial prescription drug benefit for Medicare recipients that began in 2006. Government payment for some of the costs of prescription drugs may increase demand for any products for which we receive marketing approval. However, to obtain payments under this program, we would be required to sell products to Medicare recipients through managed care organizations and other health care delivery systems operating pursuant to this legislation. These organizations would negotiate prices for our products, which are likely to be lower than we might otherwise obtain. Federal, state and local governments in the U.S. continue to consider legislation to limit the growth of healthcare costs, including the cost of prescription drugs. Future legislation could limit payments for pharmaceuticals such as the drug candidates that we are developing.

The marketability of any products for which we receive regulatory approval for commercial sale may suffer if the government and third party payors fail to provide adequate coverage and reimbursement. In addition, an increasing emphasis on managed care in the U.S. has increased and will continue to increase the pressure on pharmaceutical pricing.

### **Employees**

As of December 31, 2010, we had 99 full-time employees, 75 of whom were primarily engaged in research and development activities and 24 of whom provide administrative services. A total of 26 employees have an M.D. or Ph.D. degree. None of our employees are represented by a labor union. We have not experienced any work stoppages and consider our employee relations to be good.

**Our Corporate Information**

We were incorporated under the laws of the State of Delaware on February 4, 2002. Our principal executive offices are located at 6 Cedar Brook Drive, Cranbury, NJ 08512 and our telephone number is (609) 662-2000. Our website address is [www.amicustherapeutics.com](http://www.amicustherapeutics.com). We make available free of charge on our website our annual, quarterly and current reports, including amendments to such reports, as soon as reasonably practicable after we electronically file such material with, or furnish such material to, the U.S. Securities and Exchange Commission.

Information relating to corporate governance at Amicus Therapeutics, including our Code of Business Conduct for Employees, Executive Officers and Directors, Corporate Governance Guidelines, and information concerning our senior management team, Board of Directors, including Board Committees and Committee charters, and transactions in our securities by directors and executive officers, is available on our website at [www.amicustherapeutics.com](http://www.amicustherapeutics.com) under the “Investors—Corporate Governance” caption and in print to any stockholder upon request. Any waivers to the Codes by directors or executive officers and any material amendment to the Code of Business Conduct and Ethics for Employees, Executive Officers and Directors will be posted promptly on our website.

We have filed applications to register certain trademarks in the U.S. and abroad, including AMICUS™, AMICUS THERAPEUTICS™ and design, AMIGAL™ and PLICERA™. We plan to seek FDA approval of the trademarks Amigal and Plicera for migalastat hydrochloride and isofagomine tartrate, respectively. Fabrazyme®, Cerezyme®, Myozyme®, Replagal®, VPRIV® and Zavesca® are the property of their respective owners.

## ITEM 1A. RISK FACTORS

*The occurrence of any of the following risks could harm our business, financial condition, results of operations and/or growth prospects. In that case, the trading price of our common stock could decline, and you may lose all or part of your investment. Additional risks and uncertainties not presently known to the Company; or risks that the Company currently considers immaterial, may also impair the Company's operations.*

### **Risks Related to Our Financial Position and Need for Additional Capital**

**We have incurred significant operating losses since our inception. We currently do not, and since inception never have had, any products available for commercial sale. We expect to incur operating losses for the foreseeable future and may never achieve or maintain profitability.**

Since inception, we have incurred significant operating losses. Our cumulative net loss attributable to common stockholders since inception was \$245.9 million and we had an accumulated deficit of \$225.7 million as of December 31, 2010. To date, we have financed our operations primarily through private placements of our redeemable convertible preferred stock, proceeds from our initial public offering and March 2010 registered direct offering, and from our collaboration agreement with GSK and prior collaboration agreement with Shire. We have devoted substantially all of our efforts to research and development, including our preclinical development activities and clinical trials. We have not completed development of any drugs. We expect to continue to incur significant and increasing operating losses for at least the next several years and we are unable to predict the extent of any future losses as we:

- continue our ongoing Phase 3 clinical trials of Amigal (migalastat hydrochloride) for the treatment of Fabry disease to support approval in the United States (Study 011) and in the European Union (Study 012);
- continue our ongoing Phase 2 clinical trial of Amigal co-administered with ERT for Fabry disease and potentially initiate a Phase 2 clinical trial of AT2220 co-administered with ERT for Pompe disease;
- continue our preclinical studies on the use of pharmacological chaperones for the treatment of diseases of neurodegeneration, including Parkinson's Disease and Alzheimer's Disease;
- continue our preclinical studies on the use of pharmacological chaperones co-administered with ERT;
- continue the research and development of additional product candidates;
- seek regulatory approvals for our product candidates that successfully complete clinical trials; and
- establish a sales and marketing infrastructure to commercialize products for which we may obtain regulatory approval.

To become and remain profitable, we must succeed in developing and commercializing drugs with significant market potential. This will require us to be successful in a range of challenging activities, including the discovery of product candidates, successful completion of preclinical testing and clinical trials of our product candidates, obtaining regulatory approval for these product candidates and manufacturing, marketing and selling those products for which we may obtain regulatory approval. We are only in the preliminary stages of these activities. We may never succeed in these activities and may never generate revenues that are large enough to achieve profitability. Even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become or remain profitable could depress the market price of our common stock and could impair our ability to raise capital, expand our business, diversify our product offerings or continue our operations.

**We will need substantial funding and may be unable to raise capital when needed, which would force us to delay, reduce or eliminate our product development programs or commercialization efforts.**

We expect to continue to incur substantial research and development expenses in connection with our ongoing activities, particularly as we continue our Phase 3 development of Amigal. Further, subject to obtaining regulatory approval of any of our product candidates besides Amigal, we expect to incur significant commercialization expenses for product sales and marketing, securing commercial quantities of product from our manufacturers and product distribution. While research and development costs associated with our Amigal program will be shared with GSK so long as our collaboration continues, we remain responsible for all costs related to our other programs.

We believe that our existing cash and cash equivalents and marketable securities, along with reimbursements of development costs and achievement of milestones under our collaboration with GSK, will be sufficient to enable us to fund our operating expenses and capital expenditure requirements through the anticipated commercial launch of Amigal in the United States. However, should GSK terminate our collaboration agreement, we may need to seek additional funding in order to complete any clinical trials related to Amigal, seek regulatory approvals of Amigal, and launch Amigal and continue our other clinical and preclinical programs. Capital may not be available when needed on terms that are acceptable to us, or at all, especially in light of the current challenging economic environment. If adequate funds are not available to us on a timely basis, we may be required to reduce or eliminate research development programs or commercial efforts.

Our future capital requirements will depend on many factors, including:

- the progress and results of our clinical trials of Amigal;
- the continuation of, and our achievement of milestone payments under, our collaboration agreement with GSK;
- the scope, progress, results and costs of preclinical development, laboratory testing and clinical trials for our other product candidates including those testing the use of pharmacological chaperones co-administered with ERT and for the treatment of diseases of neurodegeneration;
- the costs, timing and outcome of regulatory review of our product candidates;
- the number and development requirements of other product candidates that we pursue;
- the costs of commercialization activities, including product marketing, sales and distribution;
- the emergence of competing technologies and other adverse market developments;
- the costs of preparing, filing and prosecuting patent applications and maintaining, enforcing and defending intellectual property related claims;
- the extent to which we acquire or invest in businesses, products and technologies; and
- our ability to establish additional collaborations and obtain milestone, royalty or other payments from any such collaborators.

**Any capital that we obtain may not be on terms favorable to us or our stockholders or may require us to relinquish valuable rights.**

Until such time, if ever, as we generate product revenue to finance our operations, we expect to finance our cash needs through public or private equity offerings and debt financings, corporate collaboration and licensing arrangements and grants from patient advocacy groups, foundations and government agencies. If we are able to raise capital by issuing equity securities, as we did in March 2010, our stockholders will experience dilution. In addition, stockholders may experience dilution if the holders of the warrants issued in connection with our March 2010 offering exercise their warrants. Debt financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends and may include rights that are senior to the holders of our common stock. Any debt financing or additional equity that we raise may contain terms, such as liquidation and other preferences, which are not favorable to us or our stockholders. If we raise capital through additional collaboration and licensing arrangements with third parties, it may be necessary to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or to grant licenses on terms that may not be favorable to us or our stockholders.

**Our short operating history may make it difficult to evaluate the success of our business to date and to assess our future viability.**

We are a development stage company. We commenced operations in February 2002. Our operations to date have been limited to organizing and staffing our company, acquiring and developing our technology and undertaking preclinical studies and clinical trials of our most advanced product candidates. We have not yet generated any commercial sales for any of our product candidates. We have not yet demonstrated our ability to successfully complete large-scale, clinical trials, obtain regulatory approvals, manufacture a commercial-scale product or arrange for a third party to do so on our behalf, or conduct sales and marketing activities necessary for successful product commercialization. Consequently, any predictions about our future success or viability may not be as accurate as they could be if we had a longer operating history.

In addition, as a new business, we may encounter unforeseen expenses, difficulties, complications, delays and other known and unknown factors. If we are successful in obtaining marketing approval for any of our lead product candidates, we will need to transition from a company with a research focus to a company capable of supporting commercial activities. We may not be successful in such a transition.

### **Risks Related to the Development and Commercialization of Our Product Candidates**

**We depend heavily on the success of our most advanced product candidate, Amigal. All of our product candidates are still in either preclinical or clinical development. Clinical trials of our product candidates may not be successful. If we are unable to commercialize Amigal, or experience significant delays in doing so, our business will be materially harmed.**

We have invested a significant portion of our efforts and financial resources in the development of our most advanced product candidates, including Amigal. Our ability to generate product revenue, which may never occur, will depend heavily on the successful development and commercialization of these product candidates. The successful commercialization of our product candidates will depend on several factors, including the following:

- successful enrollment of patients in our clinical trials on a timely basis;
- obtaining supplies of product candidates for completion of our clinical trials on a timely basis;
- successful completion of preclinical studies and clinical trials;
- obtaining regulatory agreement in the structure and design of our clinical programs;
- obtaining marketing approvals from the United States Food and Drug Administration (FDA), and similar regulatory authorities outside the U.S.;
- establishing commercial-scale manufacturing arrangements with third party manufacturers whose manufacturing facilities are operated in compliance with current good manufacturing practice (cGMP) regulations;
- launching commercial sales of the product, whether alone or in collaboration with others;
- acceptance of the product by patients, the medical community and third party payors;
- competition from other companies and their therapies;
- successful protection of our intellectual property rights from competing products in the U.S. and abroad; and
- a continued acceptable safety and efficacy profile of our product candidates following approval.

**If the market opportunities for our product candidates are smaller than we believe they are, then our revenues may be adversely affected and our business may suffer.**

Each of the diseases that our most advanced product candidates are being developed to address is rare. Our projections of both the number of people who have these diseases, as well as the subset of people with these diseases who have the potential to benefit from treatment with our product candidates, are based on estimates.

Currently, most reported estimates of the prevalence of these diseases are based on studies of small subsets of the population of specific geographic areas, which are then extrapolated to estimate the prevalence of the diseases in the broader world population. In addition, as new studies are performed the estimated prevalence of these diseases may change. In fact, as a result of some recent studies, we believe that previously reported studies do not accurately account for the prevalence of Fabry disease and that the prevalence of Fabry disease could be many times higher than previously reported. There can be no assurance that the prevalence of Fabry disease or Pompe disease in the study populations, particularly in these newer studies, accurately reflects the prevalence of these diseases in the broader world population.

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We estimate the number of potential patients in the broader world population who have those diseases and may respond to treatment with our product candidates by further extrapolating estimates of the prevalence of specific types of genetic mutations giving rise to these diseases. For example, we base our estimate of the percentage of Fabry patients who may respond to treatment with Amigal on the frequency of missense and other similar mutations that cause Fabry disease reported in the Human Gene Mutation Database. As a result of recent studies that estimate that the prevalence of Fabry disease could be many times higher than previously reported, we believe that the number of patients diagnosed with Fabry disease will increase and estimate that the number of Fabry patients who may benefit from the use of Amigal is significantly higher than some previously reported estimates of Fabry disease generally. If our estimates of the prevalence of Fabry disease or of the number of patients who may benefit from treatment with our product candidates prove to be incorrect, the market opportunities for our product candidates may be smaller than we believe they are, our prospects for generating revenue may be adversely affected and our business may suffer.

### **Initial results from a clinical trial do not ensure that the trial will be successful and success in early stage clinical trials does not ensure success in later-stage clinical trials.**

We will only obtain regulatory approval to commercialize a product candidate if we can demonstrate to the satisfaction of the FDA or the applicable non-U.S. regulatory authority, in well-designed and conducted clinical trials, that the product candidate is safe and effective and otherwise meets the appropriate standards required for approval for a particular indication. Clinical trials are lengthy, complex and extremely expensive processes with uncertain results. A failure of one or more of our clinical trials may occur at any stage of testing. We have limited experience in conducting and managing the clinical trials necessary to obtain regulatory approvals, including approval by the FDA.

Success in preclinical testing and early clinical trials does not ensure that later clinical trials will be successful, and initial results from a clinical trial do not necessarily predict final results. We cannot be assured that these trials will ultimately be successful. In addition, patients may not be compliant with their dosing regimen or trial protocols or they may withdraw from the study at any time for any reason.

Even if our early stage clinical trials are successful, we will need to conduct additional clinical trials with larger numbers of patients receiving the drug for longer periods for all of our product candidates before we are able to seek approvals to market and sell these product candidates from the FDA and regulatory authorities outside the U.S. In addition, each of our product candidates is based on our pharmacological chaperone technology. To date, we are not aware that any product based on chaperone technology has been approved by the FDA. As a result, while we have reached agreement with the FDA on the use of a surrogate primary endpoint in our Phase 3 study for Amigal, we cannot be sure what endpoints the FDA will require us to measure in later-stage clinical trials of our other product candidates. If the FDA requires different endpoints than the endpoints we anticipate using, it may be more difficult for us to obtain, or we may be delayed in obtaining, FDA approval of our product candidates. If we are not successful in commercializing any of our lead product candidates, or are significantly delayed in doing so, our business will be materially harmed.

### **We have limited experience in conducting and managing the preclinical development activities and clinical trials necessary to obtain regulatory approvals, including approval by the FDA.**

We have limited experience in conducting and managing the preclinical development activities and clinical trials necessary to obtain regulatory approvals, including approval by the FDA. We have not obtained regulatory approval nor commercialized any of our product candidates. We are currently conducting Phase 3 clinical trials for Amigal and a Phase 2 clinical trial for the co-administration of Amigal with ERT but have not yet completed a Phase 3 clinical trial for any of our product candidates. We have on-going preclinical studies on the use of pharmacological chaperones co-administered with ERT in Gaucher and Pompe and on the use of pharmacological chaperones to treat diseases of neurodegeneration. Our limited experience might prevent us from successfully designing or implementing a clinical trial. We have limited experience in conducting and managing the application process necessary to obtain regulatory approvals and we might not be able to demonstrate that our product candidates meet the appropriate standards for regulatory approval. If we are not successful in conducting and managing our preclinical development activities or clinical trials or obtaining regulatory approvals, we might not be able to commercialize our lead product candidates, or might be significantly delayed in doing so, which will materially harm our business.

### **We may find it difficult to enroll patients in our clinical trials.**

Each of the diseases that our lead product candidates are intended to treat is relatively rare and we expect only a subset of the patients with these diseases to be eligible for our clinical trials. For example, the entry criteria for our ongoing Phase 3 study in Amigal for Fabry disease to support approval in the United States requires that patients must have a genetic mutation that we believe is responsive to Amigal, and may not have received enzyme replacement therapy in the past or must have stopped treatment for at least six months prior to enrolling in the study. We may not be able to initiate or continue clinical trials for each or all of our product candidates if we are unable to locate a sufficient number of eligible patients to participate in the clinical trials required by the FDA or other non-U.S. regulatory agencies. The requirements of our clinical testing mandate that a patient cannot be involved in another clinical trial for the same indication. We are aware that our competitors have ongoing clinical trials for products that are competitive with our product candidates and patients who would otherwise be eligible for our clinical trials may be involved in such testing, rendering them unavailable for testing of our product candidates. Further, if we are required to include patients in our clinical trials who have never received enzyme replacement therapy, we may experience yet further difficulty and delay enrolling patients in our trials. Our inability to enroll a sufficient number of patients for any of our current or future clinical trials would result in significant delays or may require us to abandon one or more clinical trials altogether.



**If our preclinical studies do not produce positive results, if our clinical trials are delayed or if serious side effects are identified during drug development, we may experience delays, incur additional costs and ultimately be unable to commercialize our product candidates.**

Before obtaining regulatory approval for the sale of our product candidates, we must conduct, at our own expense, extensive preclinical tests to demonstrate the safety of our product candidates in animals, and clinical trials to demonstrate the safety and efficacy of our product candidates in humans. Preclinical and clinical testing is expensive, difficult to design and implement and can take many years to complete. A failure of one or more of our preclinical studies or clinical trials can occur at any stage of testing. We may experience numerous unforeseen events during, or as a result of, preclinical testing and the clinical trial process that could delay or prevent our ability to obtain regulatory approval or commercialize our product candidates, including:

- our preclinical tests or clinical trials may produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional preclinical testing or clinical trials or we may abandon projects that we expect to be promising;
- we may decide to amend existing protocols for on-going clinical trials;
- regulators or institutional review boards may not authorize us to commence a clinical trial or conduct a clinical trial at a prospective trial site;
- conditions imposed on us by the FDA or any non-U.S. regulatory authority regarding the scope or design of our clinical trials may require us to resubmit our clinical trial protocols to institutional review boards for re-inspection due to changes in the regulatory environment;
- the number of patients required for our clinical trials may be larger than we anticipate or participants may drop out of our clinical trials at a higher rate than we anticipate;
- our third party contractors or clinical investigators may fail to comply with regulatory requirements or fail to meet their contractual obligations to us in a timely manner;
- we might have to suspend or terminate one or more of our clinical trials if we, the regulators or the institutional review boards determine that the participants are being exposed to unacceptable health risks;
- regulators or institutional review boards may require that we hold, suspend or terminate clinical research for various reasons, including noncompliance with regulatory requirements;
- the cost of our clinical trials may be greater than we anticipate;
- the supply or quality of our product candidates or other materials necessary to conduct our clinical trials, such as existing treatments like ERT, may be insufficient or inadequate or we may not be able to reach agreements on acceptable terms with prospective clinical research organizations;
- a continued shortage in the supply of ERT, which we require to conduct Study 012 and may be required for future studies; and
- the effects of our product candidates may not be the desired effects or may include undesirable side effects or the product candidates may have other unexpected characteristics.

If we are required to conduct additional clinical trials or other testing of our product candidates beyond those that we currently contemplate, if we are unable to successfully complete our clinical trials or other testing, if the results of these trials or tests are not positive or are only modestly positive or if there are safety concerns, we may:

- be delayed in obtaining, or may not be able to obtain, marketing approval for one or more of our product candidates and milestone payments from our collaborators;
- obtain approval for indications that are not as broad as intended or entirely different than those indications for which we sought approval; or
- have the product removed from the market after obtaining marketing approval.

Our product development costs will also increase if we experience delays in testing or approvals. We do not know whether any preclinical tests or clinical trials will be initiated as planned, will need to be restructured or will be completed on schedule, if at all. Significant preclinical or clinical trial delays also could shorten the patent protection period during which we may have the exclusive right to commercialize our product candidates. Such delays could allow our competitors to bring products to market before we do and impair our ability to commercialize our products or product candidates. In addition, GSK has significant influence on the conduct of our Amigal program, and could compel us to perform unanticipated clinical trials of Amigal or delay the approval process for a variety of reasons.

**The commercial success of any product candidates that we may develop, including Amigal, will depend upon the degree of market acceptance by physicians, patients, third party payors and others in the medical community.**

Any products that we bring to the market, including Amigal, may not gain market acceptance by physicians, patients, third party payors and others in the medical community. If these products do not achieve an adequate level of acceptance, we may not generate significant product revenue and we may not become profitable. The degree of market acceptance of our product candidates, if approved for commercial sale, will depend on a number of factors, including:

- the prevalence and severity of any side effects, including any limitations or warnings contained in a product's approved labeling;
- the efficacy and potential advantages over alternative treatments;
- the pricing of our product candidates;
- relative convenience and ease of administration;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- the strength of marketing and distribution support, which in the case of Amigal will be the responsibility of our collaborator, GSK, and timing of market introduction of competitive products;
- publicity concerning our products or competing products and treatments; and
- sufficient third party insurance coverage or reimbursement.

Even if a potential product displays a favorable efficacy and safety profile in preclinical and clinical trials, market acceptance of the product will not be known until after it is launched. Our efforts to educate the medical community and third party payors on the benefits of our product candidates may require significant resources and may never be successful. In the case of Amigal, we will be relying in large part on the efforts of our collaborator, GSK for such efforts. Such efforts to educate the marketplace may require more resources than are required by the conventional technologies marketed by our competitors.

**If we are unable to obtain adequate reimbursement from governments or third party payors for any products that we may develop or if we are unable to obtain acceptable prices for those products, our prospects for generating revenue and achieving profitability will suffer.**

Our prospects for generating revenue and achieving profitability will depend heavily upon the availability of adequate reimbursement for the use of our approved product candidates from governmental and other third party payors, both in the U.S. and in other markets. Reimbursement by a third party payor may depend upon a number of factors, including the third party payor's determination that use of a product is:

- a covered benefit under its health plan;
- safe, effective and medically necessary;
- appropriate for the specific patient;
- cost-effective; and
- neither experimental nor investigational.

Obtaining reimbursement approval for a product from each government or other third party payor is a time consuming and costly process that could require us to provide supporting scientific, clinical and cost effectiveness data for the use of our products to each payor. We may not be able to provide data sufficient to gain acceptance with respect to reimbursement or we might need to conduct post-marketing studies in order to demonstrate the cost-effectiveness of any future products to such payors' satisfaction. Such studies might require us to commit a significant amount of management time and financial and other resources. Even when a payor determines that a product is eligible for reimbursement, the payor may impose coverage limitations that preclude payment for some uses that are approved by the FDA or non-U.S. regulatory authorities. In addition, there is a risk that full reimbursement may not be available for high priced products. Moreover, eligibility for coverage does not imply that any product will be reimbursed in all cases or at a rate that allows us to make a profit or even cover our costs. Interim payments for new products, if applicable, may also not be sufficient to cover our costs and may not be made permanent. In the case of Amigal, we will be reliant on GSK to seek reimbursement approvals from governments and third-party payors.

A primary trend in the U.S. healthcare industry and elsewhere is toward cost containment. We expect recent changes in the Medicare program and increasing emphasis on managed care to continue to put pressure on pharmaceutical product pricing. For example, the Medicare Prescription Drug Improvement and Modernization Act of 2003 provides a new Medicare prescription drug benefit that began in 2006 and mandates other reforms. While we cannot predict the full outcome of the implementation of this legislation, it is possible that the new Medicare prescription drug benefit, which will be managed by private health insurers and other managed care organizations, will result in additional government reimbursement for prescription drugs, which may make some prescription drugs more affordable but may further exacerbate industry wide pressure to reduce prescription drug prices. If one or more of our product candidates reaches commercialization, such changes may have a significant impact on our ability to set a price we believe is fair for our products and may affect our ability to generate revenue and achieve or maintain profitability.

**Governments outside the U.S. tend to impose strict price controls and reimbursement approval policies, which may adversely affect our prospects for generating revenue.**

In some countries, particularly European Union (EU) countries, the pricing of prescription pharmaceuticals is subject to governmental control. In these countries, pricing negotiations with governmental authorities can take considerable time (6 to 12 months or longer) after the receipt of marketing approval for a product. To obtain reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost effectiveness of our product candidate to other available therapies. If reimbursement of our products is unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, our prospects for generating revenue, if any, could be adversely affected and our business may suffer.

**If we are unable to establish sales and marketing capabilities or enter into agreements with third parties to market and sell our product candidates, we may be unable to generate product revenue.**

At present, we have no sales or marketing personnel. In order to commercialize any of our product candidates, we must either acquire or internally develop sales, marketing and distribution capabilities, or enter into collaborations with partners to perform these services for us, as we have done with GSK for the commercialization of Amigal. We may not be able to establish sales and distribution partnerships for other product candidates on acceptable terms or at all, and if we do enter into a distribution arrangement, our success will be dependent upon the performance of our partner.

In the event that we attempt to acquire or develop our own in-house sales, marketing and distribution capabilities, factors that may inhibit our efforts to commercialize our products without strategic partners or licensees include:

- our inability to recruit and retain adequate numbers of effective sales and marketing personnel;
- the inability of sales personnel to obtain access to or persuade adequate numbers of physicians to prescribe our products;
- the lack of complementary products to be offered by our sales personnel, which may put us at a competitive disadvantage against companies with broader product lines;
- unforeseen costs associated with creating our own sales and marketing team or with entering into a partnering agreement with an independent sales and marketing organization; and
- efforts by our competitors to commercialize products at or about the time when our product candidates would be coming to market.

We may co-promote our product candidates in various markets with pharmaceutical and biotechnology companies in instances where we believe that a larger sales and marketing presence will expand the market or accelerate penetration. If we do enter into arrangements with third parties to perform sales and marketing services, our product revenues will be lower than if we directly sold and marketed our products and any revenues received under such arrangements will depend on the skills and efforts of others.

We may not be successful in entering into distribution arrangements and marketing alliances with third parties. Our failure to enter into these arrangements on favorable terms could delay or impair our ability to commercialize our product candidates and could increase our costs of commercialization. Dependence on distribution arrangements and marketing alliances to commercialize our product candidates will subject us to a number of risks, including:

- we may not be able to control the amount and timing of resources that our distributors may devote to the commercialization of our product candidates;
- our distributors may experience financial difficulties;
- business combinations or significant changes in a distributor's business strategy may also adversely affect a distributor's willingness or ability to complete its obligations under any arrangement; and
- these arrangements are often terminated or allowed to expire, which could interrupt the marketing and sales of a product and decrease our revenue.

If we are unable to establish adequate sales, marketing and distribution capabilities, whether independently or with third parties, we may not be able to generate product revenue and may not become profitable.

**Product liability lawsuits against us could cause us to incur substantial liabilities and to limit commercialization of any products that we may develop.**

We face an inherent risk of product liability exposure related to the testing of our product candidates in human clinical trials and will face an even greater risk if we commercially sell any products that we may develop and which are approved for sale. We may be exposed to product liability claims and product recalls, including those which may arise from misuse or malfunction of, or design flaws in, such products, whether or not such problems directly relate to the products and services we have provided. If we cannot successfully defend ourselves against claims that our product candidates or products caused injuries, we will incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for any product candidates or products that we may develop;
- damage to our reputation;
- regulatory investigations that could require costly recalls or product modifications;
- withdrawal of clinical trial participants;
- costs to defend the related litigation;
- substantial monetary awards to trial participants or patients, including awards that substantially exceed our product liability insurance, which we would then be required to pay from other sources, if available, and would damage our ability to obtain liability insurance at reasonable costs, or at all, in the future;
- loss of revenue;
- the diversion of management's attention from managing our business; and
- the inability to commercialize any products that we may develop.

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We have liability insurance policies for our clinical trials in the geographies in which we are conducting trials. The amount of insurance that we currently hold may not be adequate to cover all liabilities that we may incur. Insurance coverage is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost and we may not be able to obtain insurance coverage that will be adequate to satisfy any liability that may arise. On occasion, large judgments have been awarded in class action lawsuits based on drugs that had unanticipated side effects. A successful product liability claim or a series of claims brought against us could cause our stock price to fall and, if judgments exceed our insurance coverage, could decrease our available cash and adversely affect our business.

### **We face substantial competition which may result in others discovering, developing or commercializing products before or more successfully than we do.**

The development and commercialization of new drugs is highly competitive and competition is expected to increase. We face competition with respect to our current product candidates and any products we may seek to develop or commercialize in the future from major pharmaceutical companies, specialty pharmaceutical companies and biotechnology companies worldwide. For example, several large pharmaceutical and biotechnology companies currently market and sell products for the treatment of lysosomal storage diseases, including Fabry disease. These products include Genzyme Corporation's Fabrazyme<sup>®</sup> and Shire plc's Replagal<sup>®</sup>. In addition, Genzyme Corporation and Actelion, Ltd. market and sell Cerezyme<sup>®</sup> and Zavesca<sup>®</sup>, respectively, for the treatment of Gaucher disease, and Genzyme Corporation markets and sells Myozyme<sup>®</sup> for the treatment of Pompe disease. We are also aware of other enzyme replacement and substrate reduction therapies in development by third parties, including eliglustat tartrate, an oral treatment developed by Genzyme and in Phase 3 development for the treatment of Gaucher disease, and taliglucerase alfa and velaglucerase, new enzyme replacement therapies for the treatment of Gaucher disease which are being developed by Protalix BioTherapeutics and Shire plc, respectively.

Potential competitors also include academic institutions, government agencies and other public and private research organizations that conduct research, seek patent protection and establish collaborative arrangements for research, development, manufacturing and commercialization. Our competitors may develop products that are more effective, safer, more convenient or less costly than any that we are developing or that would render our product candidates obsolete or noncompetitive. Our competitors may also obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for ours. We may also face competition from off-label use of other approved therapies. There can be no assurance that developments by others that will not render our product candidates obsolete or noncompetitive either during the research phase or once the products reach commercialization.

We believe that many competitors, including academic institutions, government agencies, public and private research organizations, large pharmaceutical companies and smaller more focused companies, are attempting to develop therapies for many of our target indications. Many of our competitors have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals, prosecuting intellectual property rights and marketing approved products than we do. Smaller and other early stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These third parties compete with us in recruiting and retaining qualified scientific and management personnel, establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to or necessary for our programs or advantageous to our business. In addition, if we obtain regulatory approvals for our products, manufacturing efficiency and marketing capabilities are likely to be significant competitive factors. We currently have no commercial manufacturing capability, sales force or marketing infrastructure. Further, many of our competitors have substantial resources and expertise in conducting collaborative arrangements, sourcing in-licensing arrangements and acquiring new business lines or businesses that are greater than our own.

### **Our business activities involve the use of hazardous materials, which require compliance with environmental and occupational safety laws regulating the use of such materials. If we violate these laws, we could be subject to significant fines, liabilities or other adverse consequences.**

Our research and development programs involve the controlled use of hazardous materials, including microbial agents, corrosive, explosive and flammable chemicals and other hazardous compounds in addition to certain biological hazardous waste. Ultimately, the activities of our third party product manufacturers when a product candidate reaches commercialization will also require the use of hazardous materials. Accordingly, we are subject to federal, state and local laws governing the use, handling and disposal of these materials. Although we believe that our safety procedures for handling and disposing of these materials comply in all material respects with the standards prescribed by local, state and federal regulations, we cannot completely eliminate the risk of accidental contamination or injury from these materials. In addition, our collaborators may not comply with these laws. In the event of an accident or failure to comply with environmental laws, we could be held liable for damages that result, and any such liability could exceed our assets and resources or we could be subject to limitations or stoppages related to our use of these materials which may lead to an interruption of our business operations or those of our third party contractors. While we believe that our existing insurance coverage is generally adequate for our normal handling of these hazardous materials, it may not be sufficient to cover pollution conditions or other extraordinary or unanticipated events. Furthermore, an accident could damage or force us to shut down our operations. Changes in environmental laws may impose costly compliance requirements on us or otherwise subject us to future liabilities and additional laws relating to the management, handling, generation, manufacture, transportation, storage, use and disposal of materials used in or generated by the manufacture of our products or related to our clinical trials. In addition, we cannot predict the effect that these potential requirements may have on us, our suppliers and contractors or our customers.



## Risks Related to Our Dependence on Third Parties

**Use of third parties to manufacture our product candidates may increase the risk that we will not have sufficient quantities of our product candidates or such quantities at an acceptable cost, and clinical development and commercialization of our product candidates could be delayed, prevented or impaired.**

We do not own or operate manufacturing facilities for clinical or commercial production of our product candidates. We have limited personnel with experience in drug manufacturing and we lack the resources and the capabilities to manufacture any of our product candidates on a clinical or commercial scale. We currently outsource all manufacturing and packaging of our preclinical and clinical product candidates and products to third parties. The manufacture of pharmaceutical products requires significant expertise and capital investment, including the development of advanced manufacturing techniques and process controls. Manufacturers of pharmaceutical products often encounter difficulties in production, particularly in scaling up initial production. These problems include difficulties with production costs and yields and quality control, including stability of the product candidate. The occurrence of any of these problems could significantly delay our clinical trials or the commercial availability of our products.

We do not currently have any agreements with third party manufacturers for the long-term commercial supply of any of our product candidates. We may be unable to enter into agreements for commercial supply with third party manufacturers, or may be unable to do so on acceptable terms. Even if we enter into these agreements, the manufacturers of each product candidate will be single source suppliers to us for a significant period of time.

Reliance on third party manufacturers' entails risks, to which we would not be subject if we manufactured product candidates or products ourselves, including:

- reliance on the third party for regulatory compliance and quality assurance;
- limitations on supply availability resulting from capacity and scheduling constraints of the third parties;
- impact on our reputation in the marketplace if manufacturers of our products, once commercialized, fail to meet the demands of our customers;
- the possible breach of the manufacturing agreement by the third party because of factors beyond our control; and
- the possible termination or non-renewal of the agreement by the third party, based on its own business priorities, at a time that is costly or inconvenient for us.

The failure of any of our contract manufacturers to maintain high manufacturing standards could result in injury or death of clinical trial participants or patients using products. Such failure could also result in product liability claims, product recalls, product seizures or withdrawals, delays or failures in testing or delivery, cost overruns or other problems that could seriously harm our business or profitability.

Our contract manufacturers are required to adhere to FDA regulations setting forth cGMP. These regulations cover all aspects of the manufacturing, testing, quality control and recordkeeping relating to our product candidates and any products that we may commercialize. Our manufacturers may not be able to comply with cGMP regulations or similar regulatory requirements outside the U.S. Our failure, or the failure of our third party manufacturers, to comply with applicable regulations could significantly and adversely affect regulatory approval and supplies of our product candidates.

Our product candidates and any products that we may develop may compete with other product candidates and products for access to manufacturing facilities. There are a limited number of manufacturers that operate under cGMP regulations and that are both capable of manufacturing for us and willing to do so. If the third parties that we engage to manufacture products for our preclinical tests and clinical trials should cease to continue to do so for any reason, we likely would experience delays in advancing these trials while we identify and qualify replacement suppliers and we may be unable to obtain replacement supplies on terms that are favorable to us. Later relocation to another manufacturer will also require notification, review and other regulatory approvals from the FDA and other regulators and will subject our production to further cost and instability in the availability of our product candidates. In addition, if we are not able to obtain adequate supplies of our product candidates or the drug substances used to manufacture them, it will be more difficult for us to develop our product candidates and compete effectively.

Our current and anticipated future dependence upon others for the manufacture of our product candidates may adversely affect our future profit margins and our ability to develop product candidates and commercialize any products that obtain regulatory approval on a timely and competitive basis.

**Materials necessary to manufacture our product candidates may not be available on commercially reasonable terms, or at all, which may delay the development and commercialization of our product candidates.**

We rely on the manufacturers of our product candidates to purchase from third party suppliers the materials necessary to produce the compounds for our preclinical and clinical studies and will rely on these other manufacturers for commercial distribution if we obtain marketing approval for any of our product candidates. Suppliers may not sell these materials to our manufacturers at the time we need them or on commercially reasonable terms and all such prices are susceptible to fluctuations in price and availability due to transportation costs, government regulations, price controls and changes in economic climate or other foreseen circumstances. We do not have any control over the process or timing of the acquisition of these materials by our manufacturers. Moreover, we currently do not have any agreements for the commercial production of these materials. If our manufacturers are unable to obtain these materials for our preclinical and clinical studies, product testing and potential regulatory approval of our product candidates would be delayed, significantly impacting our ability to develop our product candidates. If our manufacturers or we are unable to purchase these materials after regulatory approval has been obtained for our product candidates, the commercial launch of our product candidates would be delayed or there would be a shortage in supply, which would materially affect our ability to generate revenues from the sale of our product candidates.

**We rely on third parties to conduct certain preclinical development activities and our clinical trials and those third parties may not perform satisfactorily, including failing to meet established deadlines for the completion of such activities and trials.**

We do not independently conduct clinical trials for our product candidates or certain preclinical development activities of our product candidates, such as long-term safety studies in animals. We rely on, or work in conjunction with, third parties, such as contract research organizations, medical institutions and clinical investigators, to perform these functions. For example, we rely heavily on a contract research organization to help us conduct our ongoing Phase 3 clinical trials in Amigal for the treatment of Fabry disease. Our reliance on these third parties for preclinical and clinical development activities reduces our control over these activities. We are responsible for ensuring that each of our preclinical development activities and our clinical trials is conducted in accordance with the applicable general investigational plan and protocols, however, we have no direct control over these researchers or contractors (except by contract), as they are not our employees. Moreover, the FDA requires us to comply with standards, commonly referred to as Good Clinical Practices for conducting, recording and reporting the results of our preclinical development activities and our clinical trials to assure that data and reported results are credible and accurate and that the rights, safety and confidentiality of trial participants are protected. Our reliance on third parties that we do not control does not relieve us of these responsibilities and requirements. Furthermore, these third parties may also have relationships with other entities, some of which may be our competitors. If these third parties do not successfully carry out their contractual duties, meet expected deadlines or conduct our preclinical development activities or our clinical trials in accordance with regulatory requirements or our stated protocols, we will not be able to obtain, or may be delayed in obtaining, regulatory approvals for our product candidates and will not be able to, or may be delayed in our efforts to, successfully commercialize our product candidates. Moreover, these third parties may be bought by other entities or they may go out of business, thereby preventing them from meeting their contractual obligations.

We also rely on other third parties to store and distribute drug supplies for our preclinical development activities and our clinical trials. Any performance failure on the part of our existing or future distributors could delay clinical development or regulatory approval of our product candidates or commercialization of our products, producing additional losses and depriving us of potential product revenue.

Extensions, delays, suspensions or terminations of our preclinical development activities and our clinical trials as a result of the performance of our independent clinical investigators and contract research organizations will delay, and make more costly, regulatory approval for any product candidates that we may develop. Any change in a contract research organization during an ongoing preclinical development activity or clinical trial could seriously delay that trial and potentially compromise the results of the activity or trial.

**We may not be successful in maintaining or establishing collaborations, which could adversely affect our ability to develop and, particularly in international markets, commercialize products.**

For each of our product candidates, we are collaborating with physicians, patient advocacy groups, foundations and government agencies in order to assist with the development of our products. We plan to pursue similar activities in future programs and plan to evaluate the merits of retaining commercialization rights for ourselves or entering into selective collaboration arrangements with leading pharmaceutical or biotechnology companies, such as our collaboration for Amigal with GSK. We also may seek to establish collaborations for the sales, marketing and distribution of our products. If we elect to seek collaborators in the future but are unable to reach agreements with suitable collaborators, we may fail to meet our business objectives for the affected product or program. We face, and will continue to face, significant competition in seeking appropriate collaborators. Moreover, collaboration arrangements are complex and time consuming to negotiate, document and implement. We may not be successful in our efforts, if any, to establish and implement collaborations or other alternative arrangements. The terms of any collaboration or other arrangements that we establish, if any, may not be favorable to us.



Any collaboration that we enter into may not be successful. The success of our collaboration arrangements, if any, will depend heavily on the efforts and activities of our collaborators. It is likely that any collaborators of ours will have significant discretion in determining the efforts and resources that they will apply to these collaborations. The risks that we may be subject to in possible future collaborations include the following:

- our collaboration agreements are likely to be for fixed terms and subject to termination by our collaborators;
- our collaborators are likely to have the first right to maintain or defend our intellectual property rights and, although we would likely have the right to assume the maintenance and defense of our intellectual property rights if our collaborators do not, our ability to do so may be compromised by our collaborators' acts or omissions; and
- our collaborators may utilize our intellectual property rights in such a way as to invite litigation that could jeopardize or invalidate our intellectual property rights or expose us to potential liability.

Collaborations with pharmaceutical companies and other third parties often are terminated or allowed to expire by the other party. Such terminations or expirations may adversely affect us financially and could harm our business reputation in the event we elect to pursue collaborations that ultimately expire or are terminated.

**Our collaboration with GSK is important to our business. If this collaboration is unsuccessful or if GSK terminates this collaboration, our business could be adversely affected.**

We expect that a substantial amount of the funding for our operations will come from our collaboration with GSK. We and GSK are jointly developing Amigal and sharing costs associated with the development program in accordance with an agreed upon development plan. Under the plan, we are responsible for 50% of joint development costs of Amigal in 2011 and 25% of such costs in 2012 and beyond, subject to annual and aggregate caps. We are also eligible to receive approximately \$170 million if certain clinical, regulatory and sales milestones are met, as well as tiered double-digit royalties on sales of Amigal. Our business plan and financial guidance currently include assumptions regarding GSK's cost-sharing obligations and our achievement of milestones. However, GSK may elect to terminate this collaboration at its discretion. If this collaboration is unsuccessful, or if it is terminated in whole or in part, our business could be adversely affected. As a result, we could require additional financing earlier than we currently expect, or need to take additional steps to manage the financial risk associated with such termination, including actions that may affect our other programs.

In addition, while we are collaborating with GSK on the development of Amigal, GSK has decision making authority with respect to clinical development, regulatory and commercialization matters. The collaboration provides GSK with exclusive worldwide commercialization rights to Amigal, and we, therefore, are solely reliant on GSK for the commercialization of Amigal.

### **Risks Related to Our Intellectual Property**

**If we are unable to obtain and maintain protection for the intellectual property relating to our technology and products, the value of our technology and products will be adversely affected.**

Our success will depend in large part on our ability to obtain and maintain protection in the U.S. and other countries for the intellectual property covering or incorporated into our technology and products. The patent situation in the field of biotechnology and pharmaceuticals generally is highly uncertain and involves complex legal, technical, scientific and factual questions. We may not be able to obtain additional issued patents relating to our technology or products. Even if issued, patents issued to us or our licensors may be challenged, narrowed, invalidated, held to be unenforceable or circumvented, which could limit our ability to stop competitors from marketing similar products or reduce the term of patent protection we may have for our products. Changes in either patent laws or in interpretations of patent laws in the U.S. and other countries may diminish the value of our intellectual property or narrow the scope of our patent protection.

The degree of future protection for our proprietary rights is uncertain, and we cannot ensure that:

- we or our licensors were the first to make the inventions covered by each of our pending patent applications;
- we or our licensors were the first to file patent applications for these inventions;
- others will not independently develop similar or alternative technologies or duplicate any of our technologies;
- any patents issued to us or our licensors will provide a basis for commercially viable products, will provide us with any competitive advantages or will not be challenged by third parties;
- we will develop additional proprietary technologies that are patentable;
- we will file patent applications for new proprietary technologies promptly or at all;
- our patents will not expire prior to or shortly after commencing commercialization of a product; or
- the patents of others will not have a negative effect on our ability to do business.

In addition, we cannot be assured that any of our pending patent applications will result in issued patents. In particular, we have filed patent applications in the European Patent Office and other countries outside the U.S. that have not been issued as patents. These pending applications include, among others, the patent applications we license pursuant to a license agreement with Mount Sinai School of Medicine of New York University. If patents are not issued in respect of our pending patent applications, we may not be able to stop competitors from marketing similar products in Europe and other countries in which we do not have issued patents.

The patents and patent applications that we have licensed from Mt. Sinai School of Medicine relating to use of Amigal to treat Fabry disease expire in 2018 in the U.S., 2019 in Europe and Japan and the foreign counterpart patent application in Canada, if issued, will expire in 2019. These patents and application covering Amigal to treat Fabry disease have been sublicensed by Amicus to GSK, which now controls the prosecution and enforcement of said patents and patent applications to the extent they relate to Amigal. Patents that we have licensed claiming isofagomine (the active chemical moiety in Plicera) expire between 2015 and 2016 in the U.S. and in 2015 in the UK, France, Sweden, Germany, Switzerland and Japan. In the U.S., we have several issued patents that were licensed from the Mt. Sinai School of Medicine covering Plicera's methods of use which expire in 2018. We own a U.S. patent and its corresponding foreign applications covering isofagomine tartrate (the specific salt form of the active pharmaceutical ingredient in Plicera) and its use to treat Gaucher disease, which expires in 2027. Other than the patent application covering the use of isofagomine tartrate to treat Gaucher disease, we currently have no pending or issued patents covering methods of using Plicera outside of the U.S. Patents and patent applications that we own or have licensed relating to the use of AT2220 expire in 2018 in the U.S. Further, we currently do not have composition of matter or method of use protection for AT2220 in the U.S. or outside of the country. Where we lack patent protection outside of the U.S., we intend to seek orphan medicinal product designation and to rely on statutory data exclusivity provisions in jurisdictions outside the U.S. where such protections are available, including Europe. If we are unable to obtain such protection outside the U.S., our competitors may be free to use and sell Plicera and/or AT2220 outside of the U.S. and there will be no liability for infringement or any other barrier to competition. The patent rights that we own or have licensed relating to our product candidates are limited in ways that may affect our ability to exclude third parties from competing against us if we obtain regulatory approval to market these product candidates. In particular:

- We do not hold composition of matter patents covering Amigal and AT2220. Composition of matter patents can provide protection for pharmaceutical products to the extent that the specifically covered compositions are important. For our product candidates for which we do not hold composition of matter patents, competitors who obtain the requisite regulatory approval can offer products with the same composition as our products so long as the competitors do not infringe any method of use patents that we may hold.
- For some of our product candidates, the principal patent protection that covers or those we expect will cover, our product candidate is a method of use patent. This type of patent only protects the product when used or sold for the specified method. However, this type of patent does not limit a competitor from making and marketing a product that is identical to our product that is labeled for an indication that is outside of the patented method, or for which there is a substantial use in commerce outside the patented method.

Moreover, physicians may prescribe such a competitive identical product for indications other than the one for which the product has been approved, or off-label indications, that are covered by the applicable patents. Although such off-label prescriptions may infringe or induce infringement of method of use patents, the practice is common and such infringement is difficult to prevent or prosecute.

Our patents also may not afford us protection against competitors with similar technology. Because patent applications in the U.S. and many other jurisdictions are typically not published until 18 months after filing, or in some cases not at all, and because publications of discoveries in the scientific literature often lag behind the actual discoveries, neither we nor our licensors can be certain that we or they were the first to make the inventions claimed in our or their issued patents or pending patent applications, or that we or they were the first to file for protection of the inventions set forth in these patent applications. If a third party has also filed a U.S. patent application covering our product candidates or a similar invention, we may have to participate in an adversarial proceeding, known as an interference, declared by the U.S. Patent and Trademark Office to determine priority of invention in the U.S. The costs of these proceedings could be substantial and it is possible that our efforts could be unsuccessful, resulting in a loss of our U.S. patent position.

**If we fail to comply with our obligations in our intellectual property licenses with third parties, we could lose license rights that are important to our business.**

We are a party to a number of license agreements including agreements with the Mount Sinai School of Medicine of New York University, the University of Maryland, Baltimore County and Novo Nordisk A/S, pursuant to which we license key intellectual property relating to our lead product candidates. We expect to enter into additional licenses in the future. Under our existing licenses, we have the right to enforce the licensed patent rights. Our existing licenses impose, and we expect that future licenses will impose, various diligence, milestone payment, royalty, insurance and other obligations on us. If we fail to comply with these obligations, the licensor may have the right to terminate the license, in which event we might not be able to market any product that is covered by the licensed patents.

**If we are unable to protect the confidentiality of our proprietary information and know-how, the value of our technology and products could be adversely affected.**

We seek to protect our know-how and confidential information, in part, by confidentiality agreements with our employees, corporate partners, outside scientific collaborators, sponsored researchers, consultants and other advisors. We also have confidentiality and invention or patent assignment agreements with our employees and our consultants. If our employees or consultants breach these agreements, we may not have adequate remedies for any of these breaches. In addition, our trade secrets may otherwise become known to or be independently developed by others. Enforcing a claim that a party illegally obtained and is using our trade secrets is difficult, expensive and time consuming, and the outcome is unpredictable. In addition, courts outside the U.S. may be less willing to protect trade secrets. Costly and time consuming litigation could be necessary to seek to enforce and determine the scope of our proprietary rights, and failure to obtain or maintain trade secret protection could adversely affect our competitive business position.

**If we infringe or are alleged to infringe the intellectual property rights of third parties, it will adversely affect our business.**

Our research, development and commercialization activities, as well as any product candidates or products resulting from these activities, may infringe or be accused of infringing one or more claims of an issued patent or may fall within the scope of one or more claims in a published patent application that may subsequently issue and to which we do not hold a license or other rights. Third parties may own or control these patents or patent applications in the U.S. and abroad. These third parties could bring claims against us that would cause us to incur substantial expenses and, if successful against us, could cause us to pay substantial damages. Further, if a patent infringement suit were brought against us, we or they could be forced to stop or delay research, development, manufacturing or sales of the product or product candidate that is the subject of the suit.

No assurance can be given that patents do not exist, have not been filed, or could not be filed or issued, which contain claims covering our products, technology or methods. Because of the number of patents issued and patent applications filed in our field, we believe there is a risk that third parties may allege they have patent rights encompassing our products, technology or methods.

We are aware, for example, of U.S. patents, and corresponding international counterparts, owned by third parties that contain claims related to treating protein misfolding. If we were to challenge the validity of any issued U.S. patent in court, we would need to overcome a presumption of validity that attaches to every patent. This burden is high and would require us to present clear and convincing evidence as to the invalidity of the patent's claims. There is no assurance that a court would find in our favor on infringement or validity.

In order to avoid or settle potential claims with respect to any of the patent rights described above or any other patent rights of third parties, we may choose or be required to seek a license from a third party and be required to pay license fees or royalties or both. These licenses may not be available on acceptable terms, or at all. Even if we or our collaborators were able to obtain a license, the rights may be nonexclusive, which could result in our competitors gaining access to the same intellectual property. Ultimately, we could be prevented from commercializing a product, or be forced to cease some aspect of our business operations, if, as a result of actual or threatened patent infringement claims, we are unable to enter into licenses on acceptable terms. This could harm our business significantly.



Others may sue us for infringing their patent or other intellectual property rights or file nullity, opposition or interference proceedings against our patents, even if such claims are without merit, which would similarly harm our business. Furthermore, during the course of litigation, confidential information may be disclosed in the form of documents or testimony in connection with discovery requests, depositions or trial testimony. Disclosure of our confidential information and our involvement in intellectual property litigation could materially adversely affect our business.

There has been substantial litigation and other proceedings regarding patent and other intellectual property rights in the pharmaceutical and biotechnology industries. In addition to infringement claims against us, we may become a party to other patent litigation and other proceedings, including interference proceedings declared by the U.S. Patent and Trademark Office and opposition proceedings in the European Patent Office, regarding intellectual property rights with respect to our products and technology. Even if we prevail, the cost to us of any patent litigation or other proceeding could be substantial.

Some of our competitors may be able to sustain the costs of complex patent litigation more effectively than we can because they have substantially greater resources. In addition, any uncertainties resulting from any litigation could significantly limit our ability to continue our operations. Patent litigation and other proceedings may also absorb significant management time.

Many of our employees were previously employed at universities or other biotechnology or pharmaceutical companies, including our competitors or potential competitors. We try to ensure that our employees do not use the proprietary information or know-how of others in their work for us. However, we may be subject to claims that we or these employees have inadvertently or otherwise used or disclosed intellectual property, trade secrets or other proprietary information of any such employee's former employer. Litigation may be necessary to defend against these claims and, even if we are successful in defending ourselves, could result in substantial costs to us or be distracting to our management. If we fail to defend any such claims, in addition to paying monetary damages, we may jeopardize valuable intellectual property rights, disclose confidential information or lose personnel.

### **Risks Related to Regulatory Approval of Our Product Candidates**

**If we are not able to obtain and maintain required regulatory approvals, we will not be able to commercialize our product candidates, and our ability to generate revenue will be materially impaired.**

Our product candidates, including Amigal, and the activities associated with their development and commercialization, including their testing, manufacture, safety, efficacy, recordkeeping, labeling, storage, approval, advertising, promotion, sale and distribution, are subject to comprehensive regulation by the FDA and other regulatory agencies in the U.S. and by comparable authorities in other countries. Failure to obtain regulatory approval for a product candidate will prevent us from commercializing the product candidate in the jurisdiction of the regulatory authority. We have not obtained regulatory approval to market any of our product candidates in any jurisdiction. We have only limited experience in filing and prosecuting the applications necessary to obtain regulatory approvals and expect to rely on third party contract research organizations to assist us in this process. In the case of Amigal, GSK will have primary responsibility for the preparation, filing and prosecution of applications for approval with regulatory agencies.

Securing FDA approval requires the submission of extensive preclinical and clinical data and supporting information to the FDA for each therapeutic indication to establish the product candidate's safety and efficacy. Securing FDA approval also requires the submission of information about the product manufacturing process to, and inspection of manufacturing facilities by, the FDA. Our future products may not be effective, may be only moderately effective or may prove to have undesirable or unintended side effects, toxicities or other characteristics that may preclude our obtaining regulatory approval or prevent or limit commercial use.

Our product candidates may fail to obtain regulatory approval for many reasons, including:

- our failure to demonstrate to the satisfaction of the FDA or comparable regulatory authorities that a product candidate is safe and effective for a particular indication;
- the results of clinical trials may not meet the level of statistical significance required by the FDA or comparable regulatory authorities for approval;
- our inability to demonstrate that a product candidate's benefits outweigh its risks;
- our inability to demonstrate that the product candidate is at least as effective as existing therapies;
- the FDA's or comparable regulatory authorities' disagreement with the manner in which we interpret the data from preclinical studies or clinical trials;
- the FDA's or comparable regulatory authorities' failure to approve the manufacturing processes, quality procedures or manufacturing facilities of third party manufacturers with which we contract for clinical or commercial supplies; and
- a change in the approval policies or regulations of the FDA or comparable regulatory authorities or a change in the laws governing the approval process.



The process of obtaining regulatory approvals is expensive, often takes many years, if approval is obtained at all, and can vary substantially based upon a variety of factors, including the type, complexity and novelty of the product candidates involved. Changes in regulatory approval policies during the development period, changes in or the enactment of additional statutes or regulations, or changes in regulatory review for each submitted product application may cause delays in the approval or rejection of an application. The FDA and non-U.S. regulatory authorities have substantial discretion in the approval process and may refuse to accept any application or may decide that our data is insufficient for approval and require additional preclinical, clinical or other studies. In addition, varying interpretations of the data obtained from preclinical and clinical testing could delay, limit or prevent regulatory approval of a product candidate. Any regulatory approval we ultimately obtain may be limited or subject to restrictions or post approval commitments that render the approved product not commercially viable. Any FDA or other regulatory approval of our product candidates, once obtained, may be withdrawn, including for failure to comply with regulatory requirements or if clinical or manufacturing problems follow initial marketing. Under the terms of our collaboration with GSK, GSK will have considerable influence and decision making authority over matters relating to the submission of an NDA for Amigal in the U.S. and applications for approval of Amigal outside the U.S. GSK will also have primary responsibility for interactions with the FDA and other regulatory agencies outside the U.S. We, therefore, are heavily reliant on GSK for the prosecution of such applications.

**Our product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval or commercialization.**

Undesirable side effects caused by our product candidates could interrupt, delay or halt clinical trials and could result in the denial of regulatory approval by the FDA or other regulatory authorities for any or all targeted indications, and in turn prevent us from commercializing our product candidates and generating revenues from their sale. In addition, if any of our product candidates receive marketing approval and we or others later identify undesirable side effects caused by the product:

- regulatory authorities may require the addition of restrictive labeling statements;
- regulatory authorities may withdraw their approval of the product; and
- we may be required to change the way the product is administered or conduct additional clinical trials.

Any of these events could prevent us from achieving or maintaining market acceptance of the affected product or could substantially increase the costs and expenses of commercializing the product candidate, which in turn could delay or prevent us from generating significant revenues from its sale or adversely affect our reputation.

**We may not be able to obtain orphan drug exclusivity for our product candidates. If our competitors are able to obtain orphan drug exclusivity for their products that are the same drug as our product candidates, we may not be able to have competing products approved by the applicable regulatory authority for a significant period of time.**

Regulatory authorities in some jurisdictions, including the U.S. and Europe, may designate drugs for relatively small patient populations as orphan drugs. We obtained orphan drug designations from the FDA for Amigal for the treatment of Fabry disease on February 25, 2004, for the active ingredient in Plicera for the treatment of Gaucher disease on January 10, 2006 and for AT2220 for the treatment of Pompe disease on June 18, 2007. We also obtained orphan medicinal product designation in the EU for Amigal on May 22, 2006 and for Plicera on October 23, 2007. We anticipate filing for orphan drug designation in the EU for AT2220 for the treatment of Pompe disease. Generally, if a product with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, the product is entitled to a period of marketing exclusivity, which precludes the applicable regulatory authority from approving another marketing application for the same drug for that time period. The applicable period is 7 years in the U.S. and 10 years in Europe. For a drug composed of small molecules, the FDA defines “same drug” as a drug that contains the same active molecule and is intended for the same use. Obtaining orphan drug exclusivity for Amigal and Plicera may be important to each of the product candidate’s success. Even if we obtain orphan drug exclusivity for our products, we may not be able to maintain it. For example, if a competitive product that is the same drug as our product candidate is shown to be clinically superior to our product candidate, any orphan drug exclusivity we have obtained will not block the approval of such competitive product and we may effectively lose what had previously been orphan drug exclusivity.

**Any product for which we obtain marketing approval could be subject to restrictions or withdrawal from the market and we may be subject to penalties if we fail to comply with regulatory requirements or if we experience unanticipated problems with our products, when and if any of them are approved.**

Any product for which we obtain marketing approval, along with the manufacturing processes, post approval clinical data, labeling, advertising and promotional activities for such product, will be subject to continual requirements of and review by the FDA and comparable regulatory authorities. These requirements include submissions of safety and other post marketing information and reports, registration requirements, cGMP requirements relating to quality control, quality assurance and corresponding maintenance of records and documents, requirements regarding the distribution of samples to physicians and recordkeeping. Even if we obtain regulatory approval of a product, the approval may be subject to limitations on the indicated uses for which the product may be marketed or to the conditions of approval, or contain requirements for costly post marketing testing and surveillance to monitor the safety or efficacy of the product. We also may be subject to state laws and registration requirements covering the distribution of our products. Later discovery of previously unknown problems with our products, manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may result in actions such as:

- restrictions on such products, manufacturers or manufacturing processes;
- warning letters;
- withdrawal of the products from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- voluntary or mandatory recall;
- fines;
- suspension or withdrawal of regulatory approvals or refusal to approve pending applications or supplements to approved applications that we submit;
- refusal to permit the import or export of our products;
- product seizure or detentions;
- injunctions or the imposition of civil or criminal penalties; and
- adverse publicity.

If we, or our suppliers, third party contractors, clinical investigators or collaborators are slow to adapt, or are unable to adapt, to changes in existing regulatory requirements or adoption of new regulatory requirements or policies, we or our collaborators may lose marketing approval for our products when and if any of them are approved, resulting in decreased revenue from milestones, product sales or royalties.

**Failure to obtain regulatory approval in international jurisdictions would prevent us from marketing our products abroad.**

We intend to have our products marketed outside the U.S. In order to market our products in the EU and many other jurisdictions, we must obtain separate regulatory approvals and comply with numerous and varying regulatory requirements. The approval procedures vary among countries and can involve additional testing and clinical trials. The time required to obtain approval may differ from that required to obtain FDA approval. The regulatory approval process outside the U.S. may include all of the risks associated with obtaining FDA approval. In addition, in many countries outside the U.S., it is required that the product be approved for reimbursement by government-backed healthcare regulators or insurance providers before the product can be approved for sale in that country. We may not obtain approvals from regulatory authorities outside the U.S. on a timely basis, if at all. Approval by the FDA does not ensure approval by regulatory authorities in other countries or jurisdictions, and approval by one regulatory authority outside the U.S. does not ensure approval by regulatory authorities in other countries or jurisdictions or by the FDA. We may not be able to file for regulatory approvals and may not receive necessary approvals to commercialize our products in any market. Under the terms of our collaboration with GSK, GSK will have considerable influence and decision making authority over matters relating to the submission of applications for approval of Amigal outside the U.S. GSK will also have primary responsibility for interactions with regulatory agencies outside the U.S. We, therefore, are heavily reliant on GSK for the prosecution of such applications.

## **Risks Related to Employee Matters**

### **Our future success depends on our ability to retain our Chief Executive Officer and other key executives and to attract, retain and motivate qualified personnel.**

We are highly dependent on John F. Crowley, our Chairman and Chief Executive Officer, Matthew R. Patterson, our President and Chief Operating Officer, David J. Lockhart, Ph.D., our Chief Scientific Officer and Pol F. Boudes, M.D., our Chief Medical Officer. These executives each have significant pharmaceutical industry experience, including Mr. Crowley, with whom we have entered into an employment agreement that runs for successive one year terms until either we or Mr. Crowley elect to terminate the agreement. We may terminate Mr. Crowley's employment without cause at any time, or we may decide not to extend Mr. Crowley's agreement at the end of any term, or he may terminate his employment for good reason at any time, in each case subject to certain severance payments and benefits. Mr. Crowley is a commissioned officer in the U.S. Navy (Reserve), and he may be called to active duty service at any time. The loss of Mr. Crowley for protracted military duty could materially adversely affect our business. We are also parties to employment agreements with each of Mr. Patterson, Dr. Lockhart and Dr. Boudes. These employment agreements each provide for an initial term of two years, and will continue thereafter for successive two-year periods until we provide the executive with written notice of the end of the agreement in accordance with its terms. We may terminate any of these executives without cause at any time, or one of these executives may quit for good reason within six months of the occurrence of certain corporate changes, in each case subject to certain severance payments and benefits. The loss of the services of any of these executives might impede the achievement of our research, development and commercialization objectives and materially adversely affect our business. We do not maintain "key person" insurance on Mr. Crowley or on any of our other executive officers.

Recruiting and retaining qualified scientific, clinical and sales and marketing personnel will also be critical to our success. In addition, maintaining a qualified finance and legal department is key to our ability to meet our regulatory obligations as a public company and important in any potential capital raising activities. Our industry has experienced a high rate of turnover in recent years. We may not be able to attract and retain these personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar personnel, particularly in New Jersey and surrounding areas. Although we believe we offer competitive salaries and benefits, we may have to increase spending in order to retain personnel. If we fail to retain our remaining qualified personnel or replace them when they leave, we may be unable to continue our development and commercialization activities.

In addition, we rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development and commercialization strategy. Our consultants and advisors may be employed by employers other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us.

## **Risks Related to Our Common Stock**

### **Our executive officers, directors and principal stockholders maintain the ability to control all matters submitted to our stockholders for approval.**

Our executive officers, directors and principal stockholders beneficially own shares representing approximately 74% of our common stock as of December 31, 2010. As a result, if these stockholders were to choose to act together, they would be able to control all matters submitted to our stockholders for approval, as well as our management and affairs. For example, these persons, if they choose to act together, will control the election of directors and approval of any merger, consolidation, sale of all or substantially all of our assets or other business combination or reorganization. This concentration of voting power could delay or prevent an acquisition of us on terms that other stockholders may desire. The interests of this group of stockholders may not always coincide with the interests of other stockholders, and they may act, whether by meeting or written consent of stockholders, in a manner that advances their best interests and not necessarily those of other stockholders, including obtaining a premium value for their common stock, and might affect the prevailing market price for our common stock.

### **Provisions in our corporate charter documents and under Delaware law could make an acquisition of us, which may be beneficial to our stockholders, more difficult and may prevent attempts by our stockholders to replace or remove our current management.**

Provisions in our corporate charter and our bylaws may discourage, delay or prevent a merger, acquisition or other change in control of us that stockholders may consider favorable, including transactions in which our stockholders might otherwise receive a premium for their shares. These provisions could also limit the price that investors might be willing to pay in the future for shares of our common stock, thereby depressing the market price of our common stock. In addition, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our board of directors. Because our board of directors is responsible for appointing the members of our management team, these provisions could in turn affect any attempt by our stockholders to replace current members of our management team. Among others, these provisions:

- establish a classified board of directors, and, as a result, not all directors are elected at one time;
- allow the authorized number of our directors to be changed only by resolution of our board of directors;



- limit the manner in which stockholders can remove directors from our board of directors;
- establish advance notice requirements for stockholder proposals that can be acted on at stockholder meetings and nominations to our board of directors;
- require that stockholder actions must be effected at a duly called stockholder meeting and prohibit actions by our stockholders by written consent;
- limit who may call stockholder meetings;
- authorize our board of directors to issue preferred stock, without stockholder approval, which could be used to institute a “poison pill” that would work to dilute the stock ownership of a potential hostile acquirer, effectively preventing acquisitions that have not been approved by our board of directors; and
- require the approval of the holders of at least 67% of the votes that all our stockholders would be entitled to cast to amend or repeal certain provisions of our charter or bylaws.

Moreover, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, which prohibits a person who owns in excess of 15% of our outstanding voting stock from merging or combining with us for a period of three years after the date of the transaction in which the person acquired in excess of 15% of our outstanding voting stock, unless the merger or combination is approved in a prescribed manner.

### **An active trading market for our common stock may not develop.**

We completed our initial public offering of equity securities in June 2007, and prior to this offering, there was no public market for our common stock. Although we have been listed on The NASDAQ Global Market, an active trading market for our common stock may never develop or be sustained. If an active market for our common stock does not develop or is not sustained, it may be difficult for our stockholders to sell shares since our initial public offering without depressing the market price for our common stock.

### **If the price of our common stock is volatile, purchasers of our common stock could incur substantial losses.**

The price of our common stock is volatile. The stock market in general and the market for biotechnology companies in particular have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. The market price for our common stock may be influenced by many factors, including:

- results of clinical trials of our product candidates or those of our competitors;
- our entry into or the loss of a significant collaboration;
- regulatory or legal developments in the U.S. and other countries, including changes in the health care payment systems;
- variations in our financial results or those of companies that are perceived to be similar to us;
- changes in the structure of healthcare payment systems;
- market conditions in the pharmaceutical and biotechnology sectors and issuance of new or changed securities analysts’ reports or recommendations;
- general economic, industry and market conditions;
- results of clinical trials conducted by others on drugs that would compete with our product candidates;
- developments or disputes concerning patents or other proprietary rights;
- public concern over our product candidates or any products approved in the future;
- litigation;
- future sales or anticipated sales of our common stock by us or our stockholders; and
- the other factors described in this “Risk Factors” section.

For these reasons and others potential purchasers of our common stock should consider an investment in our common stock as risky and invest only if they can withstand a significant loss and wide fluctuations in the marked value of their investment.



**If securities or industry analysts do not publish research or reports or publish unfavorable research about our business, the price of our common stock and trading volume could decline.**

The trading market for our common stock depends in part on the research and reports that securities or industry analysts publish about us or our business. If securities or industry analysts do not initiate or continue coverage of us, the trading price for our common stock would be negatively affected. In the event we obtain securities or industry analyst coverage, if one or more of the analysts who covers us downgrades our common stock, the price of our common stock would likely decline. If one or more of these analysts ceases to cover us or fails to publish regular reports on us, interest in the purchase of our common stock could decrease, which could cause the price of our common stock or trading volume to decline.

**Item 1B. UNRESOLVED STAFF COMMENTS.**

None.

**Item 2. PROPERTIES.**

We currently lease approximately 59,000 square feet of office and laboratory space in Cranbury, New Jersey and 7,700 square feet of office and laboratory space in San Diego, California under various lease agreements that terminate no later than February 2012. We believe that our current office and laboratory facilities are adequate and suitable for our current and anticipated needs.

**Item 3. LEGAL PROCEEDINGS.**

We are not currently a party to any material legal proceedings.

**Item 4. RESERVED.**

**PART II**

**Item 5. MARKET FOR THE REGISTRANT’S COMMON EQUITY, RELATED STOCKHOLDER MATTERS AND ISSUER PURCHASES OF EQUITY SECURITIES.**

**Market For Our Common Stock**

Our common stock has been traded on the NASDAQ Global Market under the symbol “FOLD” since May 31, 2007. Prior to that time, there was no public market for our common stock. The following table sets forth the range of high and low closing sales prices of our common stock as quoted on the NASDAQ Global Market for the periods indicated.

	<u>High</u>	<u>Low</u>
<b>2010</b>		
First Quarter	\$ 4.47	\$ 3.04
Second Quarter	3.38	1.98
Third Quarter	4.05	1.88
Fourth Quarter	4.84	3.55
<b>2009</b>		
First Quarter	\$ 12.30	\$ 6.26
Second Quarter	13.50	6.44
Third Quarter	12.49	8.66
Fourth Quarter	9.05	3.21

The closing price for our common stock as reported by the NASDAQ Global Market on February 18, 2011 was \$6.53 per share. As of February 18, 2011, there were 44 holders of record of our common stock.

**Dividends**

We have never declared or paid any dividends on our capital stock. We currently intend to retain any future earnings to finance our research and development efforts, the further development of our pharmacological chaperone technology and the expansion of our business. We do not intend to declare or pay cash dividends to our stockholders in the foreseeable future.

**Recent Sales of Unregistered Securities**

None.

**Use of Proceeds from the Sale of Registered Securities**

*Initial Public Offering*

Our initial public offering of common stock was effected through a Registration Statement on Form S-1 (File No. 333-141700) that was declared effective by the Securities and Exchange Commission (SEC) on May 30, 2007. We registered an aggregate of 5,750,000 shares of our common stock. On June 5, 2007, at the closing of the offering, 5,000,000 shares of common stock were sold on our behalf at an initial public offering price of \$15.00 per share, for aggregate offering proceeds of \$75.0 million. The initial public offering was underwritten and managed by Morgan Stanley, Merrill Lynch & Co., JPMorgan, Lazard Capital Markets and Pacific Growth Equities, LLC. Following the sale of the 5,000,000 shares, the public offering terminated.

After deducting expenses of approximately \$6.9 million, we received net offering proceeds of approximately \$68.1 million from our initial public offering. As of December 31, 2010, approximately \$18.4 million of the net proceeds from our initial public offering were maintained in money market funds and in investment-grade, interest bearing instruments, pending their use. We have used the remaining proceeds of approximately \$49.7 million for clinical development of our projects, research and development activities relating to additional preclinical projects and to fund working capital and other general corporate purposes.

*March 2010 Registered Direct Offering*

In March 2010, we sold 4,946,524 million shares of our common stock and warrants to purchase 1,854,946 million shares of common stock in a registered direct offering to a select group of institutional investors through a Registration Statement on Form S-3 (File No. 333-158405) that was declared effective by the SEC on May 27, 2009. The shares of common stock and warrants were sold in units consisting of one share of common stock and one warrant to purchase 0.375 shares of common stock at a price of \$3.74 per unit. The warrants have a term of four years and are exercisable any time on or after the six month anniversary of the date they were issued, at an exercise price of \$4.43 per share. The aggregate offering proceeds were \$18.5 million. Leerink Swann LLC served as sole placement agent for the offering. Following the sale of the common stock and warrants, the public offering terminated.

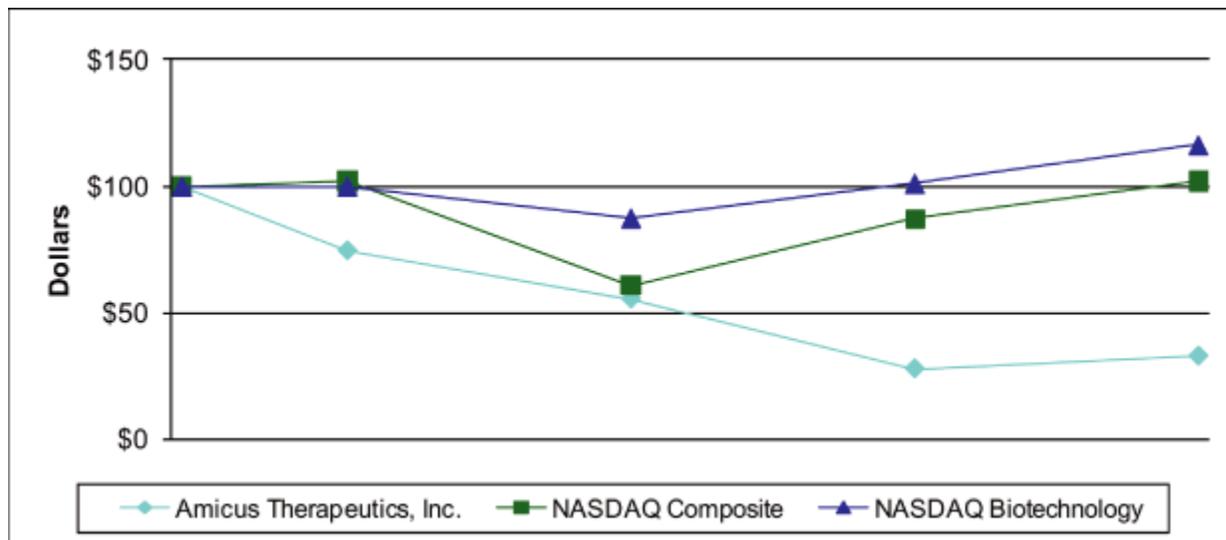
We paid Leerink Swann a placement agency fee equal to 5.7% of the aggregate offering proceeds, approximately \$1.05 million. The net proceeds of the offering were \$17.1 million after deducting the placement agency fee and all other estimated offering expenses. No offering expenses were paid directly or indirectly to any of our directors or officers (or their associates) or persons owning ten percent or more of any class of our equity securities or to any other affiliates.

As of December 31, 2010, we had invested the \$17.1 million in net proceeds from our registered direct offering in money market funds and in investment-grade, interest bearing instruments, pending their use. Through December 31, 2010, we have not used the net proceeds from this offering. We intend to use the proceeds from this offering to further advance the development of our lead product candidate, Amigal, and the completion of certain activities required for the submission of a license application globally, as well as for general corporate matters.

The foregoing represents our best estimate of our use of proceeds for the period indicated.

**Performance Graph**

The following performance graph shows the total shareholder return of an investment of \$100 cash on May 31, 2007, the date our common stock first started trading on the NASDAQ Global Market, for (i) our common stock, (ii) the NASDAQ Composite Index (U.S.) and (iii) the NASDAQ Biotechnology Index as of December 31, 2010. Pursuant to applicable SEC rules, all values assume reinvestment of the full amount of all dividends, however no dividends have been declared on our common stock to date. The stockholder return shown on the graph below is not necessarily indicative of future performance, and we do not make or endorse any predictions as to future stockholder returns.



\* \$100 invested on May 31, 2007 in Amicus Therapeutics, Inc. stock or in index-including reinvestment of dividends.

	<u>5/31/2007</u>	<u>12/31/2007</u>	<u>12/31/2008</u>	<u>12/31/2009</u>	<u>12/31/2010</u>
<b>Amicus Therapeutics, Inc.</b>	100	74	55	28	33
<b>NASDAQ Composite</b>	100	102	61	87	102
<b>NASDAQ Biotechnology</b>	100	100	87	101	116

*The stock price performance included in this graph is not necessarily indicative of future stock price performance.*

**Issuer Purchases of Equity Securities**

The following table sets forth purchases of our common stock for the three months ended December 31, 2010:

<b>Period</b>	<b>(a) Total number of shares purchased</b>	<b>(b) Average Price Paid per Share</b>	<b>(c) Total number of shares purchased as part of publicly announced plans or programs</b>	<b>(d) Maximum number of shares that may yet be purchased under the plans or programs</b>
October 1, 2010 – October 31, 2010	223	\$ 3.92	—	—
Total	<u>223</u>		<u>—</u>	

Pursuant to a restricted stock award dated October 2, 2006 between Amicus Therapeutics, Inc. and James E. Dentzer, our former Chief Financial Officer, Mr. Dentzer was granted 40,000 shares, 25% of which vested on October 2, 2007 and the remaining shares vested in a series of thirty-six successive equal monthly installments ending on October 1, 2010. In order to comply with the minimum statutory federal tax withholding rate of 25% plus 1.45% for Medicare, Mr. Dentzer surrendered a portion of his vested shares on each vesting date, representing 26.45% of the total value of the shares then vested.

## Item 6. SELECTED FINANCIAL DATA.

(in thousands except share and per share data)

	Year Ended December 31,					Period from
	2006	2007	2008	2009	2010	February 4, 2002 (inception) to December 31, 2010
<b>Statement of Operations Data:</b>						
Revenue:						
Research revenue	\$ —	\$ 1,375	\$ 12,189	\$ 17,545	\$ —	\$ 31,108
Collaboration revenue	—	409	2,778	46,813	922	50,922
Total revenue	—	1,784	14,967	64,358	922	82,030
Operating expenses:						
Research and development	33,630	31,074	37,764	48,081	39,042	214,764
General and administrative	12,277	15,278	19,666	19,973	15,660	93,369
Restructuring charges	—	—	—	1,522	—	1,522
Impairment of leasehold improvements	—	—	—	—	—	1,030
Depreciation and amortization	952	1,237	1,493	2,132	2,058	8,478
In-process research and development	—	—	—	—	—	418
Total operating expenses	46,859	47,589	58,923	71,708	56,760	319,581
Loss from operations	(46,859)	(45,805)	(43,956)	(7,350)	(55,838)	(237,551)
Other income (expenses):						
Interest income	1,990	5,135	4,819	997	156	13,913
Interest expense	(273)	(348)	(218)	(278)	(260)	(2,185)
Change in fair value of warrant liability	(23)	(149)	—	—	(1,410)	(1,864)
Other (expense)/income, net	(1,180)	—	—	64	1,277	(161)
Loss before tax benefit	(46,345)	(41,167)	(39,355)	(6,567)	(56,075)	(227,526)
Income tax benefit	—	—	—	—	1,139	1,834
Net loss	(46,345)	(41,167)	(39,355)	(6,567)	(54,936)	(225,692)
Deemed dividend	(19,424)	—	—	—	—	(19,424)
Preferred stock accretion	(159)	(351)	—	—	—	(802)
Net loss attributable to common stockholders	<u>\$ (65,928)</u>	<u>\$ (41,518)</u>	<u>\$ (39,355)</u>	<u>\$ (6,567)</u>	<u>\$ (54,936)</u>	<u>(245,918)</u>
Net loss attributable to common stockholders per common share — basic and diluted	<u>\$ (89.58)</u>	<u>\$ (3.14)</u>	<u>\$ (1.75)</u>	<u>\$ (0.29)</u>	<u>\$ (1.98)</u>	
Weighted-average common shares outstanding — basic and diluted	<u>735,967</u>	<u>13,235,755</u>	<u>22,493,803</u>	<u>22,624,134</u>	<u>27,734,797</u>	

## As of December 31,

	2006	2007	2008	2009	2010
<b>Balance Sheet Data:</b>					
Cash and cash equivalents and marketable securities	\$ 54,699	\$ 161,527	\$ 121,124	\$ 78,224	\$ 107,445

Working capital	44,814	147,247	110,209	69,293	93,458
Total assets	59,645	167,097	128,773	85,370	112,552
Total liabilities	13,071	63,800	57,730	13,537	47,618
Redeemable convertible preferred stock	124,089	—	—	—	—
Deficit accumulated during the development stage	(83,667)	(124,834)	(164,189)	(170,756)	(225,692)
Total stockholders' (deficiency) equity	\$ (77,515)	\$ 103,297	\$ 71,043	\$ 71,833	\$ 64,934

**Item 7. MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS.**

**Overview**

Amicus Therapeutics, Inc. (Amicus) is a biopharmaceutical company focused on the discovery, development and commercialization of orally-administered, small molecule drugs known as pharmacological chaperones for the treatment of rare diseases. Pharmacological chaperones are a novel, first-in-class approach to treating a broad range of diseases including lysosomal storage disorders and diseases of neurodegeneration. We believe that our pharmacological chaperone technology, our advanced product pipeline, especially our lead product candidate, Amigal, and our strategic collaboration with GlaxoSmithKline uniquely position us as a leader in the development of treatments for rare diseases.

Our current areas of focus include the following:

- the Phase 3 development of our lead product candidate, Amigal for Fabry disease;
- the preclinical and clinical development of pharmacological chaperones co-administered with enzyme replacement therapy; and
- the preclinical evaluation of the use of pharmacological chaperones for diseases of neurodegeneration.

Our novel approach to the treatment of human genetic diseases consists of using pharmacological chaperones that selectively bind to the target protein; increasing the stability of the protein and helping it fold into the correct three-dimensional shape. This allows proper trafficking of the protein within the cell, thereby increasing protein activity, improving cellular function and potentially reducing cell stress. We have also demonstrated in preclinical studies that pharmacological chaperones can further stabilize normal, or "wild-type" proteins. This stabilization could lead to a higher percentage of the target proteins folding correctly and more stably, which can increase cellular levels of that target protein and improve cellular function, making chaperones potentially applicable to a wide range of diseases.

Our lead product candidate, Amigal (migalastat hydrochloride) for Fabry disease, is in Phase 3 development. We are developing and commercializing Amigal with an affiliate of GlaxoSmithKline PLC (GSK) pursuant to a License and Collaboration Agreement entered into in October 2010. Our partnership with GSK allows us to utilize GSK's significant expertise in clinical, regulatory, commercial and manufacturing matters in the development in Amigal. In addition, the cost-sharing arrangements and potential milestone and royalty payments under the License and Collaboration Agreement provide us with financial strength and allow us to continue the development of Amigal while also advancing our other programs. We also believe this collaboration is important in validating our status as a leader in the development of treatments for rare diseases given the increasing focus placed on the rare disease field.

Our Phase 3 clinical development program for the use of Amigal as monotherapy in Fabry disease includes two clinical trials: Study 011 and Study 012. We have enrolled a majority of the planned 60 patients for Study 011, and intend to commence an additional Phase 3 study (Study 012) in the first half of 2011. We plan to use the data from Study 011 to support the filing of a New Drug Application, or NDA, for marketing approval in the United States and the data from Study 012 to support the filing of an application for marketing authorization in Europe.

While our initial clinical efforts have focused on the use of pharmacological chaperones to treat lysosomal storage diseases, we believe that our technology may be applicable to the treatment of certain diseases of neurodegeneration. Our lead preclinical program in this area is focused on Parkinson's disease, where we expect to complete late-stage preclinical proof of concept studies, including IND-enabling activities, for our pharmacological chaperone molecule AT3375 during 2011. Our second preclinical program in this area is focused on Alzheimer's disease. Our preclinical work in both Parkinson's and Alzheimer's disease is presently focused on genetically-defined subpopulations of Parkinson's and Alzheimer's patients and leverages our expertise and knowledge in the rare disease field.

We have generated significant losses to date and expect to continue to generate losses as we continue the clinical development of our drug candidates, including Amigal, and conduct preclinical studies on other programs. These activities are budgeted to expand over time and will require further resources if we are to be successful. From our inception in February 2002 through December 31, 2010, we have accumulated a deficit of \$225.7 million. As we have not yet generated commercial sales revenue from any of our product candidates, our losses will continue and are likely to be substantial over at least the next couple of years.

In June 2007, we completed our initial public offering (IPO) of 5,000,000 shares of common stock at a public offering price of \$15.00 per share. Net cash proceeds from the initial public offering were approximately \$68.1 million. In March 2010, we sold 4.95 million shares of our common stock and warrants to purchase 1.85 million shares of common stock in a registered direct offering to a select group of institutional investors. The shares of common stock and warrants were sold in units consisting of one share of common stock and one warrant to purchase 0.375 shares of common stock at a price of \$3.74 per unit. The warrants have a term of four years and are exercisable any time on or after the six month anniversary of the date they were issued, at an exercise price of \$4.43 per share. The net proceeds of the offering were \$17.1 million.



### ***Collaboration with GSK***

On October 28, 2010, the Company entered into the License and Collaboration Agreement with Glaxo Group Limited, an affiliate of GSK, to develop and commercialize Amigal. Under the terms of the License and Collaboration Agreement, GSK received an exclusive worldwide license to develop, manufacture and commercialize Amigal. In consideration of the license grant, the Company received an upfront, license payment of \$30 million from GSK and is eligible to receive further payments of approximately \$170 million upon the successful achievement of development and commercialization milestones, as well as tiered double-digit royalties on global sales of Amigal. GSK and the Company will jointly fund development costs in accordance with an agreed upon development plan. This plan provides that the Company will fund 50% of the development costs for 2011 and 25% of the development costs in 2012 and beyond. The Company's development costs are subject to annual and aggregate caps. Additionally, GSK purchased approximately 6.9 million shares of the Company's common stock at a price of \$4.56 per share. The total value of this equity investment to the Company is approximately \$31 million and represents a 19.9% ownership position in the Company. Under the terms of the collaboration agreement, while we will collaborate with GSK, GSK will have decision-making authority over clinical, regulatory and commercial matters related to Amigal. Additionally, GSK will have primary responsibility for interactions with regulatory agencies and prosecuting applications for marketing and reimbursement approvals worldwide.

### **Financial Operations Overview**

#### ***Revenue***

In November 2010, GSK paid us an initial, non-refundable license fee of \$30 million and a premium of \$3.2 million related to GSK's purchase of an equity investment in Amicus. The total upfront consideration received of \$33.2 million will be recognized as Collaboration Revenue on a straight-line basis over the development period of the collaboration agreement which is approximately 5.2 years. At December 31, 2010, we recognized approximately \$0.9 million of the total upfront consideration as Collaboration Revenue.

#### ***Research and Development Expenses***

We expect to continue to incur substantial research and development expenses as we continue to develop our product candidates and explore new uses for our pharmacological chaperone technology. However, we will share future research and development costs related to Amigal with GSK in accordance with the License and Collaboration Agreement.

- internal costs associated with our research and clinical development activities;
- payments we make to third party contract research organizations, contract manufacturers, investigative sites, and consultants;
- technology license costs;
- manufacturing development costs;
- personnel related expenses, including salaries, benefits, travel, and related costs for the personnel involved in drug discovery and development;
- activities relating to regulatory filings and the advancement of our product candidates through preclinical studies and clinical trials; and
- facilities and other allocated expenses, which include direct and allocated expenses for rent, facility maintenance, as well as laboratory and other supplies.

We have multiple research and development projects ongoing at any one time. We utilize our internal resources, employees and infrastructure across multiple projects. We record and maintain information regarding external, out-of-pocket research and development expenses on a project specific basis.

We expense research and development costs as incurred, including payments made to date under our license agreements. We believe that significant investment in product development is a competitive necessity and plan to continue these investments in order to realize the potential of our product candidates. From our inception in February 2002 through December 31, 2010, we have incurred research and development expense in the aggregate of \$214.8 million.

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The following table summarizes our principal product development projects through December 31, 2010, including the related stages of development for each project, and the out-of-pocket, third party expenses incurred with respect to each project (in thousands).

Projects	Years Ended December 31,			Period from
	2008	2009	2010	February 4, 2002 (Inception) to December 31, 2010
Projects				
Third party direct project expenses				
Amigal (Fabry Disease — Phase 3)	\$ 4,410	\$ 8,634	\$ 11,956	\$ 46,030
Plicera (Gaucher Disease — Phase 2*)	2,796	6,961	362	26,227
AT2220 (Pompe Disease — Phase 1)	2,836	1,874	236	13,134
Neurodegenerative Diseases	1,801	3,194	784	6,399
Total third party direct project expenses	11,843	20,663	13,338	91,790
Other project costs <sup>(1)</sup>				
Personnel costs	14,535	18,801	16,671	74,437
Other costs <sup>(2)</sup>	11,386	8,617	9,033	48,537
Total other project costs	25,921	27,418	25,704	122,974
Total research and development costs	\$ 37,764	\$ 48,081	\$ 39,042	\$ 214,764

(1) Other project costs are leveraged across multiple projects.

(2) Other costs include facility, supply, overhead, and licensing costs that support multiple projects.

\* We do not plan to advance Plicera into Phase 3 development at this time.

The successful development of our product candidates is highly uncertain. At this time, we cannot reasonably estimate or know the nature, timing and costs of the efforts that will be necessary to complete the remainder of the development of our product candidates. As a result, we are not able to reasonably estimate the period, if any, in which material net cash inflows may commence from our product candidates, including Amigal or any of our other preclinical product candidates. This uncertainty is due to the numerous risks and uncertainties associated with the conduct, duration and cost of clinical trials, which vary significantly over the life of a project as a result of evolving events during clinical development, including:

- the number of clinical sites included in the trials;
- the length of time required to enroll suitable patients;
- the number of patients that ultimately participate in the trials;
- the results of our clinical trials; and
- any mandate by the U.S. Food and Drug Administration (FDA) or other regulatory authority to conduct clinical trials beyond those currently anticipated.

Our expenditures are subject to additional uncertainties, including the terms and timing of regulatory approvals, and the expense of filing, prosecuting, defending and enforcing any patent claims or other intellectual property rights. We may obtain unexpected results from our clinical trials. We may elect to discontinue, delay or modify clinical trials of some product candidates or focus on others. In addition, GSK has considerable influence over and decision-making authority related to our Amigal program. A change in the outcome of any of the foregoing variables with respect to the development of a product candidate could mean a significant change in the costs and timing associated with the development, regulatory approval and commercialization of that product candidate. For example, if the FDA or other regulatory authorities were to require us to conduct clinical trials beyond those which we currently anticipate, or if we experience significant delays in enrollment in any of our clinical trials, we could be required to expend significant additional financial resources and time on the completion of clinical development. Drug development may take several years and millions of dollars in development costs.

### General and Administrative Expense

General and administrative expense consists primarily of salaries and other related costs, including stock-based compensation expense, for persons serving in our executive, finance, accounting, legal, information technology and human resource functions. Other general and administrative expense includes facility-related costs not otherwise included in research and development expense, promotional expenses, costs associated with industry and trade shows, and professional fees for legal services, including patent-related

expense and accounting services. From our inception in February 2002 through December 31, 2010, we spent \$93.4 million on general and administrative expense.

### ***Interest Income and Interest Expense***

Interest income consists of interest earned on our cash and cash equivalents and marketable securities. Interest expense consists of interest incurred on our capital lease facility and our equipment financing agreement.

### **Critical Accounting Policies and Significant Judgments and Estimates**

The discussion and analysis of our financial condition and results of operations are based on our financial statements, which we have prepared in accordance with U.S. generally accepted accounting principles. The preparation of these financial statements requires us to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of the financial statements, as well as the reported revenues and expenses during the reporting periods. On an ongoing basis, we evaluate our estimates and judgments, including those described in greater detail below. We base our estimates on historical experience and on various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions. We believe that the following discussion represents our critical accounting policies.

### ***Revenue Recognition***

We recognize revenue when amounts are realized or realizable and earned. Revenue is considered realizable and earned when the following criteria are met: (1) persuasive evidence of an arrangement exists; (2) delivery has occurred or services have been rendered; (3) the price is fixed or determinable; and (4) collection of the amounts due are reasonably assured.

In multiple element arrangements, revenue is allocated to each separate unit of accounting and each deliverable in an arrangement is evaluated to determine whether it represents separate units of accounting. A deliverable constitutes a separate unit of accounting when it has standalone value and there is no general right of return for the delivered elements. In instances when the aforementioned criteria are not met, the deliverable is combined with the undelivered elements and the allocation of the arrangement consideration and revenue recognition is determined for the combined unit as a single unit of accounting. Allocation of the consideration is determined at arrangement inception on the basis of each unit's relative selling price. In instances where there is determined to be a single unit of accounting, the total consideration is applied as revenue for the single unit of accounting and is recognized over the period of inception through the date where the last deliverable within the single unit of accounting is expected to be delivered.

Our current revenue recognition policies, which were applied in fiscal 2010, provide that, when a collaboration arrangement contains multiple deliverables, such as license and research and development services, we allocate revenue to each separate unit of accounting based on a selling price hierarchy. The selling price hierarchy for a deliverable is based on (i) its vendor specific objective evidence (VSOE) if available, (ii) third party evidence (TPE) if VSOE is not available, or (iii) estimated selling price (BESP) if neither VSOE nor TPE is available. We would establish the VSOE of selling price using the price charged for a deliverable when sold separately. The TPE of selling price would be established by evaluating largely similar and interchangeable competitor products or services in standalone sales to similarly situated customers. The best estimate of selling price would be established considering internal factors such as an internal pricing analysis or an income approach using a discounted cash flow model.

The revenue associated with reimbursements for research and development costs under collaboration agreements is included in Research Revenue and the costs associated with these reimbursable amounts are included in research and development expenses. We record these reimbursements as revenue and not as a reduction of research and development expenses as we have not commenced our planned principal operations (i.e., selling commercial products) and we are a development stage enterprise, therefore development activities are part of our ongoing central operations.

Our collaboration agreement with GSK provides for, and any future collaboration agreements we may enter into also may provide for contingent milestone payments. In order to determine the revenue recognition for these contingent milestones, we evaluate the contingent milestones using the criteria as provided by the FASB guidance on the milestone method of revenue recognition at the inception of a collaboration agreement. The criteria requires that (i) we determine if the milestone is commensurate with either our performance to achieve the milestone or the enhancement of value resulting from our activities to achieve the milestone, (ii) the milestone be related to past performance, and (iii) the milestone be reasonable relative to all deliverable and payment terms of the collaboration arrangement. If these criteria are met then the contingent milestones can be considered as substantive milestones and will be recognized as revenue in the period that the milestone is achieved.

**Accrued Expenses**

When we are required to estimate accrued expenses because we have not yet been invoiced or otherwise notified of actual cost, we identify services that have been performed on our behalf and estimate the level of service performed and the associated cost incurred. The majority of our service providers invoice us monthly in arrears for services performed. We make estimates of our accrued expenses as of each balance sheet date in our financial statements based on facts and circumstances known to us. Examples of estimated accrued expenses include:

- fees owed to contract research organizations in connection with preclinical and toxicology studies and clinical trials;
- fees owed to investigative sites in connection with clinical trials;
- fees owed to contract manufacturers in connection with the production of clinical trial materials;
- fees owed for professional services, and
- unpaid salaries, wages and benefits.

**Stock-Based Compensation**

We adopted the fair value method of measuring stock-based compensation, which requires a public entity to measure the cost of employee services received in exchange for an award of equity instruments based upon the grant-date fair value of the award. We chose the “straight-line” attribution method for allocating compensation costs and recognized the fair value of each stock option on a straight-line basis over the vesting period of the related awards.

We recognized stock-based compensation expense of \$6.4 million, \$7.8 million, and \$6.2 million for the years ended 2008, 2009 and 2010, respectively. The following table summarizes information related to stock compensation expense recognized in the income statement (in millions):

	<b>Years Ended December 31,</b>		
	<b>2008</b>	<b>2009</b>	<b>2010</b>
Stock compensation expense recognized in:			
Research and development expense	\$ 2.5	\$ 3.2	\$ 2.6
General and administrative expense	3.9	4.6	3.6
Total stock compensation expense	<u>\$ 6.4</u>	<u>\$ 7.8</u>	<u>\$ 6.2</u>

We use the Black-Scholes option pricing model when estimating the value for stock-based awards. Use of a valuation model requires management to make certain assumptions with respect to selected model inputs. Expected volatility was calculated based on a blended weighted average of historical information of our stock and the weighted average of historical information of similar public entities for which historical information was available. We will continue to use a blended weighted average approach using our own historical volatility and other similar public entity volatility information until our historical volatility is relevant to measure expected volatility for future option grants. The average expected life was determined using the mid-point between the vesting date and the end of the contractual term. The risk-free interest rate is based on U.S. Treasury, zero-coupon issues with a remaining term equal to the expected life assumed at the date of grant. Forfeitures are estimated based on voluntary termination behavior, as well as a historical analysis of actual option forfeitures. The weighted average assumptions used in the Black-Scholes option pricing model are as follows:

	<b>Years Ended December 31,</b>		
	<b>2008</b>	<b>2009</b>	<b>2010</b>
Expected stock price volatility	78.2%	80.6%	80.5%
Risk free interest rate	3.0%	2.4%	2.4%
Expected life of options (years)	6.25	6.25	6.25
Expected annual dividend per share	\$ 0.00	\$ 0.00	\$ 0.00

The weighted-average grant-date fair value per share of options granted during 2008, 2009 and 2010 were \$7.36, \$4.83 and \$2.09, respectively.

**Warrants**

We allocated \$3.3 million of proceeds from our March 2010 registered direct offering to warrants issued in connection with the offering that was classified as a liability and is subject to fair value mark-to-market adjustment each period. The valuation of the warrants is determined using the Black-Scholes model using inputs such as the underlying price of the shares issued when the warrant is exercised, volatility, risk free interest rate and expected life of the instrument. The fair value of the warrants at December 31, 2010 was determined by using the Black-Scholes model assuming a risk free interest rate of 1.15%, volatility of 78.7% and an expected life of 3.17 years which is equal to the contractual life of the warrants. As a result, for the year ended December 31, 2010, we recorded a

change in warrant liability expense of \$1.4 million. The resulting fair value of the warrant liability at December 31, 2010 was \$4.7 million.

## Basic and Diluted Net Loss Attributable to Common Stockholders per Common Share

We calculated net loss per share as a measurement of the Company's performance while giving effect to all dilutive potential common shares that were outstanding during the reporting period. We had a net loss for all periods presented; accordingly, the inclusion of common stock options and warrants would be anti-dilutive. Therefore, the weighted average shares used to calculate both basic and diluted earnings per share are the same.

The following table provides a reconciliation of the numerator and denominator used in computing basic and diluted net loss attributable to common stockholders per common share (in thousands except share amounts):

	<b>Years Ended December 31,</b>		
	<b>2008</b>	<b>2009</b>	<b>2010</b>
<b>Historical</b>			
Numerator:			
Net loss attributable to common stockholders	<u>\$ (39,355)</u>	<u>\$ (6,567)</u>	<u>\$ (54,936)</u>
Denominator:			
Weighted average common shares outstanding — basic and diluted	<u>22,493,803</u>	<u>22,624,134</u>	<u>27,734,797</u>

Dilutive common stock equivalents would include the dilutive effect of common stock options for common stock equivalents. Potentially dilutive common stock equivalents totaled approximately 3.1 million, 4.8 million and 7.0 million for the years ended December 31, 2008, 2009 and 2010, respectively. Potentially dilutive common stock equivalents were excluded from the diluted earnings per share denominator for all periods because of their anti-dilutive effect.

## Results of Operations

### *Year Ended December 31, 2010 Compared to Year Ended December 31, 2009*

*Research and Development Expense.* Research and development expense was \$39.0 million in 2010 representing a decrease of \$9.1 million or 19% from \$48.1 million in 2009. The variance was primarily attributable to lower personnel costs of \$2.1 million associated with the 2009 work force reduction, a \$0.7 million decrease in contract manufacturing costs due to the timing of batch production and a \$6.0 million decrease in contract research related to clinical trials.

*General and Administrative Expense.* General and administrative expense was \$15.7 million in 2010, a decrease of \$4.3 million or 22% from \$20.0 million in 2009. The variance was primarily attributable to lower personnel costs of \$2.2 million associated with the 2009 work force reduction and a decrease in legal and professional fees of \$1.6 million.

*Restructuring Charges.* Restructuring charges were \$1.5 million in 2009 due to the corporate restructuring implemented in the fourth quarter of 2009. The restructuring charges included \$0.9 million for employment termination costs payable in cash and a facilities consolidation restructuring charge of \$0.6 million, consisting of lease payments of \$0.5 million related to the net present value of the net future minimum lease payments at the cease-use date and the write-down of the net book value of fixed assets in the vacated building of \$0.1 million. There were no restructuring costs in 2010.

*Depreciation and Amortization.* Depreciation and amortization expense was \$2.1 million in both 2009 and 2010. There was no increase in depreciation and amortization expense due to less property, plant and equipment purchased in 2010 as compared to prior years.

*Interest Income and Interest Expense.* Interest income was \$0.2 million in 2010, compared to \$1.0 million in 2009. The decrease of \$0.8 million or 80% was due to lower average cash and cash equivalents balances throughout the year. Interest expense was \$0.3 million in both 2010 and 2009.

*Change in Fair Value of Warrant Liability.* In connection with the sale of our common stock and warrants from the registered direct offering in March 2010, we recorded the warrants as a liability at their fair value using a Black-Scholes model and will remeasure them at each reporting date until exercised or expired. Changes in the fair value of the warrants are reported in the statements of operations as non-operating income or expense. For the year ended December 31, 2010, we reported a loss of \$1.4 million related to the increase in fair value of these warrants from issuance dates. The market price for our common stock has been and may continue to be volatile. Consequently, future fluctuations in the price of our common stock may cause significant increases or decreases in the fair value of these warrants.



*Other Income/Expense.* Other Income increased due to funds received from the U.S. Treasury Department in 2010 of \$1.4 million for the Qualified Therapeutic Discovery Projects tax credit and grant program. Other expense increased during the year due to certain items from property and equipment being disposed of during the year resulting in a charge of \$0.1 million.

*Tax Benefit.* During 2010, we sold a portion of our New Jersey state net operating loss carry forwards, which resulted in the recognition of \$1.1 million in income tax benefits. Assuming the State of New Jersey continues to fund this program, which is uncertain, the future amount of net operating loss and research and development credit carry forwards which we may sell will also depend upon the allocation among qualifying companies of an annual pool established by the State of New Jersey.

*Net Operating Loss Carry forwards.* As of December 31, 2010, the Company had federal and state net operating loss carry forwards, or NOLs, of approximately \$71 million and \$133 million, respectively. The federal carry forward will begin to expire in 2026 and will end in 2031. The state carry forwards acquired prior to 2009, will begin to expire in 2013 and will end in 2017. Section 382 of the Internal Revenue Code of 1986, as amended, contains provisions which limit the amount of NOLs that companies may utilize in any one year in the event of cumulative changes in ownership over a three-year period in excess of 50%. We completed a detailed study of our NOLs and determined that as a result of our Registered Direct Offering in March 2010, there was an ownership change in excess of 50% and the federal NOLs subject to the 382 limitations were written down to their net realizable value. Additionally, we determined that the annual limitation on the utilization of the pre-ownership change loss will be approximately \$3.0 million. Ownership changes in future periods may place additional limits on our ability to utilize net operating loss and tax credit carry forwards.

### ***Year Ended December 31, 2009 Compared to Year Ended December 31, 2008***

*Research and Development Expense.* Research and development expense was \$48.1 million in 2009 representing an increase of \$10.3 million or 27% from \$37.8 million in 2008. The variance was primarily attributable to higher personnel costs of \$4.3 million associated with headcount growth prior to the 2009 work force reduction, a \$5.5 million increase in contract manufacturing costs due to the timing of batch production and a \$2.5 million increase in contract research related to clinical trials.

*General and Administrative Expense.* General and administrative expense was \$20.0 million in 2009, an increase of \$0.3 million or 2% from \$19.7 million in 2008. The variance was primarily attributable to higher personnel costs related to stock compensation expense of \$0.6 million and an increase in rent of \$0.2 million related to additional office space obtained in 2009, partially offset by a reduction in consulting fees.

*Restructuring Charges.* Restructuring charges were \$1.5 million in 2009 due to the corporate restructuring implemented in the fourth quarter of 2009. This measure was intended to reduce costs and to align the Company's resources with its key strategic priorities. The restructuring charges included \$0.9 million for employment termination costs payable in cash and a facilities consolidation restructuring charge of \$0.6 million, consisting of lease payments of \$0.5 million related to the net present value of the net future minimum lease payments at the cease-use date and the write-down of the net book value of fixed assets in the vacated building of \$0.1 million.

*Depreciation and Amortization.* Depreciation and amortization expense was \$2.1 million in 2009, an increase of \$0.6 million or 40%, from \$1.5 million in 2008 due to assets acquired in 2009.

*Interest Income and Interest Expense.* Interest income was \$1.0 million in 2009, compared to \$4.8 million in 2008. The decrease of \$3.8 million or 79% was due to lower average cash and cash equivalents balances and the decline in interest rates. Interest expense was \$0.3 million in 2009, compared to \$0.2 million in 2008. The increase of \$0.1 million or 50% was due to the secured loan obtained in June 2009.

## Liquidity and Capital Resources

### Source of Liquidity

As a result of our significant research and development expenditures and the lack of any approved products to generate product sales revenue, we have not been profitable and have generated operating losses since we were incorporated in 2002. We have funded our operations principally with \$148.7 million of proceeds from redeemable convertible preferred stock offerings, \$75.0 million of gross proceeds from our IPO in June 2007, \$18.5 million of gross proceeds from our Registered Direct Offering in March 2010, \$80.0 million from the non-refundable license fees from the collaboration agreements and \$31.0 million from GSK's investment in the Company at the time the collaboration was formed. In the future, we expect to fund our operations, in part, through the receipt of cost-sharing and milestone payments from GSK. The following table summarizes our significant funding sources as of December 31, 2010:

<b>Funding</b> <sup>(2)</sup>	<b>Year</b>	<b>No. Shares</b>	<b>Approximate Amount</b> <sup>(1)</sup> <b>(in thousands)</b>
Series A Redeemable Convertible Preferred Stock	2002	444,443	\$ 2,500
Series B Redeemable Convertible Preferred Stock	2004, 2005,	4,917,853	31,189
Series C Redeemable Convertible Preferred Stock	2006, 2007	5,820,020	54,999
Series D Redeemable Convertible Preferred Stock	2005, 2006	4,930,405	60,000
Common Stock	2006, 2007	5,000,000	75,000
Upfront License Fee from Shire	2007	—	50,000
Registered Direct Offering	2010	4,946,525	18,500
Upfront License Fee from GSK	2010	—	30,000
Common Stock — GSK	2010	6,866,245	31,285
		<u>32,925,491</u>	<u>\$ 353,473</u>

(1) Represents gross proceeds

(2) The Series A, B, C and D Redeemable Convertible Preferred Stock was converted to common stock upon the effectiveness of our IPO

In addition, in conjunction with the Shire collaboration agreement, we received reimbursement of research and development expenditures from the date of the agreement (November 7, 2007) through year-end 2009 of \$31.1 million.

As of December 31, 2010, we had cash and cash equivalents and marketable securities of \$107.4 million. We invest cash in excess of our immediate requirements with regard to liquidity and capital preservation in a variety of interest-bearing instruments, including obligations of U.S. government agencies and money market accounts. Wherever possible, we seek to minimize the potential effects of concentration and degrees of risk. Although we maintain cash balances with financial institutions in excess of insured limits, we do not anticipate any losses with respect to such cash balances. Our investment portfolio has not been materially adversely impacted by the recent disruption in the credit markets. However, if there is continued and expanded disruption in the credit markets, there can be no assurance that our investment portfolio will not be adversely affected in the future.

In March 2010, we sold 4.95 million shares of our common stock and warrants to purchase 1.85 million shares of common stock in a registered direct offering to a select group of institutional investors. The shares of common stock and warrants were sold in units consisting of one share of common stock and one warrant to purchase 0.375 shares of common stock at a price of \$3.74 per unit. The warrants have a term of four years and are exercisable any time on or after the six month anniversary of the date they were issued, at an exercise price of \$4.43 per share. The net proceeds of the offering were \$17.1 million after deducting the placement agency fee and all other estimated offering expenses.

In October 2010, we entered into a License and Collaboration Agreement with GSK, which included the sale of approximately 6.9 million shares of the Company's common stock to GSK at a price of \$4.56 per share. The total value of this equity investment to the Company is approximately \$31 million and represents a 19.9% ownership position in the Company.

### Net Cash Used in Operating Activities

Net cash used in operations for the year ended December 31, 2010 was \$14.0 million due primarily to the net loss for the year ended December 31, 2010 of \$54.9 million, partially offset by the change in operating assets and liabilities of \$31.2 million. The change in operating assets and liabilities of \$31.2 million was due primarily to deferred revenue related to the collaboration agreement with GSK.

Net cash used in operations for the year ended December 31, 2009 was \$43.4 million due primarily to the operating expenses for the year ended December 31, 2009 of \$71.7 million, partially offset by the reimbursed research and development costs of \$17.5 million.



### *Net Cash Used in and Provided by Investing Activities*

Net cash used in investing activities for the year ended December 31, 2010 was \$19.4 million. Net cash used in investing activities reflects \$94.6 million for the sale and redemption of marketable securities, offset by \$113.7 million for the purchase of marketable securities and \$0.4 million for the acquisition of property and equipment.

Net cash provided by investing activities for the year ended December 31, 2009 was \$31.9 million. Net cash provided by investing activities reflects \$131.8 million for the sale and redemption of marketable securities, offset by \$98.1 million for the purchase of marketable securities and \$1.8 million for the acquisition of property and equipment.

### *Net Cash Provided by and Used in Financing Activities*

Net cash provided by financing activities for the year ended December 31, 2010 was \$43.7 and reflects the \$17.1 million from the issuance of common stock and the \$28.1 million from common stock issued to GSK as part of the collaboration agreement. These cash inflows were partially offset by the payments of our secured loan agreement and capital lease obligations of \$1.3 million and \$0.3 million, respectively.

Net cash provided by financing activities for the year ended December 31, 2009 was \$2.8 million and reflected the proceeds of our secured loan agreement of \$3.7 million and \$0.1 million of proceeds from the exercise of stock options, partially offset by the payments of our capital lease obligations and secured loan agreement of \$0.8 million and \$0.2 million, respectively.

### *Funding Requirements*

We expect to incur losses from operations for the foreseeable future primarily due to research and development expenses, including expenses related to conducting clinical trials. Our future capital requirements will depend on a number of factors, including:

- the progress and results of our clinical trials of our drug candidates, including Amigal;
- our ability to achieve development and commercialization milestone payments and sales royalties under our collaboration with GSK;
- the scope, progress, results and costs of preclinical development, laboratory testing and clinical trials for our product candidates including those testing the use of pharmacological chaperones co-administered with ERT and for the treatment of diseases of neurodegeneration;
- the costs, timing and outcome of regulatory review of our product candidates;
- the number and development requirements of other product candidates that we pursue;
- the costs of commercialization activities, including product marketing, sales and distribution;
- the emergence of competing technologies and other adverse market developments;
- the costs of preparing, filing and prosecuting patent applications and maintaining, enforcing and defending intellectual property related claims;
- the extent to which we acquire or invest in businesses, products and technologies; and
- our ability to establish collaborations and obtain milestone, royalty or other payments from any such collaborators.

We do not anticipate that we will generate revenue from commercial sales for at least the next two years, if at all. In the absence of additional funding, we expect our continuing operating losses to result in increases in our cash used in operations over the next several quarters and years. However, we believe that our existing cash and cash equivalents and short-term investments, including anticipated payments from GSK in connection with the collaboration, is expected to be sufficient to fund our operating expenses and capital expenditure requirements through the anticipated commercial launch of Amigal in the United States.

### *Financial Uncertainties Related to Potential Future Payments*

#### *Milestone Payments*

We have acquired rights to develop and commercialize our product candidates through licenses granted by various parties. While our license agreements for Amigal and AT2220 do not contain milestone payment obligations, two of these agreements related to Plicera do require us to make such payments if certain specified pre-commercialization events occur. Upon the satisfaction of certain milestones and assuming successful development of Plicera, we may be obligated, under the agreements that we have in place, to make future milestone payments aggregating up to approximately \$7.9 million. However, such potential milestone payments are subject to many uncertain variables that would cause such payments, if any, to vary in size.



*Royalties*

Under our license agreements, if we owe royalties on net sales for one of our products to more than one licensor, then we have the right to reduce the royalties owed to one licensor for royalties paid to another. The amount of royalties to be offset is generally limited in each license and can vary under each agreement. For Amigal and AT2220, we will owe royalties only to Mt. Sinai School of Medicine (MSSM). We would expect to pay royalties to all three licensors with respect to Plicera should we advance Plicera to commercialization. To date, we have not made any royalty payments on sales of our products and believe we are at least a couple years away from selling any products that would require us to make any such royalty payments.

In accordance with our license agreement with MSSM, we paid \$3 million of the \$30 million upfront payment received from GSK to MSSM in the fourth quarter of 2010. We will also be obligated to pay MSSM royalties on worldwide net sales of Amigal.

Whether we will be obligated to make milestone or royalty payments in the future is subject to the success of our product development efforts and, accordingly, is inherently uncertain.

**Contractual Obligations**

The following table summarizes our significant contractual obligations and commercial commitments at December 31, 2010 and the effects such obligations are expected to have on our liquidity and cash flows in future periods (in thousands).

	<u>Total</u>	<u>Less than 1 Year</u>	<u>1-3 Years</u>	<u>3-5 Years</u>	<u>Over 5 Years</u>
Operating lease obligations	\$ 2,542	\$ 2,222	\$ 320	—	—
Capital lease obligations	40	40	—	—	—
Debt obligations	<u>2,297</u>	<u>1,253</u>	<u>1,044</u>	—	—
Total fixed contractual obligations (1)	<u>\$ 4,879</u>	<u>\$ 3,515</u>	<u>\$ 1,364</u>	<u>—</u>	<u>—</u>

(1) This table does not include (a) any milestone payments which may become payable to third parties under license agreements as the timing and likelihood of such payments are not known, (b) any royalty payments to third parties as the amounts of such payments, timing and/or the likelihood of such payments are not known, (c) amounts, if any, that may be committed in the future to construct additional facilities, and (d) contracts that are entered into in the ordinary course of business which are not material in the aggregate in any period presented above.

We lease office and laboratory space in Cranbury, New Jersey and these leases will expire by their terms by February 2012. In 2008, we leased office and laboratory space in San Diego, CA and this lease will expire by its terms in September 2011.

In May 2009, the Company entered into a loan and security agreement with Silicon Valley Bank that provides for up to \$4 million of equipment financing through October 2012. Borrowings under the loan agreement are collateralized by equipment purchased with the proceeds of the loan and bear interest at a fixed rate of approximately 9%.

On December 17, 2010, we amended the employment agreement with our president and chief executive officer that provides for an annual base salary, a cash bonus of up to 60% of base salary, and monthly payments up to a maximum of \$1.8 million for out-of-pocket medical expenses and the corresponding tax gross-up payments. The agreement will continue for successive one-year terms until either party provides written notice of termination to the other in accordance with the terms of the agreement.

We have entered into agreements with clinical research organizations and other outside contractors who are partially responsible for conducting and monitoring our clinical trials for our drug candidates including Amigal. These contractual obligations are not reflected in the table above because we may terminate them without penalty.

We have no other lines of credit or other committed sources of capital. To the extent our capital resources are insufficient to meet future capital requirements, we will need to raise additional capital or incur indebtedness to fund our operations. We cannot assure you that additional debt or equity financing will be available on acceptable terms, if at all.

**Off-Balance Sheet Arrangements**

We had no off-balance sheet arrangements as of December 31, 2009 and 2010.

## Recent Accounting Pronouncements

In April 2010, the Financial Accounting Standards Board (FASB) issued guidance on revenue recognition related to the milestone method of revenue recognition. This guidance provides criteria on defining a substantive milestone and determining when it may be appropriate to apply the milestone method of revenue recognition for research or development transactions. Early adoption is permitted retrospectively from the beginning of an entity's fiscal year. The Company early adopted this guidance on the milestone method of revenue recognition and retrospectively applied this guidance to the beginning of 2010. This method was first applied in conjunction with the License and Collaboration Agreement with GSK during the fourth quarter of 2010; there have been no milestones recognized in the year of adoption. This guidance did not have a material impact on the timing or pattern of revenue recognition relative to the agreement nor is expected to in future periods.

In January 2010, the FASB issued amendments to its fair value guidance which requires additional disclosures that include: 1) separate disclosures on significant transfers into and out of Level 3; 2) the amount of transfers between Level 1 and Level 2 and the reasons for such transfers; 3) lower level of disaggregation for fair value disclosures by class rather than by major category and 4) additional details on the valuation techniques and inputs used to determine Level 2 and Level 3 measurements. The Company has included these additional disclosures within the Form 10-K for the period ended December 31, 2010 and they did not have a significant impact on the financial statements disclosures of the Company.

In October 2009, the FASB issued guidance on revenue recognition related to multiple-element arrangements. This guidance requires companies to allocate revenue in multiple-element arrangements based on an element's estimated selling price if vendor-specific or other third party evidence of value is not available. This guidance is effective prospectively for revenue arrangements entered into or materially modified in fiscal years beginning on or after June 15, 2010. Early adoption is permitted retrospectively from the beginning of an entity's fiscal year. The Company adopted this guidance on revenue recognition in the fourth quarter of 2010 in conjunction with the License and Collaboration Agreement with GSK. The adoption of this guidance did not have a material impact on the timing or pattern of revenue recognition relative to the agreement.

## Item 7A. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK.

Market risk is the risk of change in fair value of a financial instrument due to changes in interest rates, equity prices, creditworthiness, financing, exchange rates or other factors. Our primary market risk exposure relates to changes in interest rates in our cash, cash equivalents and marketable securities. We place our investments in high-quality financial instruments, primarily money market funds, corporate debt securities, asset backed securities and U.S. government agency notes with maturities of less than one year, which we believe are subject to limited interest rate and credit risk. The securities in our investment portfolio are not leveraged, are classified as available-for-sale and, due to the short-term nature, are subject to minimal interest rate risk. We currently do not hedge interest rate exposure and consistent with our investment policy, we do not use derivative financial instruments in our investment portfolio. At December 31, 2010, we held \$107.4 million in cash, cash equivalents and available for sale securities and due to the short-term maturities of our investments, we do not believe that a 10% change in average interest rates would have a significant impact on our interest income. As December 31, 2010, our cash, cash equivalents and available for sale securities were all due on demand or within one year. Our outstanding debt has a fixed interest rate and therefore, we have no exposure to interest rate fluctuations.

We have operated primarily in the U.S., although we do conduct some clinical activities with vendors outside the U.S. While most expenses are paid in U.S. dollars, there are minimal payments made in local foreign currency. If exchange rates undergo a change of 10%, we do not believe that it would have a material impact on our results of operations or cash flows.

**Item 8. FINANCIAL STATEMENTS AND SUPPLEMENTARY DATA.**

**Management’s Report on Consolidated Financial Statements and Internal Control over Financial Reporting**

The management of Amicus Therapeutics, Inc. has prepared, and is responsible for the Company’s consolidated financial statements and related footnotes. These consolidated financial statements have been prepared in conformity with U.S. generally accepted accounting principles.

We are responsible for establishing and maintaining adequate internal control over financial reporting. Internal control over financial reporting is defined in Rule 13a-15(f) or 15d-15(f) promulgated under the Securities Exchange Act of 1934 as a process designed by, or under the supervision of the Company’s principal executive and principal financial officers and effected by the Company’s board of directors, management, and other personnel, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles and includes those policies and procedures that:

- pertain to the maintenance of records that in reasonable detail accurately and fairly reflect the transactions and dispositions of the assets of Amicus Therapeutics, Inc.;
- provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that receipts and expenditures of Amicus therapeutics, Inc. are being made only in accordance with authorizations of management and directors of Amicus therapeutics, Inc.; and
- provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use or disposition of the assets of Amicus Therapeutics, Inc. that could have a material effect on the financial statements.

Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Also, projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate.

We assessed the effectiveness of our internal control over financial reporting as of December 31, 2010. In making this assessment, we used the criteria set forth by the Committee of Sponsoring Organizations of the Treadway Commission (COSO) in Internal Control-Integrated Framework. Based on our assessment we believe that, as of December 31, 2010, our internal control over financial reporting is effective based on those criteria.

Dated March 4, 2011

/s/ John F. Crowley  
Chairman and Chief Executive Officer

/s/ Daphne Quimi  
Corporate Controller

**Report of Independent Registered Public Accounting Firm**

The Board of Directors and Stockholders  
Amicus Therapeutics Inc.

We have audited the accompanying consolidated balance sheets of Amicus Therapeutics, Inc. and subsidiary (a development stage company) as of December 31, 2010 and 2009, and the related consolidated statements of operations, stockholders' equity (deficiency) and cash flows for each of the three years in the period ended December 31, 2010 and the period from February 4, 2002 (inception) to December 31, 2010. These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on these financial statements based on our audits.

We conducted our audits in accordance with the standards of the Public Company Accounting Oversight Board (United States). Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement. We were not engaged to perform an audit of the Company's internal control over financial reporting. Our audits included consideration of internal control over financial reporting as a basis for designing audit procedures that are appropriate in the circumstances, but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion. An audit also includes examining, on a test basis, evidence supporting the amounts and disclosures in the financial statements, assessing the accounting principles used and significant estimates made by management, and evaluating the overall financial statement presentation. We believe that our audits provide a reasonable basis for our opinion.

In our opinion, the financial statements referred to above present fairly, in all material respects, the consolidated financial position of Amicus Therapeutics, Inc. at December 31, 2010 and 2009, and the consolidated results of their operations and their cash flows for each of the three years in the period ended December 31, 2010 and the period from February 4, 2002 (inception) to December 31, 2010, in conformity with U.S. generally accepted accounting principles.

/s/ Ernst & Young LLP

MetroPark, New Jersey  
March 4, 2011

**Amicus Therapeutics, Inc.**  
**(a development stage company)**  
**Consolidated Balance Sheets**  
**(in thousands, except share and per share amounts)**

	<u>December 31,</u>	
	<u>2009</u>	<u>2010</u>
<b>Assets:</b>		
Current assets:		
Cash and cash equivalents	\$ 19,339	\$ 29,572
Investments in marketable securities	58,885	77,873
Prepaid expenses and other current assets	<u>2,262</u>	<u>2,236</u>
Total current assets	80,486	109,681
Property and equipment, less accumulated depreciation and amortization of \$6,340 and \$8,095 at December 31, 2009 and 2010, respectively	4,399	2,604
Other non-current assets	<u>485</u>	<u>267</u>
<b>Total Assets</b>	<u><u>\$ 85,370</u></u>	<u><u>\$ 112,552</u></u>
<b>Liabilities and Stockholders' Equity</b>		
Current liabilities:		
Accounts payable and accrued expenses	\$ 9,635	\$ 8,290
Current portion of capital lease obligations	305	40
Current portion of deferred revenue	—	6,640
Current portion of secured loan	<u>1,253</u>	<u>1,253</u>
Total current liabilities	11,193	16,223
Deferred revenue, less current portion	—	25,639
Warrant liability	—	4,712
Secured loan, less current portion	2,296	1,044
Capital lease obligations, less current portion	48	—
Commitments and contingencies		
Stockholders' equity:		
Common stock, \$.01 par value, 50,000,000 shares authorized, 22,672,427 shares issued and outstanding at December 31, 2009, 50,000,000 shares authorized, 34,508,932 shares issued and outstanding at December 31, 2010	287	406
Additional paid-in capital	242,259	290,248
Accumulated other comprehensive income/(loss)	43	(28)
Deficit accumulated during the development stage	<u>(170,756)</u>	<u>(225,692)</u>
Total stockholders' equity	<u>71,833</u>	<u>64,934</u>
<b>Total Liabilities and Stockholders' Equity</b>	<u><u>\$ 85,370</u></u>	<u><u>\$ 112,552</u></u>

*See accompanying notes to consolidated financial statements*

**Amicus Therapeutics, Inc.**  
**(a development stage company)**  
**Consolidated Statements of Operations**  
**(in thousands, except share and per share amounts)**

	Years Ended December 31,			Period from
	2008	2009	2010	February 4, 2002 (Inception) to December 31, 2010
<b>Revenue:</b>				
Research revenue	\$ 12,189	\$ 17,545	\$ —	\$ 31,108
Collaboration revenue	2,778	46,813	922	50,922
Total revenue	<u>14,967</u>	<u>64,358</u>	<u>922</u>	<u>82,030</u>
<b>Operating Expenses:</b>				
Research and development	37,764	48,081	39,042	214,764
General and administrative	19,666	19,973	15,660	93,369
Restructuring charges	—	1,522	—	1,522
Impairment of leasehold improvements	—	—	—	1,030
Depreciation and amortization	1,493	2,132	2,058	8,478
In-process research and development	—	—	—	418
Total operating expenses	<u>58,923</u>	<u>71,708</u>	<u>56,760</u>	<u>319,581</u>
Loss from operations	(43,956)	(7,350)	(55,838)	(237,551)
<b>Other income (expenses):</b>				
Interest income	4,819	997	156	13,913
Interest expense	(218)	(278)	(260)	(2,185)
Change in fair value of warrant liability	—	—	(1,410)	(1,864)
Other income/(expense), net	—	64	1,277	161
Loss before income tax benefit	(39,355)	(6,567)	(56,075)	(227,526)
Income tax benefit	—	—	1,139	1,834
Net loss	(39,355)	(6,567)	(54,936)	(225,692)
Deemed dividend	—	—	—	(19,424)
Preferred stock accretion	—	—	—	(802)
Net loss attributable to common stockholders	<u>\$ (39,355)</u>	<u>\$ (6,567)</u>	<u>\$ (54,936)</u>	<u>\$ (245,918)</u>
Net loss attributable to common stockholders per common share — basic and diluted	<u>\$ (1.75)</u>	<u>\$ (0.29)</u>	<u>\$ (1.98)</u>	
Weighted-average common shares outstanding — basic and diluted	<u>22,493,803</u>	<u>22,624,134</u>	<u>27,734,797</u>	

**Amicus Therapeutics, Inc.**  
**(a development stage company)**  
**Consolidated Statements of Changes in Stockholders' (Deficiency)/Equity**  
**Period from February 4, 2002 (inception) to December 31, 2002,**  
**and the eight year period ended December 31, 2010**  
**(in thousands, except share amounts)**

	Common Stock		Additional Paid-In Capital	Other Comprehensive Gain/ (Loss)	Deferred Compensation	Deficit Accumulated During the Development Stage	Total Stockholders' (Deficiency) Equity
	Shares	Amount					
Balance at February 4, 2002 (inception)	—	\$ —	\$ —	\$ —	\$ —	\$ —	\$ —
Issuance of common stock to a consultant	74,938	6	78	—	—	—	84
Stock issued for in-process research and development	232,266	17	401	—	—	—	418
Deferred compensation	—	—	209	—	(209)	—	—
Amortization of deferred compensation	—	—	—	—	27	—	27
Issuance of warrants with financing arrangements	—	—	8	—	—	—	8
Accretion of redeemable convertible preferred stock	—	—	(11)	—	—	—	(11)
Net loss	—	—	—	—	—	(1,775)	(1,775)
Balance at December 31, 2002	307,204	23	685	—	(182)	(1,775)	(1,249)
Stock issued from exercise of stock options	333	—	—	—	—	—	—
Deferred compensation	—	—	14	—	(14)	—	—
Amortization of deferred compensation	—	—	—	—	70	—	70
Issuance of stock warrants with convertible notes	—	—	210	—	—	—	210
Issuance of stock options to consultants	—	—	4	—	—	—	4
Accretion of redeemable convertible preferred stock	—	—	(17)	—	—	—	(17)
Beneficial conversion feature related to bridge financing	—	—	41	—	—	—	41
Net loss	—	—	—	—	—	(6,768)	(6,768)
Balance at December 31, 2003	307,537	23	937	—	(126)	(8,543)	(7,709)
Deferred compensation	—	—	68	—	(68)	—	—
Amortization of deferred compensation	—	—	—	—	60	—	60
Issuance of stock options to consultants	—	—	16	—	—	—	16
Accretion of redeemable convertible preferred stock	—	—	(126)	—	—	—	(126)
Interest waived on converted convertible notes	—	—	193	—	—	—	193
Beneficial conversion feature related to bridge financing	—	—	95	—	—	—	95
Comprehensive Loss:							
Unrealized holding loss on available-for-sale securities	—	—	—	(9)	—	—	(9)
Net loss	—	—	—	—	—	(8,807)	(8,807)
Net total comprehensive loss	—	—	—	—	—	—	(8,816)
Balance at December 31, 2004	307,537	23	1,183	(9)	(134)	(17,350)	(16,287)
Stock issued from exercise of stock options	97,156	7	17	—	—	—	24
Stock issued from exercise of warrants	133,332	10	65	—	—	—	75
Deferred compensation	—	—	2,778	—	(2,778)	—	—
Amortization of deferred compensation	—	—	—	—	365	—	365
Non-cash charge for stock options to consultants	—	—	112	—	—	—	112
Accretion of redeemable convertible preferred stock	—	—	(139)	—	—	—	(139)
Comprehensive Loss:							
Unrealized holding loss on available-for-sale securities	—	—	—	(7)	—	—	(7)
Net loss	—	—	—	—	—	(19,972)	(19,972)
Net total comprehensive loss	—	—	—	—	—	—	(19,979)
Balance at December 31, 2005	538,025	\$ 40	\$ 4,016	\$ (16)	\$ (2,547)	\$ (37,322)	\$ (35,829)

**Amicus Therapeutics, Inc.**  
**(a development stage company)**  
**Consolidated Statements of Changes in Stockholders' (Deficiency) Equity**  
**Period from February 4, 2002 (inception) to December 31, 2002,**  
**and the eight year period ended December 31, 2010**  
**(in thousands, except share amounts)**

	Common Stock		Additional Paid-In Capital	Other Comprehensive Gain/ (Loss)	Deferred Compensation	Deficit Accumulated During the Development Stage	Total Stockholders' (Deficiency) Equity
	Shares	Amount					
Balance at December 31, 2005	538,025	\$ 40	\$ 4,016	\$ (16)	\$ (2,547)	\$ (37,322)	\$ (35,829)
Stock issued from exercise of options	265,801	20	138	—	—	—	158
Stock issued for license payment	133,333	10	1,210	—	—	—	1,220
Reversal of deferred compensation upon adoption of FAS 123(R)	—	—	(2,547)	—	2,547	—	—
Stock-based compensation	53,333	—	2,816	—	—	—	2,816
Issuance of stock options to consultants	—	—	476	—	—	—	476
Accretion of redeemable convertible preferred stock	—	—	(159)	—	—	—	(159)
Reclassification of warrant liability upon exercise of Series B redeemable convertible preferred stock warrants	—	—	117	—	—	—	117
Beneficial conversion on issuance of Series C redeemable convertible preferred stock	—	—	19,424	—	—	—	19,424
Beneficial conversion charge (deemed dividend) on issuance of Series C redeemable convertible preferred stock	—	—	(19,424)	—	—	—	(19,424)
Comprehensive (Loss)/ Income:							
Unrealized holding gain on available-for-sale securities	—	—	—	31	—	—	31
Net loss	—	—	—	—	—	(46,345)	(46,345)
Net total comprehensive loss	—	—	—	—	—	—	(46,314)
Balance at December 31, 2006	990,492	70	6,067	15	—	(83,667)	(77,515)
Stock issued from initial public offering	5,000,000	50	68,095	—	—	—	68,145
Stock issued from conversion of preferred shares	16,112,721	162	148,429	—	—	—	148,591
Stock issued from exercise of stock options, net	305,518	3	455	—	—	—	458
Stock based compensation	—	—	3,823	—	—	—	3,823
Issuance of stock options to consultants	—	—	162	—	—	—	162
Accretion of redeemable convertible preferred stock	—	—	(351)	—	—	—	(351)
Charge for warrant liability	—	—	758	—	—	—	758
Comprehensive (Loss)/ Income:							
Unrealized holding gain on available-for-sale securities	—	—	—	393	—	—	393
Net loss	—	—	—	—	—	(41,167)	(41,167)
Net total comprehensive loss	—	—	—	—	—	—	(40,774)
Balance at December 31, 2007	22,408,731	285	227,438	408	—	(124,834)	103,297
Stock issued from exercise of stock options, net	225,980	2	528	—	—	—	530
Stock based compensation	—	—	6,446	—	—	—	6,446
Comprehensive (Loss)/ Income:							
Unrealized holding gain on available-for-sale securities	—	—	—	125	—	—	125
Net loss	—	—	—	—	—	(39,355)	(39,355)
Net total comprehensive loss	—	—	—	—	—	—	(39,230)
Balance at December 31, 2008	22,634,711	\$ 287	\$ 234,412	\$ 533	\$ —	\$ (164,189)	\$ 71,043

**Amicus Therapeutics, Inc.**  
**(a development stage company)**  
**Consolidated Statements of Changes in Stockholders' (Deficiency) Equity**  
**Period from February 4, 2002 (inception) to December 31, 2002,**  
**and the eight year period ended December 31, 2010**  
**(in thousands, except share amounts)**

	Common Stock		Additional Paid-In Capital	Other Comprehensive Gain/ (Loss)	Deferred Compensation	Deficit Accumulated During the Development Stage	Total Stockholders' (Deficiency) Equity
	Shares	Amount					
Balance at December 31, 2008	22,634,711	\$ 287	\$ 234,412	\$ 533	\$ —	\$ (164,189)	\$ 71,043
Stock issued from exercise of stock options, net	37,716	—	60	—	—	—	60
Stock based compensation	—	—	7,787	—	—	—	7,787
Comprehensive (Loss)/ Income:							
Unrealized holding loss on available-for-sale securities	—	—	—	(490)	—	—	(490)
Net loss	—	—	—	—	—	(6,567)	(6,567)
Net total comprehensive loss	—	—	—	—	—	—	(7,057)
Balance at December 31, 2009	22,672,427	\$ 287	\$ 242,259	\$ 43	\$ —	\$ (170,756)	\$ 71,833
Stock issued from secondary offering	4,946,525	50	13,780	—	—	—	13,830
Stock issued from collaboration agreement	6,866,245	69	28,014	—	—	—	28,083
Stock issued from exercise of stock options, net	23,735	—	9	—	—	—	9
Stock based compensation	—	—	6,186	—	—	—	6,186
Comprehensive (Loss)/ Income:							
Unrealized holding loss on available-for-sale securities	—	—	—	(71)	—	—	(71)
Net loss	—	—	—	—	—	(54,936)	(54,936)
Net total comprehensive loss	—	—	—	—	—	—	(55,007)
Balance at December 31, 2010	34,508,932	\$ 406	\$ 290,248	\$ (28)	\$ —	\$ (225,692)	\$ 64,934

**Amicus Therapeutics, Inc.**  
**(a development stage company)**  
**Consolidated Statements of Cash Flows**  
**(in thousands)**

	Years Ended December 31,			Period from
	2008	2009	2010	February 4, 2002 (Inception) to December 31, 2010
<b>Operating activities</b>				
Net loss	\$ (39,355)	\$ (6,567)	\$ (54,936)	\$ (225,692)
Adjustments to reconcile net loss to net cash (used in)/provided by operating activities:				
Non-cash interest expense	—	—	—	525
Depreciation and amortization	1,493	2,132	2,058	8,478
Amortization of non-cash compensation	—	—	—	522
Stock-based compensation	6,446	7,787	6,186	27,059
Non-cash charge for stock based compensation issued to consultants	—	—	—	853
Change in fair value of warrant liability	—	—	1,410	1,864
Loss on disposal of asset	44	195	121	360
Stock-based license payment	—	—	—	1,220
Impairment of leasehold improvements	—	—	—	1,030
Non-cash charge for in process research and development	—	—	—	418
Beneficial conversion feature related to bridge financing	—	—	—	135
Changes in operating assets and liabilities:				
Prepaid expenses and other current assets	(949)	201	26	(2,237)
Other non-current assets	—	(218)	218	(288)
Account payable and accrued expenses	(1,669)	839	(1,345)	8,288
Deferred revenue	(2,873)	(47,740)	32,279	32,279
Net cash used in operating activities	(36,863)	(43,371)	(13,983)	(145,186)
<b>Investing activities</b>				
Sale and redemption of marketable securities	178,100	131,848	94,602	573,616
Purchases of marketable securities	(153,687)	(98,173)	(113,660)	(651,634)
Purchases of property and equipment	(2,667)	(1,807)	(384)	(12,469)
Net cash provided/(used in) by investing activities	21,746	31,868	(19,442)	(90,487)
<b>Financing activities</b>				
Proceeds from the issuance of preferred stock, net of issuance costs	—	—	—	143,022
Proceeds from issuance of common stock, net of issuance costs	—	—	45,214	113,307
Proceeds from the issuance of convertible notes	—	—	—	5,000
Payments of capital lease obligations	(1,528)	(840)	(313)	(5,547)
Payments of secured loan agreement	—	(209)	(1,252)	(1,461)
Proceeds from exercise of stock options	530	60	9	1,291
Proceeds from exercise of warrants (common and preferred)	—	—	—	264
Proceeds from capital asset financing arrangement	—	—	—	5,611
Proceeds from secured loan agreement	—	3,758	—	3,758
Net cash (used in)/provided by financing activities	(998)	2,769	43,658	265,245
Net (decrease)/increase in cash and cash equivalents	(16,115)	(8,734)	10,233	29,572
Cash and cash equivalents at beginning of year/ period	44,188	28,073	19,339	—
Cash and cash equivalents at end of year/period	\$ 28,073	\$ 19,339	\$ 29,572	\$ 29,572

**Supplemental disclosures of cash flow information**

Cash paid during the period for interest	\$	218	\$	250	\$	280	\$	1,884
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**Non-cash activities**

Conversion of preferred stock to common stock	\$	—	\$	—	\$	—	\$	148,951
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Conversion of notes payable to Series B redeemable convertible preferred stock	\$	—	\$	—	\$	—	\$	5,000
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Accretion of redeemable convertible preferred stock	\$	—	\$	—	\$	—	\$	802
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Beneficial conversion feature related to issuance of the additional issuance of Series C redeemable convertible preferred stock	\$	—	\$	—	\$	—	\$	19,424
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**Amicus Therapeutics, Inc.**  
**(a development stage company)**  
**Notes To Consolidated Financial Statements**

**1. Description of Business**

*Corporate Information, Status of Operations, and Management Plans*

Amicus Therapeutics, Inc. (the Company) was incorporated on February 4, 2002 in Delaware and is a biopharmaceutical company focused on the discovery, development and commercialization of orally-administered, small molecule drugs known as pharmacological chaperones for the treatment of rare diseases. Pharmacological chaperones are a novel, first-in-class approach to treating a broad range of diseases including lysosomal storage disorders and diseases of neurodegeneration. The Company's activities since inception have consisted principally of raising capital, establishing facilities, and performing research and development. Accordingly, the Company is considered to be in the development stage.

In October 2010, the Company entered into the License and Collaboration Agreement with Glaxo Group Limited, an affiliate of GlaxoSmithKline PLC (GSK), to develop and commercialize Amigal. Under the terms of the License and Collaboration Agreement, GSK received an exclusive worldwide license to develop, manufacture and commercialize Amigal. In consideration of the license grant, the Company received an upfront, license payment of \$30 million from GSK and is eligible to receive further payments of approximately \$170 million upon the successful achievement of development and commercialization milestones, as well as tiered double-digit royalties on global sales of Amigal. GSK and the Company will jointly fund development costs in accordance with an agreed upon development plan. For further information, see "— Note 12. Collaborative Agreements."

The Company had an accumulated deficit of approximately \$225.7 million at December 31, 2010 and anticipates incurring losses through the year 2011 and beyond. The Company has not yet generated commercial sales revenue and has been able to fund its operating losses to date through the sale of its redeemable convertible preferred stock, issuance of convertible notes, net proceeds from our initial public offering (IPO) and subsequent stock offerings, payments from partners during the terms of the collaboration agreements and other financing arrangements. In March 2010, the Company sold 4.95 million shares of its common stock and warrants to purchase 1.85 million shares of common stock in a registered direct offering to a select group of institutional investors for net proceeds of \$17.1 million. In October 2010, the Company sold 6.87 million shares of its common stock as part of the License and Collaboration Agreement with GSK for proceeds of \$31 million. The Company believes that its existing cash and cash equivalents and short-term investments will be sufficient to cover its cash flow requirements for 2011.

**2. Summary of Significant Accounting Policies**

*Basis of Presentation*

The accompanying consolidated financial statements have been prepared in accordance with United States (U.S.) generally accepted accounting principles (U.S. GAAP) and include all adjustments necessary for the fair presentation of the Company's financial position for the periods presented.

*Consolidation*

The financial statements include the accounts of Amicus Therapeutics, Inc. and its wholly owned subsidiary, Amicus Therapeutics UK Limited. All significant intercompany transactions and balances are eliminated in consolidation. This subsidiary is not material to the overall financial statements of the Company.

*Use of Estimates*

The preparation of financial statements in conformity with U.S. GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities, the disclosure of contingent assets and liabilities at the date of the financial statements, and the reported amounts of revenues and expenses during the reporting periods. Actual results could differ from those estimates.

*Cash, Cash Equivalents and Marketable Securities*

The Company considers all highly liquid investments purchased with a maturity of three months or less at the date of acquisition, to be cash equivalents.

**Amicus Therapeutics, Inc.**  
**(a development stage company)**  
**Notes To Consolidated Financial Statements — (Continued)**

Marketable securities consist of fixed income investments with a maturity of greater than three months and other highly liquid investments that can be readily purchased or sold using established markets. These investments are classified as available-for-sale and are reported at fair value on the Company's balance sheet. Unrealized holding gains and losses are reported within accumulated other comprehensive income/ (loss) as a separate component of stockholders' (deficiency) equity. Fair value is based on available market information including quoted market prices, broker or dealer quotations or other observable inputs. See "— Note 3. Cash, Cash Equivalents and Available-For-Sale Securities" for a summary of available-for-sale securities as of December 31, 2010 and 2009.

***Concentration of Credit Risk***

The Company's financial instruments that are exposed to concentration of credit risk consist primarily of cash and cash equivalents and marketable securities. The Company maintains its cash and cash equivalents in bank accounts, which, at times, exceed federally insured limits. The Company invests its marketable securities in high-quality commercial financial instruments. The Company has not recognized any losses from credit risks on such accounts during any of the periods presented. The Company believes it is not exposed to significant credit risk on cash and cash equivalents or its marketable securities.

***Property and Equipment***

Property and equipment are stated at cost, less accumulated depreciation and amortization. Depreciation is calculated over the estimated useful lives of the respective assets, which range from three to five years, or the lesser of the related initial term of the lease or useful life for leasehold improvements. Assets under capital leases are amortized over the terms of the related leases or their estimated useful lives, whichever is shorter.

The initial cost of property and equipment consists of its purchase price and any directly attributable costs of bringing the asset to its working condition and location for its intended use. Expenditures incurred after the fixed assets have been put into operation, such as repairs and maintenance, are charged to income in the period in which the costs are incurred. Major replacements, improvements and additions are capitalized in accordance with Company policy.

***Impairment of Long-Lived Assets***

The Company performs a review of long-lived assets for impairment when events or changes in circumstances indicate the carrying value of such assets may not be recoverable. If an indication of impairment is present, the Company compares the estimated undiscounted future cash flows to be generated by the asset to its carrying amount. If the undiscounted future cash flows are less than the carrying amount of the asset, the Company records an impairment loss equal to the excess of the asset's carrying amount over its fair value. The fair value is determined based on valuation techniques such as a comparison to fair values of similar assets or using a discounted cash flow analysis. There were no impairment charges recognized during the years ended December 31, 2008, 2009 and 2010.

***Revenue Recognition***

The Company recognizes revenue when amounts are realized or realizable and earned. Revenue is considered realizable and earned when the following criteria are met: (1) persuasive evidence of an arrangement exists; (2) delivery has occurred or services have been rendered; (3) the price is fixed or determinable; and (4) collection of the amounts due are reasonably assured.

In multiple element arrangements, revenue is allocated to each separate unit of accounting and each deliverable in an arrangement is evaluated to determine whether it represents separate units of accounting. A deliverable constitutes a separate unit of accounting when it has standalone value and there is no general right of return for the delivered elements. In instances when the aforementioned criteria are not met, the deliverable is combined with the undelivered elements and the allocation of the arrangement consideration and revenue recognition is determined for the combined unit as a single unit of accounting. Allocation of the consideration is determined at arrangement inception on the basis of each unit's relative selling price. In instances where there is determined to be a single unit of accounting, the total consideration is applied as revenue for the single unit of accounting and is recognized over the period of inception through the date where the last deliverable within the single unit of accounting is expected to be delivered.

**Amicus Therapeutics, Inc.**  
**(a development stage company)**  
**Notes To Consolidated Financial Statements — (Continued)**

The Company's current revenue recognition policies, which were applied in fiscal 2010, provide that, when a collaboration arrangement contains multiple deliverables, such as license and research and development services, the Company allocates revenue to each separate unit of accounting based on a selling price hierarchy. The selling price hierarchy for a deliverable is based on (i) its vendor specific objective evidence (VSOE) if available, (ii) third party evidence (TPE) if VSOE is not available, or (iii) estimated selling price (BESP) if neither VSOE nor TPE is available. The Company would establish the VSOE of selling price using the price charged for a deliverable when sold separately. The TPE of selling price would be established by evaluating largely similar and interchangeable competitor products or services in standalone sales to similarly situated customers. The best estimate of selling price would be established considering internal factors such as an internal pricing analysis or an income approach using a discounted cash flow model.

The revenue associated with reimbursements for research and development costs under collaboration agreements is included in Research Revenue and the costs associated with these reimbursable amounts are included in research and development expenses. The Company records these reimbursements as revenue and not as a reduction of research and development expenses as the Company has not commenced its planned principal operations (i.e., selling commercial products) and is a development stage enterprise, therefore development activities are part of its ongoing central operations.

The Company's collaboration agreement with GSK provides for, and any future collaborative agreements the Company may enter into also may provide for contingent milestone payments. In order to determine the revenue recognition for these contingent milestones, the Company evaluates the contingent milestones using the criteria as provided by the Financial Accounting Standards Boards (FASB) guidance on the milestone method of revenue recognition at the inception of a collaboration agreement. The criteria requires that (i) the Company determines if the milestone is commensurate with either its performance to achieve the milestone or the enhancement of value resulting from the Company's activities to achieve the milestone, (ii) the milestone be related to past performance, and (iii) the milestone be reasonable relative to all deliverable and payment terms of the collaboration arrangement. If these criteria are met then the contingent milestones can be considered as substantive milestones and will be recognized as revenue in the period that the milestone is achieved.

***Fair Value Measurements***

The Company records certain asset and liability balances under the fair value measurements as defined by the FASB guidance. Current FASB fair value guidance emphasizes that fair value is a market-based measurement, not an entity-specific measurement. Therefore, a fair value measurement should be determined based on the assumptions that market participants would use in pricing the asset or liability. As a basis for considering market participant assumptions in fair value measurements, current FASB guidance establishes a fair value hierarchy that distinguishes between market participant assumptions based on market data obtained from sources independent of the reporting entity (observable inputs that are classified within Levels 1 and 2 of the hierarchy) and the reporting entity's own assumptions that market participants assumptions would use in pricing assets or liabilities (unobservable inputs classified within Level 3 of the hierarchy).

Level 1 inputs utilize quoted prices (unadjusted) in active markets for identical assets or liabilities that the Company has the ability to access at measurement date. Level 2 inputs are inputs other than quoted prices included in Level 1 that are observable for the asset or liability, either directly or indirectly. Level 2 inputs may include quoted prices for similar assets and liabilities in active markets, as well as inputs that are observable for the asset or liability (other than quoted prices), such as interest rates, foreign exchange rates, and yield curves that are observable at commonly quoted intervals. Level 3 inputs are unobservable inputs for the asset or liability, which are typically based on an entity's own assumptions, as there is little, if any, related market activity. In instances where the determination of the fair value measurement is based on inputs from different levels of the fair value hierarchy, the level in the fair value hierarchy within which the entire fair value measurement falls is based on the lowest level input that is significant to the fair value measurement in its entirety. The Company's assessment of the significance of a particular input to the fair value measurement in its entirety requires judgment, and considers factors specific to the asset or liability.

***Research and Development Costs***

Research and development costs are expensed as incurred. Research and development expense consists primarily of costs related to personnel, including salaries and other personnel-related expenses, consulting fees and the cost of facilities and support services used in drug development. Assets acquired that are used for research and development and have no future alternative use are expensed as in-process research and development.

**Amicus Therapeutics, Inc.**  
**(a development stage company)**  
**Notes To Consolidated Financial Statements — (Continued)**

***Interest Income and Interest Expense***

Interest income consists of interest earned on the Company's cash and cash equivalents and marketable securities. Interest expense consists of interest incurred on capital leases and secured debt.

***Other Income and Expenses***

Other income includes funds received from the U.S. Treasury Department in 2010 for the Qualified Therapeutic Discovery Projects tax credit and grant program as well as a tax credit received from the Internal Revenue Service in 2009. Other expenses include costs directly attributable to a planned offering of the Company's securities that were subsequently withdrawn during 2006 and the losses on the disposal of certain fixed assets.

***Income Taxes***

The Company accounts for income taxes under the liability method. Under this method deferred income tax liabilities and assets are determined based on the difference between the financial statement carrying amounts and tax basis of assets and liabilities and for operating losses and tax credit carry forwards, using enacted tax rates in effect in the years in which the differences are expected to reverse. A valuation allowance is recorded if it is "more likely than not" that a portion or all of a deferred tax asset will not be realized.

***Other Comprehensive Income/ (Loss)***

Components of other comprehensive income/ (loss) include unrealized gains and losses on available-for-sale securities and are included in the statements of changes in stockholders' (deficiency) equity.

***Leases***

In the ordinary course of business, the Company enters into lease agreements for office space as well as leases for certain property and equipment. The leases have varying terms and expirations and have provisions to extend or renew the lease agreement, among other terms and conditions, as negotiated. Once the agreement is executed, the lease is assessed to determine whether the lease qualifies as a capital or operating lease.

When a non-cancelable operating lease includes any fixed escalation clauses and lease incentives for rent holidays or build-out contributions, rent expense is recognized on a straight-line basis over the initial term of the lease. The excess between the average rental amount charged to expense and amounts payable under the lease is recorded in accrued expenses.

***Stock-Based Compensation***

At December 31, 2010, the Company had three stock-based employee compensation plans, which are described more fully in "—Note 6. Stockholders' Equity." Until May 2007, the Company had one stock-based employee compensation plan. Stock-based employee compensation cost was recognized in the statement of operations for periods prior to January 1, 2006 to the extent options granted under the plan had an exercise price that was less than the fair market value of the underlying common stock on the date of grant. Effective January 1, 2006, The Company adopted the fair value method of measuring stock-based compensation, which requires a public entity to measure the cost of employee services received in exchange for an award of equity instruments based on the grant-date fair value of the award. Results for prior periods have not been restated.

**Amicus Therapeutics, Inc.**  
**(a development stage company)**  
**Notes To Consolidated Financial Statements — (Continued)**

The Company recognized stock-based compensation expense of \$6.4 million, \$7.8 million and \$6.2 million in 2008, 2009 and 2010, respectively. The following table summarizes information related to stock compensation expense recognized in the income statement (in millions):

	<b>Years Ended December 31,</b>		
	<b>2008</b>	<b>2009</b>	<b>2010</b>
Stock compensation expense recognized in:			
Research and development expense	\$ 2.5	\$ 3.2	\$ 2.6
General and administrative expense	3.9	4.6	3.6
Total stock compensation expense	<u>\$ 6.4</u>	<u>\$ 7.8</u>	<u>\$ 6.2</u>

***Basic and Diluted Net Loss Attributable to Common Stockholders per Common Share***

The Company calculates net loss per share as a measurement of the Company's performance while giving effect to all dilutive potential common shares that were outstanding during the reporting period. The Company has a net loss for all periods presented; accordingly, the inclusion of common stock options would be anti-dilutive. Therefore, the weighted average shares used to calculate both basic and diluted earnings per share are the same.

The following table provides a reconciliation of the numerator and denominator used in computing basic and diluted net loss attributable to common stockholders per common share (in thousands except share amounts):

	<b>Years Ended December 31,</b>		
	<b>2008</b>	<b>2009</b>	<b>2010</b>
<b>Historical</b>			
Numerator:			
Net loss attributable to common stockholders	<u>\$ (39,355)</u>	<u>\$ (6,567)</u>	<u>\$ (54,936)</u>
Denominator:			
Weighted average common shares outstanding — basic and diluted	<u>22,493,803</u>	<u>22,624,134</u>	<u>27,734,797</u>

Dilutive common stock equivalents would include the dilutive effect of common stock options for common stock equivalents. Potentially dilutive common stock equivalents totaled approximately 3.1 million, 4.8 million and 7.0 million for the years ended December 31, 2008, 2009 and 2010, respectively. Potentially dilutive common stock equivalents were excluded from the diluted earnings per share denominator for all periods because of their anti-dilutive effect.

***Dividends***

The Company has not paid cash dividends on its capital stock to date. The Company currently intends to retain its future earnings, if any, to fund the development and growth of the business and does not foresee payment of a dividend in any upcoming fiscal period.

**Amicus Therapeutics, Inc.**  
**(a development stage company)**  
**Notes To Consolidated Financial Statements — (Continued)**

***Recent Accounting Pronouncements***

In April 2010, the FASB issued guidance on revenue recognition related to the milestone method of revenue recognition. This guidance provides criteria on defining a substantive milestone and determining when it may be appropriate to apply the milestone method of revenue recognition for research or development transactions. Early adoption is permitted retrospectively from the beginning of an entity's fiscal year. The Company early adopted this guidance on the milestone method of revenue recognition and retrospectively applied this guidance to the beginning of 2010. This method was first applied in conjunction with the License and Collaboration Agreement with GSK during the fourth quarter of 2010; there have been no milestones recognized in the year of adoption. This guidance did not have a material impact on the timing or pattern of revenue recognition relative to the agreement nor is expected to in future periods.

In January 2010, the FASB issued amendments to its fair value guidance which requires additional disclosures that include: 1) separate disclosures on significant transfers into and out of Level 3; 2) the amount of transfers between Level 1 and Level 2 and the reasons for such transfers; 3) lower level of disaggregation for fair value disclosures by class rather than by major category and 4) additional details on the valuation techniques and inputs used to determine Level 2 and Level 3 measurements. The Company has included these additional disclosures within the Form 10-K for the period ended December 31, 2010 and they did not have a significant impact on the financial statements disclosures of the Company.

In October 2009, the FASB issued guidance on revenue recognition related to multiple-element arrangements. This new guidance requires companies to allocate revenue in multiple-element arrangements based on an element's estimated selling price if vendor-specific or other third party evidence of value is not available. This guidance is effective prospectively for revenue arrangements entered into or materially modified in fiscal years beginning on or after June 15, 2010. Early adoption is permitted retrospectively from the beginning of an entity's fiscal year. The Company adopted this guidance on revenue recognition in the fourth quarter of 2010 in conjunction with the License and Collaboration Agreement with GSK. The adoption of this guidance did not have a material impact on the timing or pattern of revenue recognition relative to the agreement.

***Segment Information***

The Company currently operates in one business segment focusing on the development and commercialization of small molecule, orally administered therapies to treat a range of human genetic diseases. The Company is not organized by market and is managed and operated as one business. A single management team reports to the chief operating decision maker who comprehensively manages the entire business. The Company does not operate any separate lines of business or separate business entities with respect to its products. Accordingly, the Company does not accumulate discrete financial information with respect to separate service lines and does not have separately reportable segments .

***Subsequent Events***

The Company evaluated events that occurred subsequent to December 31, 2010 and there were no material recognized or non-recognized subsequent events during this period.

**Amicus Therapeutics, Inc.**  
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**Notes To Consolidated Financial Statements — (Continued)**

**3. Cash, Cash Equivalents and Available-for-Sale Investments**

As of December 31, 2010, the Company held \$29.6 million in cash and cash equivalents and \$77.9 million of available-for-sale investment securities which are reported at fair value on the Company's balance sheet. Unrealized holding gains and losses are reported within accumulated other comprehensive income/(loss) as a separate component of stockholders' equity. If a decline in the fair value of a marketable security below the Company's cost basis is determined to be other than temporary, such marketable security is written down to its estimated fair value as a new cost basis and the amount of the write-down is included in earnings as an impairment charge. To date, only temporary impairment adjustments have been recorded.

Consistent with the Company's investment policy, the Company does not use derivative financial instruments in its investment portfolio. The Company regularly invests excess operating cash in deposits with major financial institutions, money market funds, notes issued by the U.S. government, as well as fixed income investments and U.S. bond funds both of which can be readily purchased and sold using established markets. The Company believes that the market risk arising from its holdings of these financial instruments is mitigated as many of these securities are either government backed or of the highest credit rating.

The Company's investment portfolio has not been materially adversely impacted by the recent disruption in the credit markets.

Cash and available for sale securities consisted of the following as of December 31, 2009 and December 31, 2010 (in thousands):

	<b>As of December 31, 2009</b>			
	<b>Cost</b>	<b>Unrealized Gain</b>	<b>Unrealized Loss</b>	<b>Fair Value</b>
Cash balances	\$ 19,339	\$ —	\$ —	\$ 19,339
U.S. government agency securities	45,020	44	(1)	45,063
Corporate debt securities	8,951	4	(7)	8,948
Commercial paper	4,521	3	—	4,524
Certificate of deposit	350	—	—	350
	<u>\$ 78,181</u>	<u>\$ 51</u>	<u>\$ (8)</u>	<u>\$ 78,224</u>
Included in cash and cash equivalents	\$ 19,339	\$ —	\$ —	\$ 19,339
Included in marketable securities	58,842	51	(8)	58,885
Total cash and available for sale securities	<u>\$ 78,181</u>	<u>\$ 51</u>	<u>\$ (8)</u>	<u>\$ 78,224</u>
	<b>As of December 31, 2010</b>			
	<b>Cost</b>	<b>Unrealized Gain</b>	<b>Unrealized Loss</b>	<b>Fair Value</b>
Cash balances	\$ 29,572	\$ —	\$ —	\$ 29,572
U.S. government agency securities	12,000	—	(9)	11,991
Corporate debt securities	42,075	2	(33)	42,044
Commercial paper	23,476	12	—	23,488
Certificate of deposit	350	—	—	350
	<u>\$ 107,473</u>	<u>\$ 14</u>	<u>\$ (42)</u>	<u>\$ 107,445</u>
Included in cash and cash equivalents	\$ 29,572	\$ —	\$ —	\$ 29,572
Included in marketable securities	77,901	14	(42)	77,873
Total cash and available for sale securities	<u>\$ 107,473</u>	<u>\$ 14</u>	<u>\$ (42)</u>	<u>\$ 107,445</u>

**Amicus Therapeutics, Inc.**  
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**Notes To Consolidated Financial Statements — (Continued)**

All of the Company's available for sale investments as of December 31, 2009 and December 31, 2010 are due in one year or less.

Unrealized gains and losses are reported as a component of accumulated other comprehensive gain/(loss) in stockholders' equity. For the year ended December 31, 2009, unrealized holding gains included in accumulated other comprehensive income was \$0.5 million. For the year ended December 31, 2010, unrealized holding gains included in accumulated other comprehensive income was \$0.1 million.

For the years ended December 31, 2009 and 2010, there were no realized gains or losses. The cost of securities sold is based on the specific identification method.

Unrealized loss positions in the available for sale securities as of December 31, 2009 and December 31, 2010 reflect temporary impairments that have been in a loss position for less than twelve months and as such are recognized in accumulated other comprehensive gain/(loss). The fair value of these available for sale securities in unrealized loss positions was \$7.8 million and \$46.1 million as of December 31, 2009 and December 31, 2010, respectively.

#### 4. Property and Equipment

Property and equipment consist of the following (in thousands):

	<u>December 31,</u>	
	<u>2009</u>	<u>2010</u>
Property and equipment consist of the following:		
Computer equipment	\$ 2,052	\$ 2,226
Computer software	1,102	694
Research equipment	4,737	4,834
Furniture and fixtures	703	726
Leasehold improvements	<u>2,145</u>	<u>2,219</u>
	10,739	10,699
Less accumulated depreciation and amortization	<u>(6,340)</u>	<u>(8,095)</u>
	<u>\$ 4,399</u>	<u>\$ 2,604</u>

Included in property and equipment are costs capitalized pursuant to capital lease obligations of \$6.9 million and \$3.7 million at December 31, 2009 and December 31, 2010, respectively. Depreciation and amortization expense relating to the capital lease obligations was \$1.1 million, \$1.9 million, \$1.7 million and \$6.9 million for the years ended December 31, 2008, 2009, and 2010, and for the Period February 4, 2002 (inception) to December 31, 2010, respectively.

**Amicus Therapeutics, Inc.**  
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**Notes To Consolidated Financial Statements — (Continued)**

**5. Accounts Payable and Accrued Expenses**

Accrued expenses consist of the following (in thousands):

	<b>December 31,</b>	
	<b>2009</b>	<b>2010</b>
Accounts payable	\$ 3,837	\$ 1,971
Accrued professional fees	411	181
Accrued contract manufacturing & contract research costs	1,901	2,782
Accrued compensation and benefits	2,557	2,912
Accrued facility costs	753	401
Accrued other	176	43
	<u>\$ 9,635</u>	<u>\$ 8,290</u>

**6. Stockholders' Equity***Common Stock*

As of December 31, 2010, the Company was authorized to issue 50,000,000 shares of common stock. Dividends on common stock will be paid when, and if declared by the board of directors. Each holder of common stock is entitled to vote on all matters and is entitled to one vote for each share held.

In October 2010 in connection with the License and Collaboration Agreement, GSK purchased approximately 6.9 million shares of the Company's common stock at \$4.56 per share, a 30% premium on the average price per share of the Company's stock over a 60 day period preceding the closing date of the transaction. The total value of this equity investment was approximately \$31 million and represents a 19.9% ownership position in the Company.

In March 2010, the Company sold 4,946,524 million shares of its common stock and warrants to purchase 1,854,946 million shares of common stock in a registered direct offering to a selected group of institutional investors through a Registration Statement on Form S-3 that was declared effective by the SEC on May 27, 2009. The shares of common stock and warrants were sold in units consisting of one share of common stock and one warrant to purchase 0.375 shares of common stock at a price of \$3.74 per unit. The warrants have a term of four years and are exercisable any time on or after the six month anniversary of the date they were issued, at an exercise price of \$4.43 per share. The aggregate offering proceeds were \$18.5 million. Leerink Swann LLC served as sole placement agent for the offering. The Company intends to use the net proceeds from the sale of the common stock and warrants for general corporate purposes and to further to advance the development of the Company's lead product candidate, Amigal, and the completion of certain activities required for the submission of a license application globally.

**Amicus Therapeutics, Inc.**  
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**Notes To Consolidated Financial Statements — (Continued)**

**Stock Option Plans**

In April 2002, the Company's Board of Directors and shareholders approved the Company's 2002 Stock Option Plan (the 2002 Plan). In May 2007, the Company's Board of Directors and shareholders approved the Company's 2007 Stock Option Plan (the 2007 Plan) and 2007 Director Option Plan (the 2007 Director Plan). In June 2010, the Company's Board of Directors and shareholders approved amendments to the 2007 Plan and the 2007 Director Plan. Both the 2002 Plan and 2007 Plan provide for the granting of restricted stock and options to purchase common stock in the Company to employees, advisors and consultants at a price to be determined by the Company's board of directors. The 2002 Plan and the 2007 Plan are intended to encourage ownership of stock by employees and consultants of the Company and to provide additional incentives for them to promote the success of the Company's business. The Options may be incentive stock options (ISOs) or non-statutory stock options (NSOs). Under the provisions of each plan, no option will have a term in excess of 10 years. The 2007 Director Plan is intended to promote the recruiting and retention of highly qualified eligible directors and strengthen the commonality of interest between directors and stockholders by encouraging ownership of common stock of the Company. The options granted under the 2007 Director Plan are NSOs and under the provisions of this plan, no option will have a term in excess of 10 years.

The Board of Directors, or its committee, is responsible for determining the individuals to be granted options, the number of options each individual will receive, the option price per share, and the exercise period of each option. Options granted pursuant to both the 2002 Plan and the 2007 Plan generally vest 25% on the first year anniversary date of grant plus an additional 1/48th for each month thereafter and may be exercised in whole or in part for 100% of the shares vested at any time after the date of grant. Options under the 2007 Director Plan may be granted to new directors upon joining the Board and vest in the same manner as options under the 2002 and 2007 Plans. In addition, options are automatically granted to all directors at each annual meeting of stockholders and vest on the date of the annual meeting of stockholders of the Company in the year following the year during which the options were granted.

As of December 31, 2010, there were no shares reserved for issuance under the 2002 Plan. The Company has reserved up to 1,867,756 shares for issuance under the 2007 Plan and the 2007 Director Plan.

The Company recognized stock-based compensation expense of \$6.4 million, \$7.8 million and \$6.2 million in 2008, 2009 and 2010, respectively. The following table summarizes information related to stock compensation expense recognized in the income statement (in millions):

	<b>Years Ended December 31,</b>		
	<b>2008</b>	<b>2009</b>	<b>2010</b>
Stock compensation expense recognized in:			
Research and development expense	\$ 2.5	\$ 3.2	\$ 2.6
General and administrative expense	3.9	4.6	3.6
Total stock compensation expense	<u>\$ 6.4</u>	<u>\$ 7.8</u>	<u>\$ 6.2</u>

Effective January 1, 2006, the Company adopted the fair value method of measuring stock-based compensation, which requires a public entity to measure the cost of employee services received in exchange for an award of equity instruments based upon the grant-date fair value of the award. The Company chose the "straight-line" attribution method for allocating compensation costs and recognized the fair value of each stock option on a straight-line basis over the vesting period of the related awards.

The Company uses the Black-Scholes option pricing model when estimating the fair value for stock-based awards. Use of a valuation model requires management to make certain assumptions with respect to selected model inputs. Expected volatility was calculated based on a blended weighted average of historical information of the Company's stock and the weighted average of historical information of similar public entities for which historical information was available. The Company will continue to use a blended weighted average approach using its own historical volatility and other similar public entity volatility information until the Company's historical volatility is relevant to measure expected volatility for future option grants. The average expected life was determined using the mid-point between the vesting date and the end of the contractual term. The risk-free interest rate is based on U.S. Treasury, zero-coupon issues with a remaining term equal to the expected life assumed at the date of grant. Forfeitures are estimated based on voluntary termination behavior, as well as a historical analysis of actual option forfeitures.

**Amicus Therapeutics, Inc.**  
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**Notes To Consolidated Financial Statements — (Continued)**

The weighted average assumptions used in the Black-Scholes option pricing model are as follows:

	<b>Years Ended December 31,</b>		
	<b>2008</b>	<b>2009</b>	<b>2010</b>
Expected stock price volatility	78.2%	80.6%	80.5%
Risk free interest rate	3.0%	2.4%	2.4%
Expected life of options (years)	6.25	6.25	6.25
Expected annual dividend per share	\$ 0.00	\$ 0.00	\$ 0.00

The weighted-average grant-date fair value per share of options granted during 2008, 2009 and 2010 were \$7.36, \$4.83 and \$2.09, respectively.

The following table summarizes information about stock options outstanding:

	<b>Number of Shares (in thousands)</b>	<b>Weighted Average Exercise Price</b>	<b>Weighted Average Remaining Contractual Life</b>	<b>Aggregate Intrinsic Value (in millions)</b>
Options outstanding, December 31, 2007	2,443.2	\$ 8.08		
Granted	965.2	\$ 10.49		
Exercised	(225.1)	\$ 2.48		
Forfeited	(106.0)	\$ 9.69		
Options outstanding, December 31, 2008	3,077.3	\$ 9.19		
Granted	2,352.0	\$ 6.88		
Exercised	(40.4)	\$ 2.03		
Forfeited	(570.0)	\$ 10.15		
Options outstanding, December 31, 2009	4,818.9	\$ 8.01	8.1 years	\$ 0.4
Granted	788.7	\$ 2.96		
Exercised	(25.9)	\$ 0.64		
Forfeited	(477.6)	\$ 7.93		
Options outstanding, December 31, 2010	5,104.1	\$ 7.27	7.5 years	\$ 2.1
Vested and unvested expected to vest, December 31, 2010	4,885.6	\$ 7.38	7.4 years	\$ 2.0
Exercisable at December 31, 2010	2,852.0	\$ 8.55	6.4 years	\$ 0.5

The aggregate intrinsic value of options exercised during the years ended December 31, 2008, 2009 and 2010, was \$1.9 million, \$0.1 million and \$0.1 million, respectively. As of December 31, 2010, the total unrecognized compensation cost related to non-vested stock options granted was \$7.1 million and is expected to be recognized over a weighted average period of 2.1 years. Cash proceeds from stock options exercised during the years ended December 31, 2008, 2009 and 2010 were \$0.6 million, \$0.1 million and \$0.02 million respectively.

*Restricted Stock Awards* — Restricted stock awards are granted subject to certain restrictions, including in some cases service conditions (restricted stock). The grant-date fair value of restricted stock awards, which has been determined based upon the market value of the Company's shares on the grant date, is expensed over the vesting period.

**Amicus Therapeutics, Inc.**  
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**Notes To Consolidated Financial Statements — (Continued)**

The following table summarizes information on the Company's restricted stock:

	<b>Restricted Stock</b>	
	<b>Number of Shares (in thousands)</b>	<b>Weighted Average Grant Date Fair Value</b>
Unvested at December 31, 2008	20.6	\$ 9.04
Granted	—	\$ —
Vested	(12.2)	\$ 8.97
Forfeited	—	\$ —
Unvested at December 31, 2009	8.4	\$ 9.15
Granted	—	\$ —
Vested	(8.4)	\$ 9.15
Forfeited	—	\$ —
Unvested at December 31, 2010	—	\$ —

Upon vesting in 2010, 2,203 shares were surrendered to fund minimum statutory tax withholding requirements. There were no restricted stock awards in 2010, 2009 or 2008. As of December 31, 2010, there was no unrecognized compensation cost related to unvested restricted stock awards. The total fair value of restricted stock awards which vested during 2010 was \$0.03 million.

#### **7. Assets and Liabilities Measured at Fair Value**

The Company's financial assets and liabilities are measured at fair value and classified within the fair value hierarchy which is defined as follows:

*Level 1* — Quoted prices in active markets for identical assets or liabilities that the Company has the ability to access at the measurement date.

*Level 2* — Inputs other than quoted prices in active markets that are observable for the asset or liability, either directly or indirectly.

*Level 3* — Inputs that are unobservable for the asset or liability.

#### ***Cash, Money Market Funds and Marketable Securities***

The Company classifies its cash and money market funds within the fair value hierarchy as Level 1 as these assets are valued using quoted prices in active market for identical assets at the measurement date. The Company considers its investments in marketable securities as available for sale and classifies these assets within the fair value hierarchy as Level 2 primarily utilizing broker quotes in a non-active market for valuation of these securities. No changes in valuation techniques or inputs occurred during the year ended December 31, 2010. No transfers of assets between Level 1 and Level 2 of the fair value measurement hierarchy occurred during the year ended December 31, 2010.

#### ***Secured Debt***

As disclosed in Note 13, the Company has a loan and security agreement with Silicon Valley Bank. The carrying amount of the Company's borrowings approximates fair value at December 31, 2010. The Company's secured debt is classified as Level 2 and the fair value is estimated using quoted prices for similar liabilities in active markets, as well as inputs that are observable for the liability (other than quoted prices), such as interest rates that are observable at commonly quoted intervals.

**Amicus Therapeutics, Inc.**  
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**Notes To Consolidated Financial Statements — (Continued)**

**Warrants**

The Company allocated \$3.3 million of proceeds from its March 2010 registered direct offering to warrants issued in connection with the offering that was classified as a liability. The valuation of the warrants is determined using the Black-Scholes model. This model uses inputs such as the underlying price of the shares issued when the warrant is exercised, volatility, risk free interest rate and expected life of the instrument. The Company has determined that the warrant liability should be classified within Level 3 of the fair value hierarchy by evaluating each input for the Black Scholes model against the fair value hierarchy criteria and using the lowest level of input as the basis for the fair value classification. There are six inputs: closing price of Amicus stock on the day of evaluation; the exercise price of the warrants; the remaining term of the warrants; the volatility of Amicus' stock over that term; annual rate of dividends; and the riskless rate of return. Of those inputs, the exercise price of the warrants and the remaining term are readily observable in the warrant agreements. The annual rate of dividends is based on the Company's historical practice of not granting dividends. The closing price of Amicus stock would fall under Level 1 of the fair value hierarchy as it is a quoted price in an active market. The riskless rate of return is a Level 2 input, while the historical volatility is a Level 3 input in accordance with the fair value accounting guidance. Since the lowest level input is a Level 3, the Company determined the warrant liability is most appropriately classified within Level 3 of the fair value hierarchy. This liability is subject to fair value mark-to-market adjustment each period. As a result, the Company recognized the change in the fair value of the warrant liability as non-operating expense of \$1.4 million for the year ended December 31, 2010. The resulting fair value of the warrant liability at December 31, 2010 was \$4.7 million.

A summary of the fair value of the Company's assets and liabilities aggregated by the level in the fair value hierarchy within which those measurements fall as of December 31, 2010 are identified in the following table (in thousands):

	<u>Level 1</u>	<u>Level 2</u>	<u>Total</u>	
<b>Assets:</b>				
Cash/Money market funds	\$ 29,572	\$ —	\$ 29,572	
U.S. government agency securities	—	11,991	11,991	
Commercial paper	—	42,044	42,044	
Corporate debt securities	—	23,488	23,488	
Certificate of deposit	—	350	350	
	<u>\$ 29,572</u>	<u>\$ 77,873</u>	<u>\$ 107,445</u>	
	<u>Level 1</u>	<u>Level 2</u>	<u>Level 3</u>	<u>Total</u>
<b>Liabilities:</b>				
Secured debt	\$ —	\$ 2,297	\$ —	\$ 2,297
Warrants liability	—	—	4,712	4,712
	<u>\$ —</u>	<u>\$ 2,297</u>	<u>\$ 4,712</u>	<u>\$ 7,009</u>

**8. 401(k) Plan**

The Company has a 401(k) plan (the Plan) covering all eligible employees. During 2007, the Board of Directors approved a company matching program that began on January 1, 2008. The matching program allows for a company match of up to 5% of salary and bonus paid during the year. The match vests 25% per year on a cliff vesting schedule over the first four years of employment for each participant. The Company's total contribution to the Plan was \$0.6 million and \$0.4 million for the years ended December 31, 2009 and 2010, respectively.

**9. Leases****Operating Leases**

The Company leases its facilities in Cranbury, NJ and these leases will expire in February 2012 or on such earlier date upon mutual agreement of both parties. In 2008, the Company entered into a lease agreement for its laboratory and office space in San Diego, CA, which will expire in September 2011.

**Amicus Therapeutics, Inc.**  
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**Notes To Consolidated Financial Statements — (Continued)**

At December 31, 2010, aggregate annual future minimum lease payments under these leases are as follows (in thousands):

Operating Leases	
Years ending December 31:	
2011	\$ 2,222
2012	320
2013	—
2014	—
	<u>\$ 2,542</u>

Rent expense for the years ended December 31, 2008, 2009 and 2010 were \$2.0 million, \$2.5 million and \$2.3 million, respectively.

#### **Capital Lease Facilities**

In August 2002, the Company entered into financing agreements that provide for up to \$1 million of equipment financing through August 2004. The facility was increased to \$3 million in May of 2005 and to \$5 million in November 2006. These financing arrangements include interest of approximately 9-12%, and lease terms of up to 48 months. Eligible assets under the lease lines include laboratory and scientific equipment, computer hardware and software, general office equipment, furniture, and leasehold improvements.

The remaining future minimum payments due for all non-cancelable capital leases as of December 31, 2010 are as follows (in thousands):

Capital Leases	
Years ending December 31:	
2011	\$ 41
2012	—
	<u>41</u>
Less payments for interest	(1)
Total principal obligation	<u>40</u>
Less short-term portion	<u>(40)</u>
Long-term portion	<u>\$ —</u>

The capital lease obligation is secured by the related assets financed by the leases.

#### **10. Income Taxes**

In June 2006, the FASB issued a single model to address accounting for uncertainty in tax positions. The model clarifies the accounting for income taxes, by prescribing a minimum recognition threshold a tax position is required to meet before being recognized in the financial statements. It also provides guidance on de-recognition, measurement, and classification of amounts relating to uncertain tax positions, accounting for and disclosure of interest and penalties, accounting in interim periods and disclosures required. The Company adopted the FASB requirements as of January 1, 2007 and determined that it did not have a material impact on the Company's financial position and results of operations. The Company did not recognize interest or penalties related to income tax during the period ended December 31, 2010 and did not accrue for interest or penalties as of December 31, 2010. The Company does not have an accrual for uncertain tax positions as of December 31, 2010. Tax returns for all years 2005 and thereafter are subject to future examination by tax authorities.

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**Notes To Consolidated Financial Statements — (Continued)**

Deferred income taxes reflect the net effect of temporary difference between the carrying amounts of assets and liabilities for financial reporting purposes and the amounts used for income tax purposes. The significant components of the deferred tax assets and liabilities are as follows (in thousands):

	<b>For Years Ended December 31,</b>		
	<b>2008</b>	<b>2009</b>	<b>2010</b>
<b>Current deferred tax asset</b>			
Non-cash stock issue	\$ 1,560	\$ 3,201	\$ 4,523
Others	141	229	163
	<u>1,701</u>	<u>3,430</u>	<u>4,686</u>
<b>Non-current deferred tax assets</b>			
Amortization/depreciation	2,682	3,271	4,070
Research tax credit	7,294	11,695	13,942
Net operating loss carry forwards	36,196	54,055	32,080
Deferred revenue	19,096	—	12,912
Others	518	257	834
	<u>67,487</u>	<u>72,708</u>	<u>68,524</u>
<b>Total deferred tax asset</b>	<b>67,487</b>	<b>72,708</b>	<b>68,524</b>
<b>Non-current deferred tax liability</b>	<u>—</u>	<u>—</u>	<u>—</u>
<b>Total net deferred tax asset</b>	<b>67,487</b>	<b>72,708</b>	<b>68,524</b>
Less valuation allowance	<u>(67,487)</u>	<u>(72,708)</u>	<u>(68,524)</u>
<b>Net deferred tax asset</b>	<b>\$ —</b>	<b>\$ —</b>	<b>\$ —</b>

The Company records a valuation allowance for temporary differences for which it is more likely than not that the Company will not receive future tax benefits. At December 31, 2008, 2009, and 2010, the Company recorded valuation allowances of \$67.5 million, \$72.7 million and \$68.5 million, respectively, representing an increase in the valuation allowance of \$5.2 million in 2009 and a decrease of \$4.2 million in 2010, due to the uncertainty regarding the realization of such deferred tax assets, to offset the benefits of net operating losses generated during those years.

As of December 31, 2010, the Company had federal and state net operating loss carry forwards (NOLs) of approximately \$71 million and \$133 million, respectively. The federal carry forward will begin to expire in 2026 and will end in 2031. The state carry forward acquired prior to 2009, will begin to expire in 2013 and will end in 2017. The state carry forward from 2010 will begin to expire in 2031 due to a change in the New Jersey state law regarding the net operating loss carry forward period. Utilization of NOLs may be subject to a substantial annual limitation in the event of an ownership change that has occurred previously or could occur in the future pursuant to Section 382 of the Internal Revenue Code of 1986, as amended, as well as similar state provisions. An ownership change may limit the amount of NOLs that can be utilized annually to offset future taxable income and tax, and may, in turn, result in the expiration of a portion of those carry forwards before utilization. In general, an ownership change, as defined by Section 382, results from transactions that increase the ownership of certain shareholders or public groups in the stock of a corporation by more than 50 percentage points over a three year period. The Company completed a detailed study of its NOLs and determined that as a result of the Registered Direct Offering in March 2010, there was an ownership change in excess of 50% and the federal NOLs subject to the 382 limitations were written down to their net realizable value. Additionally, the Company determined that the annual limitation on the utilization of the pre-ownership change loss will be approximately \$3.0 million.

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**Notes To Consolidated Financial Statements — (Continued)**

A reconciliation of the statutory tax rates and the effective tax rates for the years ended December 31, 2008, 2009 and 2010 are as follows:

	<b>Years Ended December 31,</b>		
	<b>2008</b>	<b>2009</b>	<b>2010</b>
Statutory rate	(34)%	(34)%	(34)%
State taxes, net of federal benefit	(5)	(3)	(8)
Permanent adjustments	2	19	51
R&D credit	(5)	(66)	(4)
Other	—	4	—
Valuation allowance	42	80	(7)
<b>Net</b>	<b>0%</b>	<b>0%</b>	<b>(2)%</b>

There was a federal benefit in 2009 from refundable research credits of approximately \$0.1 million. The Company recognized a tax benefit of \$1.1 million in connection with the sale of net operating losses in the New Jersey Transfer Program for the year ended December 31, 2010.

## 11. Licenses

The Company acquired rights to develop and commercialize its product candidates through licenses granted by various parties. The following summarizes the Company's material rights and obligations under those licenses:

*Mt. Sinai School of Medicine of New York University (MSSM)* — The Company acquired exclusive worldwide patent rights to develop and commercialize Amigal, Plicera and AT2220 and other pharmacological chaperones for the prevention or treatment of human diseases or clinical conditions by increasing the activity of wild-type and mutant enzymes pursuant to a license agreement with MSSM. In connection with this agreement, the Company issued 232,266 shares of common stock to MSSM in April 2002. In 2006, the Company amended its license agreement with MSSM to expand its exclusive worldwide patent rights to develop and commercialize pharmacological chaperones. In connection with the amendment, the Company paid \$1.0 million and issued 133,333 shares of its common stock with an estimated fair value of \$1.2 million to MSSM. In total, the Company recorded \$2.2 million of research and development expense in connection with the amendment in 2006. This agreement expires upon expiration of the last of the licensed patent rights, which will be in 2019, subject to any patent term extension that may be granted, or 2024 if we develop a product for combination therapy (pharmacological chaperone plus ERT) and a patent issues from the pending application covering combination therapy, subject to any patent term extension that may be granted. Under this agreement, to date the Company has paid no upfront or annual license fees and has no milestone or future payments other than royalties on net sales. On October 31, 2008, the Company amended and restated its license agreement with MSSM which consolidated previous amendments into a single agreement, clarified the portion of royalties and milestone payments the Company received from Shire that were payable to MSSM, and provided the Company with the sole right to control the prosecution of patent rights described in the amended and restated license agreement. Under the terms of the amended and restated license agreement, the Company agreed to pay \$2.6 million to MSSM in connection with the \$50 million upfront payment that the Company received from Shire in November 2007, which was already accrued for at December 31, 2007 and an additional \$2.6 million paid in the fourth quarter of 2008 for the sole right to and control over the prosecution of patent rights. In accordance with our license agreement with MSSM, the Company paid \$3 million of the \$30 million upfront payment received from GSK pursuant to the License and Collaboration Agreement to MSSM in December 2010. These payments to MSSM are classified as research and development expenses in the Company's financial statements.

**Amicus Therapeutics, Inc.**  
**(a development stage company)**  
**Notes To Consolidated Financial Statements — (Continued)**

*University of Maryland, Baltimore County* — The Company acquired exclusive U.S. patent rights to develop and commercialize Plicera for the treatment of Gaucher disease from the University of Maryland, Baltimore County. Under this agreement, the Company paid upfront and annual license fees of \$45 thousand, which were expensed as research and development expense. The Company is required to make a milestone payment upon the demonstration of safety and efficacy of Plicera for the treatment of Gaucher disease in a Phase 2 study, and another payment upon receiving FDA approval for Plicera for the treatment of Gaucher disease. Upon satisfaction of both milestones, the Company could be required to make up to \$0.2 million in aggregate payments. The Company is also required to pay royalties on net sales. This agreement expires upon expiration of the last of the licensed patent rights in 2015.

*Novo Nordisk A/S* — The Company acquired exclusive patent rights to develop and commercialize Plicera for all human indications. Under this agreement, to date the Company paid \$0.4 million in license fees which were expensed as research and development expense. The Company is also required to make milestone payments based on clinical progress of Plicera, with a payment due after initiation of a Phase 3 clinical trial for Plicera for the treatment of Gaucher disease, and a payment due upon each filing for regulatory approval of Plicera for the treatment of Gaucher disease in any of the US, Europe or Japan. An additional payment is due upon approval of Plicera for the treatment of Gaucher disease in the U.S. and a payment is also due upon each approval of Plicera for the treatment of Gaucher disease in either Europe or Japan. Assuming successful development of Plicera for the treatment of Gaucher disease in the U.S., Europe and Japan, total milestone payments would be \$7.8 million. The Company is also required to pay royalties on net sales. This license will terminate in 2016.

Under its license agreements, if the Company owes royalties on net sales for one of its products to more than one of the above licensors, then it has the right to reduce the royalties owed to one licensor for royalties paid to another. The amount of royalties to be offset is generally limited in each license and can vary under each agreement. For Amigal and AT2220, the Company will owe royalties only to MSSM and will owe no milestone payments. The Company would expect to pay royalties to all three licensors with respect to Plicera should we advance it to commercialization.

The Company's rights with respect to these agreements to develop and commercialize Amigal, Plicera and AT2220 may terminate, in whole or in part, if the Company fails to meet certain development or commercialization requirements or if the Company does not meet its obligations to make royalty payments.

## **12. Collaborative Agreements**

### *GSK*

On October 28, 2010, the Company entered into the License and Collaboration Agreement with Glaxo Group Limited, an affiliate of GSK, to develop and commercialize Amigal. Under the terms of the License and Collaboration Agreement, GSK received an exclusive worldwide license to develop, manufacture and commercialize Amigal. In consideration of the license grant, the Company received an upfront, license payment of \$30 million from GSK and is eligible to receive further payments of approximately \$170 million upon the successful achievement of development and commercialization milestones, as well as tiered double-digit royalties on global sales of Amigal. GSK and the Company will jointly fund development costs in accordance with an agreed upon development plan. This plan provides that the Company will fund 50% of the development costs for 2011 and 25% of the development costs in 2012 and beyond. The Company's development costs are subject to annual and aggregate caps. Additionally, GSK purchased approximately 6.9 million shares of the Company's common stock at \$4.56 per share, a 30% premium on the average price per share of the Company's stock over a 60 day period preceding the closing date of the transaction. The total value of this equity investment to the Company was approximately \$31 million and represents a 19.9% ownership position in the Company. Under the terms of the collaboration agreement, while the Company will collaborate with GSK, GSK will have decision-making authority over clinical, regulatory and commercial matters. Additionally, GSK will have primary responsibility for interactions with regulatory agencies and prosecuting applications for marketing and reimbursement approvals worldwide.

**Amicus Therapeutics, Inc.**  
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**Notes To Consolidated Financial Statements — (Continued)**

In accordance with the revenue recognition guidance related to multiple-element arrangements, the Company identified all of the deliverables at the inception of the agreement. The significant deliverables were determined to be the worldwide licensing rights to Amigal, the technology and “know how” transfer of Amigal development to date, the delivery of the Company’s common stock and the research services to continue and complete the development of Amigal. The Company determined that the worldwide licensing rights, the technology and “know how” transfer together with the research services represent one unit of accounting as none of these three deliverables on its own has standalone value separate from the other. The Company also determined that the delivery of the Company’s common stock does have standalone value separate from the worldwide licensing rights, the technology and “know how” transfer and the research services. As a result, the Company’s common stock is considered a separate unit of accounting and was accounted for as an issuance of common stock. However, as the Company’s common stock was sold at a premium to the market closing price, the premium amount paid over the market closing price was considered as additional consideration paid to the Company for the collaboration agreement and was included as consideration for the single unit of accounting identified above.

The total arrangement consideration which was allocated to the single unit of accounting identified above was \$33.2 million which consists of the upfront license payment of \$30 million and the premium over the closing market price of the common stock transaction of \$3.2 million. The Company will recognize this consideration as Collaboration Revenue on a straight-line basis over the development period of 5.2 years as included in the detailed development plan that was included in the collaboration agreement. The Company determined that the overall level of activity over the development period approximates a straight-line approach. At December 31, 2010, the Company recognized approximately \$0.9 million of the total arrangement consideration as Collaboration Revenue.

The Company evaluated the contingent milestones included in the collaboration agreement at the inception of the collaboration agreement and determined that the contingent milestones are substantive milestones and will be recognized as revenue in the period that the milestone is achieved. The Company determined that the research based milestones are commensurate with the enhanced value of each delivered item as a result of the Company’s specific performance to achieve the milestones. There is considerable effort underway to meet the specified milestones and complete the development of Amigal. Additionally, there is considerable time and effort involved in evaluating the data from the clinical trials that are planned and underway and if acceptable, in preparing the documentation required for filing for approval with the applicable regulatory authorities. The research based milestones would relate to past performances when achieved and are reasonable relative to the other payment terms within the collaboration agreement, including the \$30 million upfront payment and the cost sharing arrangement.

*Shire*

In November 2007, the Company entered into a collaboration agreement with Shire. Under the agreement, the Company and Shire were jointly developing the Company’s three lead pharmacological chaperone compounds for lysosomal storage disorders: Amigal, Plicera and AT2220. The Company granted Shire the rights to commercialize these products outside the U.S. and retained all rights to its other programs and to develop and commercialize Amigal, Plicera and AT2220 in the U.S.

The Company received an initial, non-refundable license fee payment of \$50 million from Shire. Joint development costs toward global approval of the three compounds were being shared 50/50. In addition, the Company was eligible to receive milestone payments if certain clinical and regulatory and sales-based milestones were met. The Company was also eligible to receive tiered double-digit royalties on net sales of the products marketed outside of the U.S.

In October 2009, the Company and Shire agreed to mutually terminate the collaboration agreement upon concluding that it was in their respective best interests to no longer collaborate on the development of the Company’s three lead pharmacological chaperone compounds for the treatment of lysosomal storage disorders. As a result of this termination, Amicus reacquired all global development and commercialization rights from Shire for these lead programs. Shire paid the Company \$5.2 million as full and final payment for amounts due to the Company under the collaboration agreement, and both parties were relieved of all other future obligations thereunder, financial or otherwise.

**Amicus Therapeutics, Inc.**  
**(a development stage company)**  
**Notes To Consolidated Financial Statements — (Continued)**

**13. Short-Term Borrowings and Long-Term Debt**

In May 2009, the Company entered into a loan and security agreement with Silicon Valley Bank that provides for up to \$4 million of equipment financing through October 2012. Borrowings under the loan agreement are collateralized by equipment purchased with the proceeds of the loan and bear interest at a fixed rate of approximately 9%. The loan agreement contains customary terms and conditions, including a financial covenant whereby the Company must maintain a minimum amount of liquidity measured at the end of each month equal to the greater of (i) \$30 million of unrestricted cash, cash equivalents, and marketable securities, or (ii) six months of trailing cash burn net of outstanding borrowings under the loan agreement. The Company has at all times been in compliance with this covenant during the term of the agreement.

At December 31, 2010, the current and long-term amounts due under the loan agreement were \$1.3 million and \$1.0 million, respectively. The carrying amount of the Company's borrowings approximates fair value at December 31, 2010.

The remaining future minimum payments due as of December 31, 2010 are as follows (in thousands):

Years ending December 31:	
2011	\$ 1,400
2012	1,080
2013	—
	<u>2,480</u>
Less payments for interest	(183)
Total principal obligation	2,297
Less short-term portion	<u>(1,253)</u>
Long-term portion	<u>\$ 1,044</u>

**14. Restructuring Charges**

In October 2009, the Company announced a work-force reduction of approximately 20 percent, or 26 employees, as a part of a corporate restructuring, with reductions occurring across all levels and organizations within the Company. This measure was intended to reduce costs and to align the Company's resources with its key strategic priorities. The Company recorded restructuring charges of \$0.9 million during the fourth quarter of 2009 for employment termination costs payable in cash in connection with the workforce reduction. There were no restructuring charges related to employment termination costs unpaid at December 31, 2010. There were no additional restructuring costs incurred in 2010.

In December 2009, the Company initiated and completed a facilities consolidation effort, closing one of its subleased locations in Cranbury, NJ. The Company recorded a charge of \$0.7 million during the fourth quarter of 2009 for minimum lease payments of \$0.5 million and the write-down of fixed assets in the facility.

The following table summarizes the restructuring charges and utilization for the year ended December 31, 2010 (in thousands):

	<b>Balance as of December 31, 2009</b>	<b>Charges</b>	<b>Cash Payments</b>	<b>Adjustments</b>	<b>Balance as of December 31, 2010</b>
Employment termination costs	\$ 271	\$ —	\$ (271)	\$ —	\$ —
Facilities consolidation	497	—	(229)	—	268
Total	<u>\$ 768</u>	<u>\$ —</u>	<u>\$ (500)</u>	<u>\$ —</u>	<u>\$ 268</u>

**Amicus Therapeutics, Inc.**  
**(a development stage company)**  
**Notes To Consolidated Financial Statements — (Continued)**

**15. Selected Quarterly Financial Data** (Unaudited — in thousands except per share data)

	<b>Quarters Ended</b>			
	<u>March 31</u>	<u>June 30</u>	<u>September 30</u>	<u>December 31</u>
<b>2009</b>				
Net (loss)/income (2)	(12,472)	(13,623)	(13,429)	32,956
Basic net (loss)/income per common share (1)	(0.55)	(0.60)	(0.59)	1.46
Diluted net (loss)/income per common share (1)	(0.55)	(0.60)	(0.59)	1.45
<b>2010</b>				
Net loss	(13,176)	(11,315)	(15,357)	(15,088)
Basic and diluted net loss per common share (1)	(0.54)	(0.41)	(0.56)	(0.48)

- (1) Per common share amounts for the quarters and full years have been calculated separately. Accordingly, quarterly amounts do not add to the annual amounts because of differences on the weighted-average common shares outstanding during each period principally due to the effect of the Company issuing shares of its common stock during the year.
- (2) Net income for the quarter ended December 31, 2009 was primarily due to the termination of the collaboration agreement with Shire and the resulting recognition of the balance of deferred revenue of \$44.7 million.

**Item 9. CHANGES IN AND DISAGREEMENTS WITH ACCOUNTANTS ON ACCOUNTING AND FINANCIAL DISCLOSURE.**

None.

**Item 9A. CONTROLS AND PROCEDURES.**

**Evaluation of Disclosure Controls and Procedures**

Our management, with the participation of our principal executive officer and principal financial officer, evaluated the effectiveness of our disclosure controls and procedures as of December 31, 2010. The term “disclosure controls and procedures,” as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act, means controls and other procedures of a company that are designed to ensure that information required to be disclosed by us in the reports that we file or submit under the Exchange Act is recorded, processed, summarized and reported within the time periods specified in the SEC rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to the company’s management, including its principal executive and principal financial officers, as appropriate to allow timely decisions regarding required disclosure. Management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Based on the evaluation of our disclosure controls and procedures as of December 31, 2010, our principal executive officer and principal financial officer concluded that, as of such date, our disclosure controls and procedures were effective at the reasonable assurance level.

There have been no changes in our internal controls over financial reporting during the fourth quarter of the year ended December 31, 2010 that have materially affected, or are reasonably likely to materially affect, our internal controls over financial reporting.

**Management’s Report on Internal Control Over Financial Reporting**

Our management is responsible for establishing and maintaining adequate internal control over financial reporting as defined in Rules 13a-15(f) and 15d-15(f) under the Securities Exchange Act of 1934, as amended. Our internal control system was designed to provide reasonable assurance to our management and board of directors regarding the reliability of financial reporting and the preparation of the consolidated financial statements in accordance with U.S. generally accepted accounting principles. Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate.

Our management assessed the effectiveness of our internal control over financial reporting as of December 31, 2010, using the criteria set forth by the Committee of Sponsoring Organizations of the Treadway Commission (COSO) in Internal Control-Integrated Framework. Management’s assessment included an evaluation of the design of our internal control over financial reporting and testing of the operational effectiveness of those controls. Based on this evaluation, management has concluded that our internal control over financial reporting was effective as of December 31, 2010.

**Item 9B. OTHER INFORMATION.**

None.

### PART III

Certain information required by Part III is omitted from this Annual Report on Form 10-K as we intend to file our definitive proxy statement for our 2010 annual meeting of stockholders, pursuant to Regulation 14A of the Securities Exchange Act, not later than 120 days after the end of the fiscal year covered by this Annual Report of Form 10-K, and certain information to be included in the proxy statement is incorporated herein by reference.

#### **Item 10. DIRECTORS, EXECUTIVE OFFICERS OF THE REGISTRANT AND CORPORATE GOVERNANCE.**

##### **Executive Officers**

The following table sets forth certain information regarding our current executive officers as of February 23, 2011.

*John F. Crowley* has served as Chairman and Chief Executive Officer since February 2011, Chairman, President and Chief Executive Officer since February 2010 and President and Chief Executive Officer since January 2005, and has also served as a Director of Amicus since August 2004, with the exception of the period from September 2006 to March 2007 when he was not an officer or director of Amicus while he was in active duty service in the United States Navy (Reserve). He was President and Chief Executive Officer of Orexigen Therapeutics, Inc. from September 2003 to December 2004. Mr. Crowley was President and Chief Executive Officer of Novazyme Pharmaceuticals, Inc., from March 2000 until that company was acquired by Genzyme Corporation in September 2001; thereafter he served as Senior Vice President of Genzyme Therapeutics until December 2002. Mr. Crowley received a B.S. degree in Foreign Service from Georgetown University's School of Foreign Service, a J.D. from the University of Notre Dame Law School, and an M.B.A. from Harvard Business School.

*Matthew R. Patterson* has served as President and Chief Operating Officer since February 2011 and Chief Operating Officer since September 2006. From December 2004 to September 2006 he served as Chief Business Officer. From 1998-2004, Mr. Patterson worked at BioMarin Pharmaceutical Inc. where he was Vice President, Regulatory and Government Affairs from 2001 to 2003 and later Vice President, Commercial Planning from 2003-2004. From 1993-1998, Mr. Patterson worked in various roles at Genzyme Corporation in Regulatory Affairs and Manufacturing. Mr. Patterson received a B.A. in Biochemistry from Bowdoin College.

*David J. Lockhart*, Ph.D., has served as Chief Scientific Officer since January 2006. Prior to joining Amicus, Dr. Lockhart served as President, Chief Scientific Officer and co-founder of Ambit Biosciences, a biotechnology company specializing in small molecule kinase inhibitors, from March 2001 to July 2005. Dr. Lockhart served as a consultant to Ambit Biosciences from August 2000 to March 2001, and as a visiting scholar at the Salk Institute for Biological Studies from October 2000 to March 2001. Prior to that, Dr. Lockhart served in various positions, including Vice President of Genomics Research at Affymetrix, and was the Director of Genomics at the Genomics Institute of the Novartis Research Foundation from February 1999 to July 2000. He received his Ph.D. from Stanford University and was a post-doctoral fellow at the Whitehead Institute for Biomedical Research at the Massachusetts Institute of Technology.

*S. Nicole Schaeffer* has served as Senior Vice President, Human Resources and Leadership Development since August 2008, and, prior thereto, as Vice President, Human Resources and Leadership Development since March 2005. From 2001 to 2004, she served as Senior Director, Human Resources, for three portfolio companies of Flagship Ventures, a venture capital firm, and in that capacity she managed human resources for three life sciences companies. Ms. Schaeffer received her B.A. from the University of Rochester and her M.B.A. from Boston University.

*Bradley L. Campbell* has served as Senior Vice President, Business Operations since January 2010. From May 2007 to January 2010, he served as Vice President, Business Planning and from April 2006 until May 2007, he served as Senior Director, Business Development. From 2002 until 2006, Mr. Campbell served as Senior Product Manager and later Business Director of CV Gene Therapy at Genzyme Corporation. Mr. Campbell received his B.A. from Duke University and his M.B.A. from Harvard Business School.

*John R. Kirk* has served as Vice President, Regulatory Affairs since January 1, 2008. Prior to joining Amicus, Mr. Kirk served as Executive Director, Regulatory Affairs at Aegerion Pharmaceuticals. From 2003 to 2007, Mr. Kirk held positions of increasing responsibility with Esperion Therapeutics which was acquired during this time by Pfizer. From 2000 to 2002, Mr. Kirk was Director, Worldwide Regulatory Affairs for Pfizer Global Research and Development. From 1988 to 2000, Mr. Kirk held various Regulatory positions with Parke-Davis Pharmaceutical Research. Mr. Kirk holds both his M.S. and B.S. from Wright State University in Ohio.

*Geoffrey P. Gilmore* has served as Senior Vice President, General Counsel and Secretary since March 2008. Prior to joining Amicus, from 2003 to 2008, Mr. Gilmore was in the Law Department at Bristol-Myers Squibb Company, where most recently he served as Vice President and Senior Counsel. From 2002 to 2003, Mr. Gilmore was a Senior Attorney at Wyeth Pharmaceuticals. From 1997 to 2002, Mr. Gilmore held various positions in the law department of Bristol Myers Squibb Company. Prior to joining Bristol-Myers Squibb Company, Mr. Gilmore was an associate with the law firms, Ballard Spahr Andrews & Ingersoll, LLP, where he practiced in the Business and Finance Group, and Montgomery, McCracken, Walker & Rhoads, LLP, where he practiced in the Corporate & Securities Group. Mr. Gilmore received his B.A. from Franklin and Marshall College, and his J.D. from University of Michigan Law School.



*Pol F. Boudes, M.D.*, has served as Chief Medical Officer since January 2009. Prior to joining Amicus, from 2004 to 2009, Dr. Boudes served as Vice President, Global Clinical Development Women's Health Care US at Bayer HealthCare Pharmaceuticals (formerly Berlex, Inc.). From 1990 to 2004, Dr. Boudes served in positions of increasing responsibility with the Wyeth-Ayerst Research division of Wyeth both in Philadelphia, PA and in Europe, with Hoffmann-La Roche, and with Pasteur Merieux serums & vaccines (now sanofi-aventis). Dr. Boudes received his M.D. from the University of Aix-Marseilles, France, completed his internship and residency in Marseilles and in Paris, France and was an Assistant Professor of Medicine at the University of Paris. Dr. Boudes is specialized in Endocrinology and Metabolic Diseases, Internal Medicine, and Geriatric diseases. Dr. Boudes practiced medicine in this capacity in academic hospitals in France where he also participated in multiple clinical research programs as an investigator.

*Enrique Diloné, Ph.D., RAC*, has served as Vice President, Technical Operations since January 2011. From August 2009 to January 2011, he served as Senior Director, Quality Control and Analytical Chemistry. Prior to joining Amicus, Dr. Diloné served as Executive Director of Quality and Analytics at NovaDel Pharma, a specialty pharmaceutical company developing oral spray formulations, from February 2007 to August 2009. Dr. Diloné served as Senior Director/Director of Analytical Operations at OSI/Eyeteck Pharmaceuticals from February 2002 to December 2006. He received a Ph.D. and an M.S., both in Chemistry, from Seton Hall University, and a B.A. in Chemistry from New York University. He is also certified in US Regulatory Affairs.

*Ken Valenzano, Ph.D.*, has served as Vice President, Pharmacology since May 2010. From July 2005 to May 2010, he served as Senior Director and Director, Pharmacology. Prior to joining Amicus, Dr. Valenzano served in a variety of scientific leadership roles at Purdue Pharma from 1999-2005. He received a Ph.D. from the joint Pharmacology program of Rutgers University and University of Medicine and Dentistry of NJ, Robert Wood Johnson Medical School in 1995. He received a B.S. in Biology from Villanova University.

*Kenneth W. Peist, Esq.*, has served as Vice President, Intellectual Property since January 2011 and, prior thereto, as Senior Director, Intellectual Property since December 2007. From 1998 to 2007, he held a variety of legal positions at Bristol-Myers Squibb Co., Vitae Pharmaceuticals and ExxonMobil. Mr. Peist received his J.D. from Seton Hall University School of Law in 1998 and a B.S. from Old Dominion University in 1986.

The other information required by this item is incorporated by reference from the definitive proxy statement which Amicus will file with the Securities and Exchange Commission no later than 120 days after December 31, 2010 (the "Proxy Statement"), under the captions "Election of Directors" and "Section 16(a) Beneficial Ownership Reporting Compliance."

In 2007, we adopted a Code of Business Ethics and Conduct for Employees, Executive Officers and Directors that applies to our employees, officers and directors and incorporate guidelines designed to deter wrongdoing and to promote the honest and ethical conduct and compliance with applicable laws and regulations. In addition, the code of ethics incorporates our guidelines pertaining to topics such as conflicts of interest and workplace behavior. We have posted the text of our code on our website at [www.amicustherapeutics.com](http://www.amicustherapeutics.com) in connection with "Investors/Corporate Governance" materials. In addition, we intend to promptly disclose (1) the nature of any amendment to our code of ethics that applies to our principal executive officer, principal financial officer, principal accounting officer or controller, or persons performing similar functions and (2) the nature of any waiver, including an implicit waiver, from provision of our code of ethics that is granted to one of these specified officers, the name of such person who is granted the waiver and the date the waiver on our website in the future.

#### **Item 11. EXECUTIVE COMPENSATION.**

The information required by this item is incorporated by reference from the Proxy Statement under the caption "Executive Compensation — Compensation Discussion and Analysis."

**Item 12. SECURITY OWNERSHIP OF CERTAIN BENEFICIAL OWNERS AND MANAGEMENT AND RELATED STOCKHOLDER MATTERS.**

The information required by this item is incorporated by reference from the Proxy Statement under the captions “Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters” and “Equity Compensation Plan Information.”

**Item 13. CERTAIN RELATIONSHIPS AND RELATED TRANSACTIONS AND DIRECTOR INDEPENDENCE.**

The information required by this item is incorporated by reference from the Proxy Statement under the captions “Certain Relationships and Related Transactions,” “Director Independence,” “Committee Compensation and Meetings of the Board of Directors,” and “Compensation Committee Interlock and Insider Participation.”

**Item 14. PRINCIPAL ACCOUNTING FEES AND SERVICES.**

The information required by this item is incorporated by reference from the Proxy Statement under the caption “Ratification of Independent Registered Public Accounting Firm.”

**PART IV**

**Item 15. EXHIBITS, FINANCIAL STATEMENT SCHEDULE**

(a) 1. *Consolidated Financial Statements*

The Consolidated Financial Statements are filed as part of this report.

2. *Consolidated Financial Statement Schedules*

All schedules are omitted because they are not required or because the required information is included in the Consolidated Financial Statements or notes thereto.

3. *Exhibits*

Exhibit No.	Filed Exhibit Description	Incorporated by Reference to SEC Filing			Filed with this Form 10-K
		Form	Date	Exhibit No.	
3.1	Restated Certificate of Incorporation of the Registrant.	S-1 (333-141700)	5/17/07	3.2	
3.2	Restated By-laws of the Registrant.	S-1/A (333-141700)	4/27/07	3.4	
4.1	Specimen Stock Certificate evidencing shares of common stock	S-1 (333-141700)	3/30/07	4.1	
4.2	Third Amended and Restated Investor Rights Agreement, dated as of September 13, 2006, as amended	S-1 (333-141700)	3/30/07	4.3	
4.3	Form of Warrant	Form 8-K	2/25/10	4.1	
10.1	2002 Equity Incentive Plan, as amended, and forms of option agreements thereunder	S-1/A (333-141700)	4/27/07	10.1	
+ 10.2	Amended and Restated License Agreement, dated October, 31, 2008, by and between the Registrant and Mount Sinai School of Medicine of New York University	Form 10-K	2/6/09	10.3	
+ 10.3	License Agreement, dated as of June 26, 2003, by and between the Registrant and University of Maryland, Baltimore County, as amended	S-1 (333-141700)	3/30/07	10.4	
+ 10.4	Exclusive License Agreement, dated as of June 8, 2005, by and between the Registrant and Novo Nordisk, A/S	S-1 (333-141700)	3/30/07	10.5	
10.5	Sublease Agreement, dated as of May 12, 2005, by and between the Registrant and Purdue Pharma, L.P.	S-1 (333-141700)	3/30/07	10.6	
10.6	Amended and Restated Employment Agreement, dated as of December 17, 2010, by and between the Registrant and John F. Crowley	Form 8-K Current Report	12/21/10	10.1	
10.7	Letter Agreement, dated as of November 9, 2004, by and between the Registrant and Matthew R. Patterson	S-1 (333-141700)	3/30/07	10.8	
10.8	Letter Agreement, dated as of December 19, 2005, by and between the Registrant and David Lockhart, Ph.D.	S-1 (333-141700)	3/30/07	10.10	
10.9	Form of Director and Officer Indemnification Agreement	S-1 (333-141700)	3/30/07	10.17	
10.10	Restricted Stock Agreement, dated as of March 8, 2007, by and between the Registrant and James E. Dentzer	S-1/A (333-141700)	4/27/07	10.20	
10.11	Restricted Stock Agreement, dated as of March 8, 2007, by and between the Registrant and Glenn P. Sblendorio	S-1/A (333-141700)	4/27/07	10.21	
10.12	Lease Agreement, dated as of July 31, 2006, by and between the Registrant and Cedar Brook II Corporate Center, L.P.	S-1/A (333-141700)	4/27/07	10.22	
10.13	Amended and Restated 2007 Director Option Plan and form of option agreement	Form 8-K Current Report	6/18/10	10.2	

10.14	2007 Employee Stock Purchase Plan	S-1/A (333-141700)	5/17/07	10.24
10.15	Amicus Therapeutics, Inc. 2007 Amended and Restated Equity Incentive Plan	Form 8-K Current Report	6/18/10	10.1

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Exhibit No.	Filed Exhibit Description	Incorporated by Reference to SEC Filing			Filed with this Form 10-K
		Form	Date	Exhibit No.	
10.16	Lease Agreement dated as of September 11, 2008 by and between the Registrant and A/G Touchstone, TP, LLC.	Form 8-K Current Report	9/15/08	10.1	
+ 10.17	License and Collaboration Agreement, dated as of November 7, 2007, by and between the Registrant and Shire Pharmaceuticals Ireland, Ltd.	Form 10-K Annual Report	2/08/08	10.20	
10.18	Letter Agreement, dated as of December 30, 2008, by and between the Registrant and David Lockhart, Ph.D.	Form 8-K Current Report	12/31/08	10.4	
10.19	Letter Agreement, dated as of December 30, 2008, by and between the Registrant and Matthew R. Patterson	Form 8-K Current Report	12/31/08	10.3	
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10.21	Letter Agreement, dated as of December 30, 2008, by and between the Registrant and S. Nicole Schaeffer	Form 10-K	2/6/09	10.28	
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10.27	Placement Agency Agreement dated February 25, 2010 between Amicus Therapeutics, Inc. and Leerink Swann LLC	Form 8-K	2/25/10	10.2	
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10.29	Letter Agreement, dated as of March 2, 2009, by and between the Registrant and John M. McAdam	Form 8-K	3/4/10	10.1	
++ 10.30	License and Collaboration Agreement dated as of October 28, 2010 by and between the Registrant and Glaxo Group Limited				X
++ 10.31	Stock Purchase Agreement dated as of October 28, 2010 by and between the Registrant and Glaxo Group Limited				X
10.32	Letter Agreement, dated as of May 10, 2010 by and between the Registrant and Ken Valenzano				X
10.33	Letter Agreement, dated as of January 3, 2011 by and between the Registrant and Kenneth Peist				X
10.34	Letter Agreement, dated as of January 3, 2011 by and between the Registrant and Enrique Dilone				X
23.1	Consent of Independent Registered Public Accounting Firm.				X
31.1	Certification of Principal Executive Officer Pursuant to Rule 13a-14(a) of the Securities Exchange Act of 1934.				X
31.2	Certification of Principal Financial Officer Pursuant to Rule 13a-14(a) of the Securities				X

Exchange Act of 1934.

32.1	Certificate of Principal Executive Officer pursuant to 18 U.S.C. Section 1350 and Section 906 of the Sarbanes-Oxley Act of 2002.	X
32.2	Certificate of Principal Financial Officer pursuant to 18 U.S.C. Section 1350 and Section 906 of the Sarbanes-Oxley Act of 2002.	X

- 
- + Confidential treated has been granted as to certain portions of the document, which portions have been omitted and filed separately with the Securities and Exchange Commission.
  - ++ Confidential treated has been requested as to certain portions of the document, which portions have been omitted and filed separately with the Securities and Exchange Commission.

**SIGNATURES**

Pursuant to the requirements of Section 13 or 15 (d) of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized on March 4, 2011.

AMICUS THERAPEUTICS, INC.  
(Registrant)

By: /s/ John F. Crowley  
John F. Crowley  
*Chief Executive Officer*

Pursuant to the requirements of the Securities Exchange Act of 1934, this Report has been signed below by the following persons on behalf of the Registrant and in the capacities and on the dates indicated.

<u>Signature</u>	<u>Title</u>	<u>Date</u>
<u>/s/ John F. Crowley</u> (John F. Crowley)	Chairman and Chief Executive Officer (Principal Executive Officer)	March 4, 2011
<u>/s/ Daphne Quimi</u> (Daphne Quimi)	Corporate Controller (Principal Financial and Accounting Officer)	March 4, 2011
<u>/s/ Donald J. Hayden</u> (Donald J. Hayden)	Director	March 4, 2011
<u>/s/ Sol J. Barer, Ph.D.</u> (Sol J. Barer, Ph.D.)	Director	March 4, 2011
<u>/s/ Alexander E. Barkas, Ph.D.</u> (Alexander E. Barkas, Ph.D.)	Director	March 4, 2011
<u>/s/ James Barrett, Ph.D.</u> (James Barrett, Ph.D.)	Director	March 4, 2011
<u>/s/ Margaret G. McGlynn, R.Ph.</u> (Margaret G. McGlynn, R.Ph.)	Director	March 4, 2011
<u>/s/ P. Sherrill Neff</u> (P. Sherrill Neff)	Director	March 4, 2011
<u>/s/ Michael G. Raab</u> (Michael G. Raab)	Director	March 4, 2011
<u>/s/ Glenn Sblendorio</u> (Glenn Sblendorio)	Director	March 4, 2011
<u>/s/ James N. Topper, M.D., Ph.D.</u> (James N. Topper, M.D., Ph.D.)	Director	March 4, 2011

**Table of Contents**

Exhibit No.	Filed Exhibit Description	Incorporated by Reference to SEC Filing			Filed with this Form 10-K
		Form	Date	Exhibit No.	
3.1	Restated Certificate of Incorporation of the Registrant.	S-1 (333-141700)	5/17/07	3.2	
3.2	Restated By-laws of the Registrant.	S-1/A (333-141700)	4/27/07	3.4	
4.1	Specimen Stock Certificate evidencing shares of common stock	S-1 (333-141700)	3/30/07	4.1	
4.2	Third Amended and Restated Investor Rights Agreement, dated as of September 13, 2006, as amended	S-1 (333-141700)	3/30/07	4.3	
4.3	Form of Warrant	Form 8-K	2/25/10	4.1	
10.1	2002 Equity Incentive Plan, as amended, and forms of option agreements thereunder	S-1/A (333-141700)	4/27/07	10.1	
+ 10.2	Amended and Restated License Agreement, dated October, 31, 2008, by and between the Registrant and Mount Sinai School of Medicine of New York University	Form 10-K	2/6/09	10.3	
+ 10.3	License Agreement, dated as of June 26, 2003, by and between the Registrant and University of Maryland, Baltimore County, as amended	S-1 (333-141700)	3/30/07	10.4	
+ 10.4	Exclusive License Agreement, dated as of June 8, 2005, by and between the Registrant and Novo Nordisk, A/S	S-1 (333-141700)	3/30/07	10.5	
10.5	Sublease Agreement, dated as of May 12, 2005, by and between the Registrant and Purdue Pharma, L.P.	S-1 (333-141700)	3/30/07	10.6	
10.6	Amended and Restated Employment Agreement, dated as of December 17, 2010, by and between the Registrant and John F. Crowley	Form 8-K Current Report	12/31/10	10.1	
10.7	Letter Agreement, dated as of November 9, 2004, by and between the Registrant and Matthew R. Patterson	S-1 (333-141700)	3/30/07	10.8	
10.8	Letter Agreement, dated as of December 19, 2005, by and between the Registrant and David Lockhart, Ph.D.	S-1 (333-141700)	3/30/07	10.10	
10.9	Form of Director and Officer Indemnification Agreement	S-1 (333-141700)	3/30/07	10.17	
10.10	Restricted Stock Agreement, dated as of March 8, 2007, by and between the Registrant and James E. Dentzer	S-1/A (333-141700)	4/27/07	10.20	
10.11	Restricted Stock Agreement, dated as of March 8, 2007, by and between the Registrant and Glenn P. Sblendorio	S-1/A (333-141700)	4/27/07	10.21	
10.12	Lease Agreement, dated as of July 31, 2006, by and between the Registrant and Cedar Brook II Corporate Center, L.P.	S-1/A (333-141700)	4/27/07	10.22	
10.13	2007 Director Option Plan and form of option agreement	S-1/A (333-141700)	5/17/07	10.23	
10.14	2007 Employee Stock Purchase Plan	S-1/A (333-141700)	5/17/07	10.24	
10.15	Amicus Therapeutics, Inc. 2007 Amended and Restated Equity Incentive Plan	Form 8-K Current Report	6/12/08	10.1	
10.16	Lease Agreement dated as of September 11, 2008 by and between the Registrant and A/G Touchstone, TP, LLC.	Form 8-K Current Report	9/15/08	10.1	
+ 10.17	License and Collaboration Agreement, dated as of November 7, 2007, by and between the Registrant and Shire Pharmaceuticals Ireland, Ltd.	Form 10-K Annual Report	2/08/08	10.20	
10.18	Letter Agreement, dated as of December 30, 2008, by and between the Registrant and David	Form 8-K Current Report	12/31/08	10.4	



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- ++ Confidential treated has been requested as to certain portions of the document, which portions have been omitted and filed separately with the Securities and Exchange Commission.

Portions of this exhibit have been omitted and filed separately with the Secretary of the Securities and Exchange Commission (the "Commission") pursuant to an application for confidential treatment filed with the Commission pursuant to Rule 24b-2 under the Securities Exchange Act of 1934, as amended. Such portions are marked as indicated below.

### LICENSE AND COLLABORATION AGREEMENT

THIS LICENSE AND COLLABORATION AGREEMENT (the "Agreement") is made as of the 28th day of October, 2010 (the "Effective Date") by and between Amicus Therapeutics, Inc., a Delaware corporation having a place of business at 6 Cedar Brook Drive, Cranbury, New Jersey, 08512 ("Amicus") and Glaxo Group Limited, a company organized under the laws of England and Wales with its registered office address at Glaxo Wellcome House, Berkeley Avenue, Greenford, Middlesex, UB6 0NN, England ("GSK"). Amicus and GSK are each referred to herein by name or as a "Party" or, collectively, as the "Parties".

### RECITALS

WHEREAS, Amicus is developing Compound (as defined below), and owns or controls certain regulatory filings and intellectual property related thereto;

WHEREAS, GSK desires to collaborate with Amicus on the Development of Compound and to obtain exclusive rights to Commercialize Products in the Field in the Territory (each as hereinafter defined) as set forth in this Agreement;

WHEREAS, Amicus desires to collaborate with GSK on the Development of the Compound and Products in the Field in the Territory as set forth in this Agreement; and

WHEREAS, Amicus further desires that GSK exclusively Commercialize Compound and Product(s) in the Field in the Territory, as set forth in this Agreement.

WHEREAS, contemporaneously with the execution of this Agreement, the Parties have executed an Equity Agreement under which GSK shall purchase common stock of Amicus, as set forth in such Equity Agreement.

NOW, THEREFORE, in consideration of the mutual agreements contained herein and other good and valuable consideration, the sufficiency of which is hereby acknowledged, the Parties agree as follows:

### I. DEFINITIONS

Unless specifically set forth to the contrary herein, the following terms, whether used in the singular or plural, shall have the respective meanings set forth below:

1.1 "011 Phase III Clinical Study" means the Phase III Clinical Study sponsored by Amicus and identified by the ClinicalTrials.gov Identifier NCT00925301.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

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1.2 “012 Phase III Clinical Study” means the Phase III Clinical Study sponsored by Amicus and identified by the ClinicalTrials.gov Identifier NCT01218659.

1.3 “AAA” has the meaning ascribed to that term in Section 16.2.2.

1.4 “Abandoning Party” has the meaning ascribed to that term in Section 7.4.

1.5 “Acceptance of Filing” has the meaning ascribed to that term in Section 3.3.2.

1.6 “Act” means the United States Food, Drug and Cosmetic Act of 1938, as amended from time to time, and its implementing regulations.

1.7 “Actual Payment Report” has the meaning ascribed to that term in Section 3.8.

1.8 “Affected Area” has the meaning ascribed to that term in Section 14.2.

1.9 “Affiliate” means, with respect to any specified Person, at any time, a Person that, directly or indirectly, through one or more intermediaries, controls, or is controlled by, or is under common control with, such specified Person at such time. For purposes of this definition and Section 1.27, “control,” when used with respect to any specified Person, shall mean (a) the direct or indirect ownership of more than fifty percent (50%) (or such lesser percentage which is the maximum allowed to be owned by a foreign corporation in a particular jurisdiction) of the total voting power of securities or other evidences of ownership interest in such Person or (b) the power to direct or cause the direction of the management and policies of such Person, directly or indirectly, whether through ownership of voting securities, by contract or otherwise; and the terms “controlling” and “controlled” have meanings correlative to the foregoing.

1.10 “Agreement” has the meaning ascribed to that term in the first paragraph of this Agreement.

1.11 “Alliance Manager” has the meaning ascribed to that term in Section 4.3.

1.12 “Amicus” has the meaning ascribed to that term in the first paragraph of this Agreement.

1.13 “Amicus Aggregate Development Cost Cap” means \*\*\*\*\*, which amount is equal to the aggregate of Amicus’ share of the Development Costs specified in the Initial Development Plan for each of the calendar years beginning with calendar year 2011 up to and including calendar year 2015.

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1.14 “ Amicus Annual Cost Cap ” means: (a) for calendar year 2010, \*\*\*\*\* (which amount represents the aggregate of one hundred percent (100%) of Amicus’s Development Costs for calendar year 2010 as set forth in the Initial Development Plan \*\*\*\*\*; (b) for calendar year 2011, \*\*\*\*\* (which amount is equal to fifty percent (50%) of the total Development Costs for calendar year 2011 as set forth in the Initial Development Plan); (c) for calendar years 2012, 2013, 2014 and 2015, respectively, \*\*\*\*\*, \*\*\*\*\*, \*\*\*\*\* and \*\*\*\*\*, respectively (which amount is equal to twenty-five percent (25%) of the total Development Costs for the applicable calendar year, as set forth in the Initial Development Plan); and (d) for calendar year 2016 and each calendar year thereafter, if applicable, \*\*\*\*\* (which amount is equal to twenty-five percent (25%) of the total Development Costs for calendar year 2015 as set forth in the Initial Development Plan); in each case, as adjusted in accordance with Section 5.1.5(d) below.

1.15 “ Amicus Auditor ” has the meaning ascribed to that term in Section 3.9.

1.16 “ Amicus House Marks ” has the meaning ascribed to that term in Section 6.3.

1.17 “ Amicus Indemnitees ” has the meaning ascribed to that term in Section 15.1.

1.18 “ Amicus Intellectual Property ” means Amicus Patents, Amicus Know-How, and any and all copyrights pertaining to the Compound and Product for the Territory that are Controlled by Amicus during the Term.

1.19 “ Amicus Know-How ” means all confidential Know-How which (i) Amicus or its Affiliates Control as of the Effective Date, or (ii) subject to Section 12.3 and Section 14.3.10(b), is Controlled by Amicus or its Affiliates after the Effective Date during the Term of this Agreement and developed or acquired by or on behalf of Amicus or its Affiliates outside the Program and without the use of Program Improvements; in each case, that is reasonably necessary or actually used to Develop, Manufacture or Commercialize Products in the Field for the Territory. Notwithstanding the foregoing, Amicus Know-How shall not include: (a) information which is or becomes part of the public domain through no breach of this Agreement by GSK; (b) information which GSK can demonstrate by its written records was known by GSK or its Affiliates prior to the disclosure thereof by Amicus or its Affiliate; (c) information which is independently developed by GSK or its Affiliates outside of the Program, so long as such development does not result from use of Amicus Know-How, and such independent development can be demonstrated by written records; and (d) information that becomes available to GSK or its Affiliates on a non-confidential basis, whether directly or indirectly, from a Third Party who is not bound by a confidentiality obligation to Amicus or its Affiliates.

1.20 “ Amicus Patents ” means: (i) all Patents Controlled by Amicus or its Affiliates as of the Effective Date which are reasonably necessary, or actually practiced, to Develop, Manufacture or Commercialize the Compound or Product for use as a therapeutic agent, including without limitation the Patents set forth on Schedule 7.2.1 hereto; and (ii) subject to Section 12.3, all Patents Controlled by Amicus or its Affiliates in the Territory during the Term of this Agreement that are reasonably necessary, or actually practiced to Develop, Manufacture or Commercialize the Compound or Product in the Territory or to the extent claiming inventions within the Amicus Know-How Controlled by Amicus or its Affiliates as of the Effective Date.

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1.21 “ Amicus Proprietary Chaperone Technology ” means Amicus’ proprietary technology used in connection with a small molecule drug that selectively binds to the active site of a target enzyme resulting in enzyme stabilization, improved trafficking, less aggregation, and/or increased activity of the enzyme, including all associated Patents and Know-How Controlled by Amicus in the Territory.

1.22 “ Amicus Prosecuted Patents ” has the meaning ascribed to that term in Section 7.2.2.

1.23 “ Amicus Terminated Product Trademark ” has the meaning ascribed to that term in Section 14.2.2.

1.24 “ Amicus Trademark ” has the meaning ascribed to that term in Section 2.4.

1.25 “ Background License Agreements ” means the agreements, letters, and other documents listed in Schedule 1.25.

1.26 “ Calendar Year Net Sales ” means the total Net Sales of all Products sold in the specified country or countries of the Territory in a particular calendar year.

1.27 “ Change of Control ” means either: (a) a sale of all or substantially all of the assets of a Party in one or a series of integrated transactions not in the ordinary course of business to a Third Party; (b) the acquisition of control (as defined in Section 1.9) of a Party by a Third Party by means of any transaction or series of related transactions to which such Party is a party (including, any stock acquisition, merger or consolidation); or (c) the acquisition by a Major Pharmaceutical Company of \*\*\*\*\* percent \*\*\*\*\* or more of the total issued capital stock of a Party and the right to direct or cause the direction of the management and policies of such Party, directly or indirectly, whether through ownership of voting securities, by contract, or otherwise; \*\*\*\*\*. For clarity, a Change of Control would not include any transaction or series of transactions in which the holders of voting securities of a Party outstanding immediately prior to such transaction continue to retain (either by such voting securities remaining outstanding or by such voting securities being converted into voting securities of the surviving entity), as a result of shares in the Party held by such holders prior to such transaction, fifty percent (50%) or more of the total voting power represented by the voting securities of the acquiring entity outstanding immediately after such transaction or series of transactions.

1.28 “ Claim ” means any action, appeal, petition, plea, charge, complaint, suit, demand, litigation, arbitration, mediation, hearing, inquiry, investigation, or similar event, occurrence, or proceeding.

1.29 “ Co-Development Opt-Out ” has the meaning ascribed to that term in Section 13.6.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

1.30 “Co-Development Opt-Out Notice” has the meaning ascribed to that term in Section 13.6.

1.31 “Combination Therapy” means the use of the Compound or Product in combination with one or more other active ingredients. Drug delivery vehicles, adjuvants, and excipients shall not be deemed to be “active ingredients”, and their presence shall not be deemed to create a Combination Therapy. Combination Therapy includes, but is not limited to: (i) adjuvant use of the Compound or Product with an ERT; (ii) co-administration of the Compound or Product with an ERT, regardless of the order or form in which the co-administration is performed; or (iii) formulation of the Compound or Product and an ERT.

1.32 “Commercialize” or “Commercialization” means activities directed to obtaining pricing and reimbursement approvals for, marketing, advertising, promoting, detailing, distributing, importing, or selling a Product in the Field in the Territory, and education, planning, product support and medical efforts related to a Product in the Field in the Territory.

1.33 “Commercially Reasonable Efforts” means that level of efforts and resources required to carry out a particular task or obligation in an active and sustained manner, consistent with the usual practice followed by a Party in the exercise of reasonable business discretion relating to other pharmaceutical products owned by it, or to which it has exclusive rights, which are of similar market potential and at a similar stage in development or product life, taking into account issues of patent coverage, safety and efficacy, scientific and product profile, the regulatory structure involved, and the strategic value and profitability of the product (including, without limitation, pricing and reimbursement status achieved). A Party may not consider payments required to be made hereunder when determining its Commercially Reasonable Efforts with regards to the Product or its obligations under this Agreement.

1.34 “Compound” means migalastat, as described in Schedule 1.34, and includes (i) any compounds with alternative names but with the same chemical structure as Migalastat, and (ii) any metabolites, prodrugs, isomers and enantiomers (excluding the isomer/enantiomer “1-deoxynorjirimycin” or “(2R,3R,4R,5S)-2-(hydroxymethyl)piperidine-3,4,5-triol”), esters, salts, hydrates, solvates, and polymorphs, whether alone or in a mixture.<sup>3</sup>

1.35 “Confidential Information” means in the case of one Party (the “disclosing Party”), that Party’s or its Affiliate’s know-how and financial or other confidential or proprietary information that is Controlled by that Party or its Affiliates and made available (in whatever form and whether prior to, on, or after the Effective Date) to the other Party (the “receiving Party”) in connection with this Agreement or generated pursuant to this Agreement. Notwithstanding the foregoing, Confidential Information shall not include:

(a) information which is or becomes part of the public domain through no breach of this Agreement by the receiving Party or any of its Affiliates;

(b) information which the receiving Party can demonstrate by its written records was known by the receiving Party or any of its Affiliates prior to the disclosure thereof by the disclosing Party;

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

(c) information which is independently developed by the receiving Party or any of its Affiliates, so long as such development does not result from use of Confidential Information of the disclosing Party, and such independent development can be demonstrated by written records of the receiving Party or any of its Affiliates; and

(d) information that becomes available to the receiving Party or its Affiliates on a non-confidential basis, whether directly or indirectly, from a Third Party who is not bound by a duty of confidentiality to the disclosing Party.

1.36 “ Confidentiality Agreement ” means the Confidentiality Agreement between Amicus and GSK dated as of \*\*\*\*\* and amended as of \*\*\*\*\*.

1.37 “ Control ” or “ Controlled ” means, with respect to any compound, material, information, or intellectual property right, that a Party owns or has a license to use, commercialize, manufacture, market, distribute or sell, and has the ability to grant to the other Party a license or a sublicense (as applicable under this Agreement) to such compound, material, information, or intellectual property right as provided for herein without violating (i) the terms of any agreement or other arrangements with any Third Party existing at the time such Party would be first required hereunder to grant the other Party such license or sublicense or (ii) any Law applicable to such license or sublicense.

1.38 “ Cooperating Party ” has the meaning ascribed to that term in Section 11.2.2.

1.39 “ Develop ” or “ Development ” means all activities related to (i) non-clinical and clinical research and drug development (including preclinical testing and clinical trials) related to obtaining, maintaining and/or expanding Marketing Approval (excluding pricing and reimbursement approvals), (ii) Phase IV Clinical Trials and preclinical studies conducted after Marketing Approval (such as carcinogenicity studies, preclinical studies to establish pediatric dosing and similar activities) that are required or requested by a Regulatory Authority to be conducted after Marketing Approval, as a condition of obtaining such Marketing Approval; (iii) manufacturing activities for the purposes of producing clinical supplies (or materials used in preclinical testing or research), as well as test method development and stability testing and process development and validation for a product prior to the first Marketing Approval of such Product (including manufacturing batches for validation and registration purposes), formulation development, delivery system development, quality assurance and quality control development for clinical supplies, and (iv) statistical analysis, regulatory affairs, and activities directed towards obtaining Marketing Approval (excluding regulatory activities directed to obtaining pricing and reimbursement approvals) and clinical study regulatory activities (excluding regulatory activities directed to pricing and reimbursement approvals); in each case, with respect to the Compound and/or Products in the Field for the Territory.

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- 1.40 “ Development Costs ” has the meaning ascribed to that term in Schedule 5.1.5 .
- 1.41 “ Development Plan ” has the meaning ascribed to that term in Section 5.1.1(b) and includes the Initial Development Plan and any amendments thereto in accordance with Section 5.1.1(b).
- 1.42 “ Discriminatory Conduct ” has the meaning ascribed to that term in Section 6.1.1(b).
- 1.43 “ Dispute ” has the meaning ascribed to that term in Section 16.2.1.
- 1.44 “ Effective Date ” has the meaning ascribed to that term in the first paragraph of this Agreement.
- 1.45 “ Election Notice ” has the meaning ascribed to that term in Section 12.2.1.
- 1.46 “ EMA ” means the European Medicines Agency of the European Union or any successor entity thereto having similar responsibilities with respect to pharmaceutical products, such as the Products.
- 1.47 “ Equity Agreement ” means the stock purchase agreement attached hereto as Exhibit A .
- 1.48 “ ERT ” means enzyme replacement therapy.
- 1.49 “ Escalation Notice ” has the meaning ascribed to that term in Section 4.1.5(a).
- 1.50 “ Estimated Payment Report ” has the meaning ascribed to that term in Section 3.8.
- 1.51 “ Excluded Item ” has the meaning ascribed to that term in Section 11.1.2.
- 1.52 “ FDA ” means the United States Food and Drug Administration or any successor entity thereto having similar responsibilities with respect to pharmaceutical products, such as the Products.
- 1.53 “ Field ” means any and all uses or purposes, including, without limitation, the treatment, palliation, and/or prevention and diagnosis of any human or animal disease, disorder or condition, including use of the Product in combination with ERT.
- 1.54 “ First Opt-Out Quarter ” has the meaning ascribed to that term in Section 13.6.
- 1.55 “ Force Majeure Event ” has the meaning ascribed to that term in Section 16.11.
- 1.56 “ FTE ” has the meaning ascribed to that term in Schedule 5.1.5.
- 1.57 “ FTE Costs ” has the meaning ascribed to that term in Schedule 5.1.5.

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1.58 “ FTE Rate ” has the meaning ascribed to that term in Schedule 5.1.5

1.59 “ Generic Equivalent ” means, as to any specific Product at issue which has received Regulatory Approval in the country at issue, a non-innovator product that: (i) has obtained Regulatory Approval by means of an abbreviated NDA filed pursuant to Section 505(j) of the Act which refers to the specific Product at issue as the Reference Listed Drug (as defined in 21 C.F.R. 314.3(b) (as amended)) in the United States, or an application similar to an abbreviated NDA filed pursuant to Section 505(j) of the Act for any jurisdiction outside the United States, in each case, without the requirement of any human clinical efficacy trials; (ii) is bioequivalent to the specific Product; and (iii) is legally marketed in such country by an entity other than GSK, its Affiliates or Sublicensees.

1.60 “ Current Good Manufacturing Practices ” or “ cGMP ” means the standards relating to manufacturing practices for fine chemicals, intermediates, bulk products or finished pharmaceutical products: (i) detailed in the U.S. Current Good Manufacturing Practices, 21 C.F.R. Parts 210 and 211 and The Rules Governing Medicinal Products in the European Community, Volume IV Good Manufacturing Practice for Medicinal Products, as each may be amended from time to time; and/or (ii) outside the United States and European Union, promulgated by any Regulatory Authority having jurisdiction over the manufacture of fine chemicals, intermediates, bulk products or finished pharmaceutical products; and subject to any arrangements, additions or clarifications agreed to from time to time by the Parties in a quality agreement.

1.61 “ GSK ” has the meaning ascribed to that term in the first paragraph of this Agreement.

1.62 “ GSK Auditor ” has the meaning ascribed to that term in Section 3.10.

1.63 “ GSK Background IP ” means all Patents and Know-How which (i) GSK or its Affiliates Controls as of the Effective Date, or (ii) is developed by or on behalf of GSK or its Affiliates or acquired by GSK or its Affiliates, in each case, after the Effective Date outside the Program and without the use of Program Improvements.

1.64 “ GSK House Marks ” has the meaning ascribed to that term in Section 6.3.

1.65 “ GSK Indemnitees ” has the meaning ascribed to that term in Section 15.2.

1.66 “ GSK Supplied Material ” has the meaning ascribed to that term in Section 14.3.6.

1.67 “ GSK Terminated Product Trademark ” has the meaning ascribed to that term in Section 14.3.9(a).

1.68 “ GSK Trademark ” has the meaning ascribed to that term in Section 2.4.

1.69 “ Indemnitee ” has the meaning ascribed to that term in Section 15.3.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

1.70 “ Indemnitor ” has the meaning ascribed to that term in Section 15.3.

1.71 “ Initial Development Plan ” has the meaning ascribed to that term in Section 5.1.1(a).

1.72 “ Initial Press Release ” has the meaning ascribed to that term in Section 11.2.1.

1.73 “ IND ” means any Investigational New Drug Application (including any amendments thereto) filed with the FDA pursuant to 21 C.F.R. §321 before the commencement of clinical trials of a Product, or any comparable filings with any Regulatory Authority in any other jurisdiction.

1.74 “ Joint Program Patent ” has the meaning ascribed to that term in Section 7.3.3.

1.75 “ Joint Steering Committee ” or “ JSC ” has the meaning ascribed to that term in Section 4.1.1.

1.76 “ Know-How ” means any proprietary technology, technical, scientific and medical information, methods of use, processes, techniques, ideas, inventions (excluding any inventions disclosed in any Patent or published Patent application), improvements, modifications, know-how, practices, trade secrets, chemistry, manufacturing and control data, quality control information and procedures, and pharmacological, toxicological and preclinical and clinical test data and results and regulatory information (including all documentation and correspondence submitted or required to be submitted to a Regulatory Authority, or received from a Regulatory Authority, in connection with a Marketing Approval in any country), all of the foregoing pertaining to the Development, Manufacture and/or Commercialization of the Compound and/or Products within the Field for the Territory, but excluding Patents associated with any of the foregoing.

1.77 “ Launch ” means, on a country-by-country and Product-by-Product basis, the date of the first \*\*\*\*\* (or one of its Affiliates or permitted Sublicensees) in such country; provided that the Launch of a Product in a country for a particular indication shall be deemed to occur upon the first commercial sale of a Product with labeling for such indication. Sales of a Product for registration samples, compassionate use sales, named patient use and the like, and inter-company transfers to Affiliates of GSK for resale will not constitute a Launch.

1.78 “ Law ” means all laws, statutes, regulations (including securities laws, regulations or guidances), or governmental, regulatory, or judicial orders or judgments in effect from time to time.

1.79 “ Liabilities ” has the meaning ascribed to that term in Section 15.1.

1.80 “ License ” has the meaning ascribed to that term in Section 2.1.

1.81 “ Licensed Technology ” means all (i) Amicus Intellectual Property, (ii) Program Improvements developed solely or jointly by Amicus or its Affiliates (subject to Section 12.3) during the Term, and (iii) Program Patents in the Territory owned solely or jointly by Amicus or its Affiliates (subject to Section 12.3). For the avoidance of doubt, the “Licensed Technology” shall include Amicus Proprietary Chaperone Technology, but solely to the extent such Amicus Proprietary Chaperone Technology is necessary for the Development, Manufacture or Commercialization of Product for the Territory.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

1.82 “MAA” means (a) a Marketing Authorization Application filed with the EMA, seeking Regulatory Approval of a Product and all variations thereto filed with the EMA; (b) an NDA submitted to the FDA in the United States; or (c) a corresponding application for Regulatory Approval that has been submitted to a Regulatory Authority in any other jurisdiction in the Territory.

1.83 “Major EU Country” means \*\*\*\*\*, \*\*\*\*\*, \*\*\*\*\*, \*\*\*\*\* or \*\*\*\*\*.

1.84 “Major Market” means \*\*\*\*\*, each \*\*\*\*\*and \*\*\*\*\*.

1.85 “Major Pharmaceutical Company” shall mean a company that is engaged in the business of selling pharmaceutical products, whose worldwide revenues from such sales (on a consolidated basis in the last full fiscal year prior to the closing of any Change of Control) was in excess of \*\*\*\*\* or a company engaged in business in the life sciences field that is of an equivalent size. Any Affiliate of such company shall be deemed to be a Major Pharmaceutical Company.

1.86 “Manufacture” or “Manufacturing” means all the activities required for the production and supply of Compound and/or Product, including without limitation, purchasing raw materials, quality control and assurance, filing, finishing, labeling, packaging, qualified person release, holding, shipping and storage and the tests and analyses conducted in connection therewith.

1.87 “Manufacturing Costs” has the meaning ascribed to that term in Schedule 5.1.5.

1.88 “Marketing Approval” means all approvals, licenses, registrations or authorizations of the Regulatory Authority in a country, necessary for the manufacture, use, storage, import, marketing and sale of a Product in such country. For countries where governmental or other similar approval of pricing and/or reimbursement is required for marketing in such country, Marketing Approval shall not be deemed to occur until \*\*\*\*\* is obtained. For clarity, however, it is understood that, as of the Effective Date, Marketing Approval in the United States shall be deemed to occur upon \*\*\*\*\*. In the event that any such \*\*\*\*\* of any governmental agency in the United States is required at the time that the Parties seek Marketing Approval for a Product in the United States, then Marketing Approval in the United States shall not be deemed to occur until \*\*\*\*\*. Notwithstanding the foregoing, Marketing Approval shall be deemed to have occurred for a particular indication for a Product in such jurisdiction upon the Launch of such Product in such jurisdiction with labeling for such indication.

1.89 “Marketing Plan” means the strategic plan for the marketing, promotion and other Commercialization of Product in the Territory, including without limitation the Marketing Strategy, which will include the projected market penetration for each Product in the Major Markets, in reasonable scope and detail, as prepared by GSK in accordance with GSK’s normal and customary format and process for such plans, and as amended from time to time by GSK during the Term.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

1.90 “Marketing Strategy” means the marketing strategy for Product in the Territory determined by GSK and reviewed by the Joint Steering Committee, including product positioning, pricing, reimbursement, education programs, medical affairs, publications, sales messages, marketing, distribution, and Phase IV Clinical Studies, as such strategy may be amended by GSK from time to time during the Term.

1.91 “NDA” means a New Drug Application as defined in Title 21 of the U.S. Code of Federal Regulations, Section 314.50, et seq., which is filed with the FDA in order to gain the FDA’s approval to commercialize a pharmaceutical product in the United States for the indications set forth in the New Drug Application.

1.92 “Negotiation Period” has the meaning ascribed to that term in Section 12.2.2.

1.93 “Net Sales” means the amount of gross sales of all Products sold by GSK, its Affiliates or Sublicensees (each, a “Selling Party”) to Third Parties less the following amounts actually and reasonably incurred, allowed, paid or accrued as reported by the Selling Party in its financial statements prepared in accordance with the International Financial Reporting Standards (“IFRS”), applied on a consistent basis:

(a) quantity, trade and cash discounts actually allowed or given;

(b) discounts, replacements, credits or refunds actually allowed for the return of rejected, outdated, damaged or returned Products;

(c) rebates, chargebacks and price adjustments actually allowed or given;

(d) sales or similar taxes (including duties or other similar governmental charges or assessments) levied, or otherwise imposed on the sale of Products to the customer (including VAT or other governmental charges measured by the billing amount, when included in such billing);

(e) charges for freight, handling, postage, transportation, insurance and other shipping charges; and

(f) a reasonable provision for uncollectible accounts not to exceed \*\*\*\*\* percent \*\*\*\*\* of gross amounts invoiced.

provided, however, that:

(i) sales or transfers of Products between or among GSK, any permitted Sublicensee or any Affiliate of GSK for resale shall be excluded from Net Sales calculations; provided, however, that the subsequent resale to a Third Party shall be included in Net Sales hereunder;

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(ii) If a Product is sold or transferred for consideration other than cash, the Net Sales from such sale or transfer shall be deemed the then fair market value of such Product;

(iii) Products that are transferred or used without charge in connection with any pre-clinical or clinical trials, or for any testing, quality control, evaluation or other Development purposes, or distributed as samples or charitable donations, shall be excluded from Net Sales calculations for all purposes; and

(iv) sales or transfers of Products for registration samples, compassionate use sales, named patient use and the like, shall be excluded from Net Sales calculations for all purposes, unless GSK recognizes revenue with respect to any such sales or transfers in which event such sales or transfers shall be included in Net Sales hereunder; and

(v) for a Combination Therapy, the computation of Net Sales in a country shall be based on \*\*\*\*\* during the applicable Quarter although, if \*\*\*\*\*. For purposes of this Section 1.93(f)(v), \*\*\*\*\*. If the Parties are unable to agree on the allocation of Net Sales with respect to a Combination Therapy as provided in this Section 1.93(f)(v), the matter shall be resolved in accordance with Section 16.2.3 below. For the avoidance of doubt, if a Product is sold in combination with a diagnostic device, the computation of Net Sales for such Product shall be based solely on \*\*\*\*\*.

The Net Sales definition may be amended upon written notice from GSK only to extent required to reflect changes to GSK's accounting rules (e.g. a change from IFRS to UK GAAP) that result from a merger, takeover, or change in applicable law.

1.94 “ Ongoing Trial ” has the meaning ascribed to that term in Section 14.3.2.

1.95 “ Out-of-Pocket Expenses ” has the meaning ascribed to that term in Schedule 5.1.5.

1.96 “ Overage ” has the meaning ascribed to that term in Section 5.1.6.

1.97 “ Party ” or “ Parties ” has the meaning ascribed to that term(s) in the first paragraph of this Agreement.

1.98 “ Patent ” means any and all existing (as of the Effective Date) and future patents and patent applications in any country or jurisdiction, including but not limited to, any provisional applications, non-provisional applications, PCT applications, re-issues, re-examinations, divisionals, continuations, continuations-in-part, registrations, confirmations, validations, re-validations, renewals, and extensions of term thereof (including supplementary protection certificates and pediatric use extensions), including utility, model, and design patents.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

1.99 “Patent Costs” means the reasonable fees and expenses paid to outside legal counsel, and filing, maintenance and other out-of-pocket expenses paid to Third Parties as incurred in connection with the prosecution and maintenance of Patents.

1.100 “Person” means any individual, corporation (including any nonprofit corporation), general or limited partnership, limited liability company, joint venture, estate, trust, association, organization, labor union, or other entity.

1.101 “Pharmacological Chaperone” means a small molecule drug that selectively binds to the active site of a target enzyme resulting in enzyme stabilization, improved trafficking, less aggregation, and/or increased activity of the enzyme.

1.102 “Phase II Clinical Studies” means early controlled human clinical studies conducted to obtain some preliminary data on the appropriate dose range and effectiveness of a drug in a disease or condition under study, as more fully defined in 21 C.F.R. §312.21(b) or its successor regulation, or the equivalent in any country other than the United States.

1.103 “Phase III Clinical Studies” means expanded and controlled human clinical studies involving administration of a drug to sufficient numbers of human patients with the goal of establishing that a drug is safe and efficacious for its intended use, and to be considered as a pivotal study for submission of an NDA, as more fully defined in 21 C.F.R. §312.21(c) or its successor regulation, including any such clinical study in any country other than the United States.

1.104 “Phase IV Clinical Studies” means human clinical studies, including marketing studies, epidemiological studies, modeling and pharmaco-economic studies, investigator sponsored clinical trials and post-marketing surveillance studies, in each case for a Product conducted after receipt of Marketing Approval for such Product in the country in which such trial is being conducted and that are required or requested by a Regulatory Authority to be conducted after Marketing Approval, as a condition of or in connection with obtaining and maintaining such Marketing Approval.

1.105 “Product” means, subject to Section 14.3.10(a), any pharmaceutical preparation that incorporates Compound, whether or not as the sole active ingredient, including any formulation thereof, such as intravenous, transdermal, oral, or other dosage form.

1.106 “Product Acquisition Agreement” has the meaning ascribed to that term in Section 12.2.2.

1.107 “Product Liability Claim” has the meaning ascribed to that term in Section 15.4.1.

1.108 “Program” means all activities directed to the Development, Manufacture and/or Commercialization of Compound or Products for the Territory performed by or on behalf of Amicus (or its Affiliates) and/or GSK (or its Affiliates or Sublicensees) under this Agreement; provided, however, it is understood that all activities related to the Development of Compound conducted either: (a) by Amicus prior to the Effective Date; or (b), by Amicus with respect to a Terminated Product(s) in the Affected Area after termination of this Agreement in such country(ies) or with respect to such Product(s) (but not in its entirety) by either GSK pursuant to Section 13.3 or by Amicus pursuant to Section 13.2, will be deemed to have been conducted outside of the Program.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

1.109 “Program Improvements” means any and all confidential Know-How, and other information that is developed by or on behalf of GSK (or its Affiliates or Sublicensees) or Amicus (or its Affiliates, subject to Section 12.3) or jointly by or on behalf of GSK and Amicus or any of their respective Affiliates (subject to Section 12.3), in the performance of the Program, including inventions, Know-How, and all other intellectual property relating to any of the foregoing; provided, however, that Program Improvements will not include Amicus Intellectual Property; and provided further that, Program Improvements shall not include: (i) information which is or becomes part of the public domain through no breach of this Agreement by GSK or Amicus or their respective Affiliates; (ii) information which GSK can demonstrate by its written records was known by GSK or its Affiliates prior to the Effective Date excluding any information received by GSK under the terms of the Confidentiality Agreement; and (iii) information which is independently developed by GSK or Amicus or their respective Affiliates outside of the Program, and such independent development can be demonstrated by written records. Any Program Improvement that is developed solely by GSK or its Affiliate under this Agreement that was not enabled by the use of any Amicus Intellectual Property or developed in the performance of the Development Plan during the period in which Amicus and GSK are sharing Development Costs pursuant to Section 5.1.5 shall be referred to herein as a “GSK-Only Program Improvement.”

1.110 “Program Patent” means a Patent or Patent application disclosing and claiming a Program Improvement.

1.111 “Protective Action” has the meaning ascribed to that term in Section 8.2.

1.112 “Quarter” means a calendar quarter consisting of any of the three-month periods ending on March 31, June 30, September 30 and December 31 in any particular year.

1.113 “Regulatory Approval” means: (a) in the United States, written notice of Marketing Approval by the FDA based on approval of an NDA, or sNDA, as applicable, and (b) in any other country in the Territory, written notice of required Marketing Approval \*\*\*\*\*, such acceptance not to be unreasonably withheld) by the Regulatory Authority having jurisdiction in such country; provided that with respect to countries in the European Union, written notice of a centralized Marketing Approval from the European Medicines Agency shall constitute written notice with respect to each and every such country.

1.114 “Regulatory Authority” means the agency, if any, of the national government of any country with which a pharmaceutical or biological therapeutic product must be registered or by which a pharmaceutical or biological therapeutic product must be approved prior to its manufacture, use, or sale in such country, provided that with respect to countries in the European Union, the European Medicines Agency shall constitute such an agency with respect to each and every such country in addition to any agency of a national government of such country.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

1.115 “ Regulatory Exclusivity ” means any exclusive marketing rights or data exclusivity rights conferred by any applicable Regulatory Authority, other than a Valid Claim, including any regulatory data protection exclusivity, including, where applicable, pediatric exclusivity and/or orphan drug exclusivity and/or any exclusivity afforded by restrictions on the granting by a Regulatory Authority of regulatory approval to market a Generic Equivalent.

1.116 “ Re-Offer Notice ” has the meaning ascribed to that term in Section 12.2.3.

1.117 “ Requesting Party ” has the meaning ascribed to that term in Section 11.2.2.

1.118 “ Revised Terms ” has the meaning ascribed to that term in Section 12.2.3.

1.119 “ Royalty Term ” has the meaning ascribed to that term in Section 3.4.2.

1.120 “ Rules ” has the meaning ascribed to that term in Section 16.2.2.

1.121 “ Safety Issue ” means any unexpected or untoward adverse event related to a Product that is reported to a Party by a patient or physician, or about which a Party becomes aware, which event raises a question about patient safety or the efficacy of such Product and which event a Party considers to be serious enough to contemplate taking a prompt affirmative action with respect to such Product.

1.122 “ Senior Executives ” has the meaning ascribed to that term in Section 4.1.5(a).

1.123 “ Subcommittee ” has the meaning ascribed to that term in Section 4.2.

1.124 “ Sublicensee ” shall mean a Third Party to whom GSK has granted a right to make, have made, sell, market, distribute and/or promote a Product in the Territory pursuant to Section 2.2; and “ Sublicense ” shall mean an agreement or arrangement between GSK and a Sublicensee granting such rights. As used in this Agreement, “Sublicensee” shall not include a wholesaler or reseller of Product who does not market such Product.

1.125 “ Supply Transition Date ” has the meaning ascribed to that term in Section 6.5.1.

1.126 “ Supply Transition Plan ” has the meaning ascribed to that term in Section 6.5.1.

1.127 “ Term ” has the meaning ascribed to that term in Section 13.1.

1.128 “ Terminated Product(s) ” has the meaning ascribed to that term in Section 14.2.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

1.129 “ Terminated Product Trademark ” has the meaning ascribed to that term in Section 14.3.9.

1.130 “ Territory ” means, subject to Section 14.3.10(a), all countries and territories in the world.

1.131 “ Third Party ” means any Person other than Amicus or GSK or an Affiliate of Amicus or GSK.

1.132 “ Third Party Claim ” has the meaning ascribed to that term in Section 15.1.

1.133 “ Total Amicus Development Cost Cap ” has the meaning ascribed to that term in Schedule 5.1.5.

1.134 “ Trademarks ” means (a) trademarks, service marks, logos, trade dress and trade names, and domain names indicating the source of goods or services, and other indicia of commercial source or origin (whether registered, common law, statutory or otherwise), (b) all registrations and applications to register the foregoing anywhere in the world, (c) all goodwill associated therewith, and (e) all rights in and to any of the foregoing.

1.135 “ Trademark License Agreement ” means an agreement in the form attached hereto as Exhibit C or Exhibit D, as applicable.

1.136 “ Treaty ” has the meaning ascribed to that term in Section 3.11.

1.137 “ Total Program Development Costs in the Initial Development Plan ” means the aggregate Development Costs for the Development of the Compound and Products specified in the Initial Development Plan for each of the calendar years beginning with calendar year 2011 up to and including calendar year 2015.

1.138 “ United States ” or “ U.S. ” means the fifty (50) states of the United States of America, the District of Columbia and Puerto Rico.

1.139 “ Valid Claim ” means a claim of an issued, unexpired Amicus Patent or a Program Patent (other than a Program Patent claiming a GSK-Only Program Improvement, a Formulation Patent or a Method of Manufacture Patent) covering i) Compound; or ii) method of use of the Compound or a Product (\*\*\*\*\*) which: (a) has not been revoked or held unenforceable or invalid by a decision of a court or other governmental agency of competent jurisdiction, which decision is not appealable or has not been appealed within the time allowed for appeal; (b) has not been disclaimed, denied or admitted to be invalid or unenforceable through reissue, re-examination or disclaimer or otherwise; or (c) has not lapsed, been cancelled or abandoned, or been dedicated to the public. For purposes of this Section 1.138, a “ Formulation Patent ” means a Patent primarily directed to an invention which is a formulation of Compound and one (1) or more excipients, and a “ Method of Manufacture Patent ” means a Patent primarily directed to an invention which is a method of manufacture of Compound or Product.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

1.140 “Wind-Down Period” has the meaning ascribed to that term in Section 14.3.3.

1.141 Construction. For purposes of this Agreement: (a) words in the singular shall be held to include the plural and vice versa as the context requires; (b) the word “including” and “include” shall be deemed to be followed by the phrase “without limitation” or like expression unless otherwise specified; (c) the terms “hereof,” “herein,” “herewith,” and “hereunder,” and words of similar import shall, unless otherwise stated, be construed to refer to this Agreement as a whole and not to any particular provision of this Agreement; (d) all references to a “business day” or “business days” in this Agreement means any day other than a day which is a Saturday, a Sunday, any day banks are authorized or required to be closed in the United States or any other day on which GSK’s corporate headquarters in the United States are closed; and (e) all references to “Section,” “Article,” “Schedule” and “Exhibit,” unless otherwise specified, are intended to refer to a Section, Article, Schedule or Exhibit of or to this Agreement.

## II. LICENSE

2.1 License Grant from Amicus. Subject to the terms and conditions of this Agreement, Amicus hereby grants to GSK an exclusive license, with the right to grant sublicenses in accordance with Section 2.2, under all Licensed Technology, to Develop, make, use, sell, offer for sale and import Compound and Products, in each case, solely in the Field and in the Territory (the “License”). The License set forth in this Section 2.1 shall be exclusive even as to Amicus, except with respect to Amicus’s right to: (i) co-Develop Compound and Products in the Field and in the Territory in accordance with Article V; (ii) Manufacture Compound and Products in accordance with Section 6.5; and (iii) Commercialize Compound and Products in the Field and in the Territory in accordance with Article VI. For the avoidance of doubt, the Licensed Technology licensed exclusively to GSK in this Section 2.1 shall include any and all data resulting from any clinical trials performed by Amicus, its Affiliates, and its licensees in the Territory with respect to the Compound and Products, in each case subject to Section 14.3.10(b) and to the extent that Amicus has the right to grant to GSK access to such data from licensees. Subject to Section 14.3.10(b), to the extent any clinical data with respect to the Compound and Product is owned or controlled by a licensee of Amicus and is not included in the Amicus Know-How, upon GSK’s written request, Amicus shall use all reasonable efforts to obtain the right, at no cost to GSK, to sublicense to GSK, or otherwise obtain the right for GSK, to access and make any other use of any such clinical trial data within the scope of the License and otherwise in accordance with the terms and conditions of this Agreement; provided that in no event shall Amicus be obligated to undertake additional payment obligations to such licensees in order to obtain such rights for GSK.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

2.2 Sublicensees . GSK shall have the right to grant sublicenses under the rights granted to GSK in Section 2.1 without the prior written consent of Amicus: (a) \*\*\*\*\*; and (b) \*\*\*\*\*. In addition to the sublicense rights provided above in clauses (a) and (b) of this Section 2.2 (a) and (b), GSK may engage Sublicensees and grant Sublicenses in any country of the Territory, provided , however, that GSK may not grant a sublicense to any Third Party listed on the attached Schedule 2.2 without the prior written consent of Amicus, such consent not to be unreasonably withheld. In any event, GSK shall ensure that each of its Sublicensees is bound by a written agreement containing provisions at least as protective of the Compound, the Products and Amicus as this Agreement; and GSK shall remain responsible to Amicus for all activities of its Affiliates and Sublicensees to the same extent as if such activities had been undertaken by GSK itself. Promptly following the execution of each Sublicense, GSK shall provide Amicus with a redacted copy of such Sublicense (redacted solely to the extent necessary to prevent the disclosure of Third Party confidential information and not redacting any terms or information that are necessary for Amicus to determine GSK's compliance with the provisions of this Agreement with respect to the grant of such Sublicense).

2.3 License Grant from GSK . Subject to the terms and conditions of this Agreement:

a) GSK hereby grants to Amicus a worldwide, non-exclusive, fully paid-up, royalty-free right and license, with the right to grant sublicenses only upon the prior written consent of GSK (which approval shall not be unreasonably withheld), under all Program Improvements Controlled by GSK or its Affiliates and Program Patents Controlled by GSK or its Affiliates to: (i) Develop Compound and Product in the Field and in the Territory in accordance with Article V; (ii) Manufacture Compound and Product as provided in Section 6.5; and (iii) engage in Commercialization activities in accordance with the then-current Marketing Plan with GSK in the Field and in the Territory solely in accordance with Article VI.

b) GSK hereby grants to Amicus a worldwide, non-exclusive, fully paid-up, royalty-free, irrevocable right and license, with the right to sublicense, under any Program Patents Controlled by GSK or its Affiliates to make, have made, use, sell, offer for sale, import, practice and otherwise exploit the Program Improvements claimed in such Program Patents, subject to the exclusive rights granted to GSK under this Agreement with respect to Compound and Products in the Field in the Territory.

2.4 Trademarks . For all Trademarks Controlled by Amicus or any of its Affiliates that the Joint Steering Committee determines should be used on a Product in a country(ies) in the Territory (each, an “ Amicus Trademark ”), Amicus shall grant to GSK a license, with the right to sublicense on the same terms as those set forth in Section 2.2 above, in accordance with the terms of the Trademark License Agreement (a form of which is attached hereto as Exhibit C ), to use the Amicus Trademark(s) in such country(ies) in the Territory in connection with the making, having made, use, sale, offering for sale, importation, packaging, distributing and promoting of Product in the Field and in such country(ies) in the Territory. Such license under the Amicus Trademarks will include a right to use the Amicus Trademark(s), other than Amicus House Marks, as part of a domain name. For all Trademarks Controlled by GSK or any of its Affiliates that the Joint Steering Committee determines should be used on a Product in a country(ies) in the Territory (each, a “ GSK Trademark ”), GSK shall grant to Amicus a license in accordance with the terms of the Trademark License Agreement, a form of which is attached hereto as Exhibit D , to use the GSK Trademark(s) solely in connection with Amicus's right to (i) Develop Compound and Product in the Field in the Territory as provided in Article V, (ii) Manufacture Compound or Product in the Field and in the Territory in accordance with Section 6.5, and (iii) engage in Commercialization activities in accordance with the then-current Marketing Plan with GSK in the Field and in the Territory solely in accordance with Article VI. Such license under the GSK Trademarks will include also a right to use the GSK Trademark(s), other than GSK House Marks, as part of a domain name.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

2.5 No Implied Licenses . Except as expressly set forth in this Agreement or in a Trademark License Agreement, neither Party shall acquire any licenses or other intellectual property right or interest, by implication or otherwise, in any Know-How disclosed to it under this Agreement or under any Patents Controlled by the other Party or its Affiliates. Without limiting the foregoing, nothing herein shall be deemed to grant to GSK a right or license to any active pharmaceutical ingredient other than the Compound.

### **III. CONSIDERATION**

As partial consideration for the License granted to GSK in this Agreement, GSK shall pay to Amicus in accordance with the payment provisions in Section 3.6 the following amounts:

3.1 License Fee . Subject to Section 3.11 with respect to payment of taxes, in addition to (and not in lieu of) royalty and milestone payments due under this Agreement, GSK will make a one-time payment in an aggregate amount of thirty million United States dollars (US \$30,000,000) to Amicus representing a license fee within ten (10) business days after receipt of an invoice therefor from Amicus as provided in Section 3.6, which invoice shall not be sent by Amicus to GSK prior to the Effective Date.

3.2 Equity Investment . In addition, GSK shall purchase from Amicus, a number of shares of common stock of Amicus equal to nineteen and nine tenths percent (19.9%) of the number of shares of common stock of Amicus issued and outstanding immediately following the closing of the Equity Agreement, for an aggregate consideration equal to the product of the number of such shares of common stock of Amicus multiplied by the Per Share Price. For the purposes of this Section 3.2, the "Per Share Price" shall be equal to \*\*\*\*\*. The Equity Agreement shall be executed by the Parties on even date herewith, with GSK's payment to Amicus for such securities payable within ten (10) business days after the Equity Agreement effective date.

3.3 Milestone Payments . Subject to Section 3.11 with respect to payment of taxes, in addition to (and not in lieu of) the license fee set forth in Section 3.1 and the royalty payments set forth in Section 3.4, GSK will pay to Amicus the milestone payments set out below following the first achievement of each of the corresponding milestone events no later than sixty (60) days following the receipt of an invoice therefor from Amicus as provided in Section 3.6. GSK shall notify Amicus in writing promptly, but in no event later than ten (10) days, after the achievement of any of the following milestone events, and no invoice for payment of a milestone shall be sent by Amicus to GSK as provided herein prior to Amicus's reasonable determination that the corresponding milestone event has been achieved. Each of the following milestone payments shall be payable only once, regardless of how many times the Product achieves the milestone event and no milestones shall be paid by GSK for milestone events that are not achieved.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

3.3.1 Filing and Approval Milestones. GSK shall pay to Amicus the amount set forth below for the achievement of the corresponding filing and approval milestones by GSK, its Affiliate or Sublicensee (or in the case of the Milestone 1 in the table below, by Amicus or its Affiliate):

<u>Filing and Approval Milestone Event</u>	<u>Milestone Payment</u>
1. *****	\$ *****
2. *****	\$ *****
3. *****	\$ *****
4. *****	\$ *****
5. *****	\$ *****
6. *****	\$ *****
7. *****	\$ *****

3.3.2 Certain Terms Pertaining to Filing and Approval Milestones .

(a) Reduction to Certain Milestone Payments. With respect to Milestone \*\*\*\*\* in the table in Section 3.3.1 above, if \*\*\*\*\* , then the corresponding milestone payment due upon the achievement of Milestone \*\*\*\*\* shall be reduced to \*\*\*\*\* . With respect to Milestone \*\*\*\*\* in the table in Section 3.3.1 above, \*\*\*\*\* , then the corresponding milestone payment due upon the achievement of Milestone \*\*\*\*\* shall be reduced to \*\*\*\*\* .

(b) Certain Definitions. For the purposes of the milestone payments due under Section 3.3.1:

(i) \*\*\*\*\*.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

3.3.3 Sales Performance Milestones. GSK shall pay to Amicus the amount set forth below following the first achievement of the corresponding Sales Performance milestones set out below:

<b>Sales Performance Milestones:</b>	<b>Milestone Payment</b>
*****	\$ *****
*****	\$ *****
*****	\$ *****
*****	\$ *****

3.4 Royalties.

3.4.1 Royalties on Products in the Territory. In addition to (and not in lieu of) the license fee set forth in Section 3.1 and the milestone payments set forth in Section 3.3, and subject to Sections 3.4.3-3.4.8, commencing on the date of Launch of a Product in a country and until the expiration of the Royalty Term in such country, GSK shall pay to Amicus royalties at the rate set forth below on Net Sales of Product on a Product-by-Product and country-by-country basis where such Product is covered by (i) a Valid Claim, or (ii) Regulatory Exclusivity:

<b>Total Annual Net Sales</b>	<b>Royalty (based on Net Sales)</b>
*****	*****%
*****	*****%
*****	*****%
*****	*****%

3.4.2 Royalty Term. Subject to Section 3.11 and Section 3.5.2(b), GSK shall pay to Amicus royalties as set forth in Section 3.4.1 based on the total Net Sales of Products during a calendar year in the Territory on a Product-by-Product and country-by-country basis, for the longer of (i) the last to expire Valid Claim covering such Product in such country, (ii) the date upon which any remaining Regulatory Exclusivity with respect to such Product in such country expires, or (iii) ten (10) years from the date of the first Launch of such Product in such country (the “Royalty Term”).

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

3.4.3 If, during the Royalty Term for a particular Product in a Major Market country, a Product in such Major Market country is not covered by a Valid Claim or Regulatory Exclusivity at the time of the first Launch of such Product in such country but is or becomes covered by a claim of a pending Patent application filed during the applicable Royalty Term that, if such claim issued would be a Valid Claim covering the Product, then the royalty rates for such Product in such Major Market country shall be reduced by \*\*\*\*\*percent (\*\*\*\*\*) of the royalty rates set forth in Section 3.4.1 otherwise due for the applicable Royalty Term. The payments representing the remaining \*\*\*\*\* of the royalties that would otherwise have been payable to Amicus if the pending Patent application had issued and had included a Valid Claim covering the Product in such Major Market country shall be deposited by GSK into a mutually agreed Third Party escrow account to be maintained by GSK. Upon the issuance of a Patent in such Major Market country based upon any such pending Patent application described above with a Valid Claim covering such Product in such Major Market country, the remaining \*\*\*\*\* of the royalties, plus interest accrued on such amount, shall be promptly paid to Amicus and thereafter, GSK shall pay royalties to Amicus on sales of such Product in such Major Market country at the full rates set forth in Section 3.4.1 for the applicable Royalty Term; provided, however, that if a Patent does not issue in such Major Market country from such pending Patent application within \*\*\*\*\* from the earliest priority filing date of such Patent application in such Major Market country, then GSK shall retain all such amounts paid into escrow, plus interest accrued to such escrow account; \*\*\*\*\*.

3.4.4 Subject to Section 3.5 below, during the applicable Royalty Term, GSK shall pay royalties on a Product based upon the royalty rates as set forth in Section 3.4.1 above for sales of such Product in all non-Major Market countries during the applicable Royalty Term, even if such Product is not covered by a Valid Claim or Regulatory Exclusivity in such non-Major Market country.

3.4.5 If, at any time during the Royalty Term, the only Valid Claim covering a particular Product is a Valid Claim of a Joint Program Patent, then the royalties rates for such Product during the applicable period shall be reduced by \*\*\*\*\* of the royalty rates set forth in Section 3.4.1; it being understood that if during the Royalty Term in such country, such Product becomes covered by any other Valid Claim, the applicable royalty rates shall be the full rates set forth in Section 3.4.1.

3.4.6 Following the expiration of GSK's obligation to pay royalties on a Product as provided in Section 3.4.2 in a country, GSK shall have a perpetual (subject to Section 14.3), exclusive, fully paid-up (subject to Section 3.5.2(b)) right and license under the Licensed Technology in such country to make, use, sell, offer for sale and import such Product in such country of the Territory.

3.4.7 If, the royalties due on Net Sales of Product in a particular Quarter in any country of the Territory could be owed by GSK to Amicus pursuant to Section 3.4.1, Section 3.4.3, Section 3.4.4 and/or Section 3.4.5, then the actual royalties to be paid by GSK to Amicus, subject to Section 3.5, shall be calculated in accordance with Section 3.4.1, Section 3.4.3, Section 3.4.4 or Section 3.4.5 (but not more than one of the foregoing Sections) such that Amicus shall be entitled to receive, and GSK shall pay to Amicus \*\*\*\*\* in such country of the Territory under any one (1) of the foregoing Sections; provided that in no event shall GSK be obligated to pay to Amicus royalties on Net Sales of Product in a particular Quarter in any country of the Territory pursuant to more than one of the foregoing Sections.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

3.4.8 For purposes of this Section 3.4, a Valid Claim covers a Product if such Valid Claim would be infringed, but for a license, by the Commercialization of such Product in such country.

### 3.5 Certain Reductions to Royalties .

3.5.1 Generic Equivalent . During the Royalty Term, on a country-by-country and Product-by-Product basis, if the cumulative unit volume of such Generic Equivalent(s) sold by Third Parties in such country are equal to or greater than \*\*\*\*\* of the combined unit volume of such Product and such Generic Equivalent(s) for all indications in the aggregate in such country in any calendar quarter determined by the number of prescriptions given for the Product and such Generic Equivalent(s), in the aggregate during such calendar quarter (as measured by a Scott Levin Associates audit or other mechanism mutually agreed by the Parties), then the royalty rates applicable to Net Sales of such Product by GSK, its Affiliate or Sublicensee in such country shall be \*\*\*\*\* of the royalty rates specified above in Section 3.4.1 with respect to Net Sales of such Product in such country for so long as such competition exists, and such reduced royalty shall be paid by GSK for the shorter of \*\*\*\*\* from the date upon which GSK's royalty obligations were reduced pursuant to this Section 3.5.1 as a result of the sales of such Generic Equivalent(s), or \*\*\*\*\* from the date of the first Launch of the Product in such county, after which time GSK's license with respect to such Product would be converted into a perpetual, exclusive, fully-paid, royalty-free (subject to Section 3.5.2(b)) license under the Licensed Technology to make, have made, use, sale, offer for sale and import such Product in such country in the Territory; provided, however, that GSK shall no longer be entitled to reduce the royalty rates nor the period of GSK's royalty obligations as set forth above in this Section 3.5.1 if at any time following a reduction in royalty rate pursuant to this Section 3.5.1 and prior to the expiration of the Royalty Term set forth in this Section 3.5.1, such Generic Equivalent(s) cease to equal \*\*\*\*\* or more of the combined unit volume of such Product and such Generic Equivalent(s) for all indications in the aggregate in such country in any calendar quarter determined by the number of prescriptions given for the Product and such Generic Equivalent(s), in the aggregate during such calendar quarter (as measured by a Scott Levin Associates audit or other mechanism mutually agreed by the Parties).

### 3.5.2 Third Party Obligations .

(a) During the Royalty Term, on a country-by-country basis, any milestones, royalties and/or other license payments actually paid to a Third Party under a written license agreement covering intellectual property which, following a reasonable evaluation in accordance with normal business practice, GSK determines is necessary to enable GSK to Develop, Manufacture, use, import or sell Product in accordance with the Agreement such that, absent such Third Party license the Development, Manufacture, or Commercialization of Product would infringe such Third Party intellectual property, then such payments shall be creditable by GSK against royalties payable to Amicus by GSK under the Agreement; provided that the royalties due by GSK to Amicus in any Quarter shall not be so reduced by more than \*\*\*\*\* of the royalties that would otherwise be payable by GSK to Amicus for such calendar quarter; provided further that GSK can credit the remainder of such amounts paid to such Third Party against future royalties payable to Amicus by GSK. If Amicus disputes the need of GSK to obtain a Third Party license for the Product, then Amicus may provide written notice of such dispute to the Joint Patent Subcommittee, and such dispute shall be resolved in accordance with 4.2.1.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

(b) Amicus shall be solely responsible for payment of any and all royalties owed by Amicus to a Third Party pursuant to any Background License Agreements that are in effect as of the Effective Date of the Agreement, and a complete description of the royalties payable under such Background License Agreements is set forth on the attached Schedule 3.5.2 ; provided, however , that in no event shall the reduction in the royalties to be paid by GSK to Amicus pursuant to Section 3.5.1 or this Section 3.5.2 result in a payment of royalties by GSK to Amicus that is less than the royalty amount(s) due by Amicus to such Third Parties under such Background License Agreements; provided, further , that if upon expiration of the Royalty Term for a particular Product in a particular country, Amicus continues to owe to a Third Party royalties pursuant to a Background License Agreement based upon sales of such Product by GSK, its Affiliates or Sublicensees in such country, GSK shall continue to pay such Third Party royalties to Amicus on sales of Products in such country at the same rate as as Amicus pays to the Third Party as set forth on Schedule 3.5.2 for so long as such royalties are payable to such Third Party under the applicable Background License Agreement.

(c) In the event that Amicus intends to modify any of the terms of a Background License Agreement pertaining to (x) the amount of royalties payable under such Background License Agreement with respect to sales of Products in the Territory, (y) the term for which such royalties are payable, or (z) the scope of any rights or obligations granted to GSK under this Agreement, Amicus shall provide notice of such intent to GSK within a reasonable period of time (but in no event longer than five (5) business days) prior to making any such modifications. If such modifications would increase the amount of any royalties payable with respect to sales of Products in the Territory or the term for which such royalties are payable or otherwise materially and adversely modify the scope of any rights or obligations granted to GSK under this Agreement, Amicus shall not proceed to so modify any such Background License Agreement without the prior consent and approval of GSK (such approval not to be unreasonably withheld or delayed).

3.5.3 Royalty Floor . Notwithstanding any other provision in this Agreement (including any provisions for deductions or offsets from or against payments due to Amicus), in no event shall the royalties payable by GSK with respect to Net Sales of Products in any Quarter be reduced to less than \*\*\*\*\* of the royalties that would otherwise be payable by GSK to Amicus for such Quarter at the royalty rates specified in Section 3.4.1 if none of the reductions, deductions and offsets specified in Section 3.4 and this Section 3.5 were available.

3.6 Method of Payment . All payments made by a Party to another Party under this Agreement shall be made by bank wire transfer in immediately available funds to an account designated in an invoice from the Party to which such payments are due, which invoice should include bank details, the contact name for any issue resolution and be marked for the attention of the Alliance Manager of the Party to whom such payment is due. All amounts owed by GSK to Amicus hereunder shall be paid by an entity resident in the United Kingdom from a bank account located in the United Kingdom. Unless otherwise expressly stated herein, all payments made by GSK to Amicus pursuant to this Agreement shall be made within sixty (60) days following receipt by GSK of an invoice from Amicus for such amounts.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

3.7 Foreign Exchange. With respect to sales of the Product invoiced in United States dollars, the Net Sales and the amounts due hereunder will be expressed in United States dollars. With respect to sales of the Product invoiced in a currency other than United States dollars, the Net Sales and amounts due hereunder will be reported in United States dollars, calculated using the average exchange rates as calculated and utilized by GSK's group reporting system and published accounts. As of the Effective Date, the method utilized by GSK's group reporting system and published accounts uses spot exchange rates sourced from Reuters/Bloomberg and, if the method used by GSK's group reporting system and published accounts is changed during the Term, GSK will notify Amicus in writing of the revised method prior to GSK applying such method to exchange rate calculations to be made with respect to Net Sales and amounts due under this Agreement.

3.8 Reports and Royalty Payment. \*\*\*\*\*. Thereafter GSK shall, within \*\*\*\*\* after the end of each Quarter, submit to Amicus, together with GSK's payment for the royalties due for each Quarter, on a Product-by-Product and country-by-country basis, a written report (an "Actual Payment Report") showing the actual Net Sales and the royalties payable in accordance with Section 3.4 in each case in U.S. dollars. In each country where Net Sales have occurred in a currency other than United States dollars, such Net Sales will be converted to United States Dollars in accordance with Section 3.7 above.

3.9 GSK Records. GSK will keep, and will require any Affiliates and Sublicensees to keep, for three (3) years from the end of the Quarter to which they pertain, or such longer period as may be required by applicable Law, complete and accurate books of account and records for the purpose of determining the amounts payable to or by Amicus pursuant to this Agreement, including Net Sales of Product in the Territory in sufficient detail to allow the royalties to be determined accurately. Amicus will have the right during such three (3) year period to appoint an independent certified public accountant reasonably acceptable to GSK (the "Amicus Auditor") to inspect those books or records of GSK for the purpose of determining the applicable amounts payable to or by Amicus pursuant to this Agreement. Upon not less than sixty (60) days' prior written notice from Amicus, GSK will make such books and records and the books and records of its Affiliates available (including any Net Sales reports received from its Sublicensees selling Products) for inspection by such Amicus Auditor during regular business hours at such place or places where such records are customarily kept, to verify the accuracy of the reports and payments. The Amicus Auditor will disclose to Amicus only the amount and accuracy of payments reported and actually paid or otherwise payable under this Agreement. The Amicus Auditor will send a copy of the report to GSK at the same time it is sent to Amicus. \*\*\*\*\* Notwithstanding the foregoing, in the event that Amicus demonstrates sufficient cause, giving due consideration to each of the Parties' resources, to support the conduct of an additional inspection pursuant to this Section 3.9 within the same calendar year, the JSC shall discuss in good faith whether to require such additional inspection to take place; provided that the JSC may not unreasonably withhold its consent to such an inspection. The Amicus Auditor shall be obligated to execute a reasonable confidentiality agreement prior to commencing any such inspection. Amicus will bear all costs and expenses associated with an audit conducted pursuant to this Section 3.9, provided, however, that if the designated auditor discovers an underpayment of \*\*\*\*\* or more for any period covered by the inspection between the payments GSK has made under this Agreement and the payments actually owed to Amicus under this Agreement, then GSK will bear all costs and expenses associated with such audit and, for the avoidance of doubt, such underpayment shall be considered a late payment subject to interest pursuant to the terms of Section 16.12.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

3.10 Amicus Records . Amicus will keep, and will require any Affiliates to keep, for three (3) years from the end of the Quarter to which they pertain, or such longer period as may be required by applicable Law, complete and accurate books of account and records of Development Costs and amounts spent on research and Development undertaken in accordance with this Agreement in sufficient detail to allow the Development Costs to be determined accurately. GSK will have the right during such three (3) year period to appoint an independent certified public accountant reasonably acceptable to Amicus (the “ GSK Auditor ”) to inspect those books or records of Amicus that pertain to Development Costs. Upon not less than sixty (60) days’ prior written notice from GSK, Amicus shall permit such GSK Auditor to inspect those books or records of Amicus that relate to its Development Costs during regular business hours, at such place or places where such records are customarily kept, for the sole purpose of verifying the amounts payable hereunder. The GSK Auditor will disclose to GSK only the amount and accuracy of payments reported and actually paid or otherwise payable under this Agreement. The GSK Auditor will send a copy of the report to Amicus at the same time it is sent to GSK. \*\*\*\*\* . Notwithstanding the foregoing, in the event that GSK demonstrates sufficient cause, giving due consideration to each of the Parties’ resources, to support the conduct of an additional inspection pursuant to this Section 3.10 within the same calendar year, the JSC shall discuss in good faith whether to require such additional inspection to take place; provided that the JSC may not unreasonably withhold its consent to such an inspection. The GSK Auditor shall be obligated to execute a reasonable confidentiality agreement prior to commencing any such inspection. Inspections conducted under this Section 3.10 shall be at the expense of GSK, provided , however , that if the designated auditor establishes an overpayment by GSK in amounts payable exceeding \*\*\*\*\* of the amount of Development Costs paid for a period covered by the inspection, then Amicus will bear all reasonable costs and expenses associated with such audit and any amounts overpaid by GSK that are established shall be paid by Amicus, together with interest on such overpaid amounts at the rate set forth in Section 16.12. GSK agrees to treat all information learned in the course of any audit or inspection as Confidential Information of Amicus.

### 3.11 Taxes .

3.11.1 Amicus warrants that Amicus is a resident for tax purposes of the United States of America and that Amicus is entitled to relief from United Kingdom income tax under the terms of the double tax agreement between the United Kingdom and the United States of America (the “ Treaty ”). Amicus shall notify GSK immediately in writing in the event that Amicus ceases to be entitled to such relief.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

3.11.2 GSK shall cooperate with Amicus in obtaining formal certification of Amicus' entitlement to relief under the Treaty. Pending receipt of formal certification from the United Kingdom Inland Revenue, GSK shall pay to Amicus the full amount of the license fee required to be paid pursuant to Section 3.1 above, without deduction of any withholding tax in accordance with the Treaty. Amicus agrees to indemnify and hold harmless GSK against any loss, damage, expense or liability arising in any way from a breach of the above warranties or any future claim by a United Kingdom tax authority alleging that GSK was not entitled to deduct withholding tax on such payments at source at the Treaty rate (other than due to Amicus having filed with the United States tax authority, but not having obtained formal certification of Amicus' entitlement to relief under the Treaty from the United Kingdom Inland Revenue, prior to receiving GSK's payment pursuant to Section 3.1 above). The royalty and other payments under this Agreement shall not be reduced by any taxes required to be withheld by any taxing authority outside of the United Kingdom.

3.11.3 If GSK assigns this Agreement to an Affiliate and GSK or its Affiliate becomes liable to withhold any taxes from royalties or other payments under this Agreement, then GSK or its Affiliate shall pay to Amicus the full amount of any royalty or other payment required to be paid, unreduced by any withholding tax and shall pay any amount owed to the relevant tax authority; provided, however, that to the extent Amicus is able to obtain credit for any taxes withheld against Amicus's tax liability and actually realizes a reduction in its tax liability as a result of the utilization of such credit, Amicus shall refund to GSK the amount of such net tax savings, as determined in the reasonable discretion of Amicus.

3.11.4 All sums payable under this Agreement are exclusive of value added tax and any other sales taxes. The Parties agree that, where appropriate, the Parties shall provide each other with a valid tax invoice, and against such invoice, the Parties shall pay the amount of any such tax to the other Party. Should such amounts of tax be refunded subsequently by the fiscal authorities, the Party receiving the refund shall immediately notify the other Party and refund these monies within thirty (30) days of receipt of such funds.

#### **IV. GOVERNANCE**

##### **4.1 Joint Steering Committee .**

4.1.1 Formation . Within thirty (30) days following the Effective Date, Amicus and GSK shall establish a joint steering committee (the "Joint Steering Committee" or "JSC") to oversee the Development and Commercialization of Product, and to review and coordinate the Development of the Product in the Field in the Territory, subject to the terms and conditions of Articles V and VI herein.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

4.1.2 Membership. The Joint Steering Committee will be composed of six (6) representatives: three (3) representatives nominated by Amicus and three (3) representatives nominated by GSK. Each such representative on the Joint Steering Committee shall be a senior executive or other member of senior management (or their designees who shall have the necessary authority to make decisions as such senior executives or other members of senior management, as applicable) of the respective Party or an Affiliate of such Party, and in each case such representatives shall have significant experience and responsibility for oversight of the Product and shall be empowered by the Party whom they represent to make decisions that are binding upon such Party with respect to the Development, Manufacture and/or Commercialization, as applicable, of the Compound and Product. Each Party may also have its Alliance Manager attend Joint Steering Committee meetings as non-voting participants. GSK and Amicus will each be entitled to replace its representatives on the Joint Steering Committee in its sole discretion at any time during the Term with representatives of similar experience and level of responsibility. The Joint Steering Committee shall be chaired by a GSK representative. With the consent of the other Party (such consent not to be unreasonably withheld), other employees or consultants of GSK or Amicus or their respective Affiliates may attend Joint Steering Committee meetings to present information or participate in discussions on an ad hoc basis as non-voting participants or observers. The Parties shall cause their respective members on the Joint Steering Committee to act in good faith in carrying out their activities on the Joint Steering Committee.

4.1.3 Duties of the Joint Steering Committee. The Joint Steering Committee will:

(a) Review and approve the Development Plan (and the associated budget for Development Costs included therein) on an annual basis, including any amendments and updates thereto;

(b) Oversee the implementation of the Development Plan by the Parties and each Party's progress towards completion of the activities allocated to such Party under the Development Plan;

(c) Review and approve changes to the Development Plan;

(d) To review and approve any necessary amendments to the Development Plan to include Phase IV Clinical Study activities;

(e) Oversee the Commercialization of Products in the Field and in the Territory during the Term;

(f) Provide a forum for the Parties to exchange information and coordinate their respective activities as set forth in this Agreement with respect to matters pertaining to the Development and Marketing Approvals for the Product in the Territory;

(g) Designate a Trademark for use on each Product;

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

(h) Review and approve all plans for publications of clinical trial results or other scientific information; and

(i) Perform such other duties as are specifically assigned to the JSC in this Agreement or otherwise agreed to in writing by the Parties.

4.1.4 Committee Meetings . The Joint Steering Committee shall meet at least once per Quarter, or more or less often as otherwise agreed to by the Parties. Joint Steering Committee meetings may be conducted by telephone, video-conference or in person as agreed to by the Parties. Unless otherwise agreed by the Parties, all in-person meetings for the Joint Steering Committee shall be held on an alternating basis between Amicus's facilities and GSK's facilities. Each Party shall bear its own personnel and travel costs and expenses relating to Joint Steering Committee, Subcommittee, or Joint Patent Subcommittee meetings, and such expenses shall not be included in Development Costs.

4.1.5 Decision-Making . Decisions of the Joint Steering Committee shall be made by unanimous vote, with each Party having (1) vote and with at least one (1) representative from each Party participating in any vote. Each Party will use reasonable efforts to achieve consensus on the Joint Steering Committee. In the event that the Joint Steering Committee fails to reach unanimous agreement with respect to a particular matter within its authority within thirty (30) days of the date such matter was first presented to the Joint Steering Committee, then such matter shall be finally decided by GSK, as follows:

(a) Disputes Related to Product Development and Regulatory Issues . Either Party may, by written notice to the other Party (an "Escalation Notice"), refer disputes regarding Development of the Product in the Territory or regulatory issues relating to the Product in the Territory to the chief executive officer of Amicus (or his/her designee) and the GSK Head of Rare Diseases Unit (or his/her designee) (the "Senior Executives"). The Parties' respective Senior Executives shall meet promptly, but in any event within thirty (30) days following the referral of such matter to the Senior Executives, and shall negotiate in good faith to resolve such matter. If the Senior Executives are unable to resolve such dispute within ten (10) days following the initial meeting of such Senior Executives, then the dispute shall be resolved by the GSK Chairman of Research and Development, such decision by the GSK Chairman of Research and Development shall become the decision of the JSC with respect to the dispute specified in the applicable Escalation Notice.

(b) Disputes Related to Manufacturing and Commercialization of Product . GSK, via the GSK representatives on the Joint Steering Committee, shall have the final decision making authority for all disputes related to Manufacturing or Commercialization of Product in the Territory, without the need to further escalate such dispute. Any such decisions made by GSK shall become the decision of the JSC.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

4.2 Subcommittees. From time to time, the Joint Steering Committee may establish subcommittees to oversee particular projects or activities within the scope of authority of the Joint Steering Committee, as it deems necessary or advisable (each, a “ Subcommittee ”). Each Subcommittee shall consist of such number of representatives of each Party as the Joint Steering Committee determines is appropriate from time to time. Each Subcommittee shall meet with such frequency as the Joint Steering Committee shall determine. Each Subcommittee shall operate by unanimous vote in all decisions, with each Party having one (1) vote and with at least one (1) representative from each Party participating in such vote. If, with respect to a matter that is subject to a Subcommittee’s decision-making authority, the Subcommittee cannot reach unanimity, except with respect to the Joint Patent Subcommittee, the matter shall be immediately referred to the Joint Steering Committee, which shall resolve such matter in accordance with Section 4.1.5.

4.2.1 Joint Patent Subcommittee. Promptly after the first Joint Steering Committee meeting the Parties will form a Joint Patent Subcommittee to oversee the Patent issues pertaining to the Compound and Products. The Joint Patent Subcommittee will be composed of one (1) representative (or such other number of representatives as the Parties may agree) from each of the Parties. The Joint Patent Subcommittee will serve as the forum to review and discuss and decide, in the first instance, all matters relating to Patents and Know How included in Amicus Intellectual Property, Program Improvements and Program Patents, shall select Patent counsel to file and prosecute Patent applications included in Amicus Intellectual Property, or constituting Program Patents, and will promptly report all discussions and decisions to the Joint Steering Committee. The Joint Patent Subcommittee shall operate by unanimous vote in all decisions, with each Party having one (1) vote and with at least one (1) representative from each Party participating in such vote. If the Joint Patent Subcommittee is unable to agree on any matter considered by the Joint Patent Subcommittee within ten (10) days after first considering such matter, it shall seek the opinion of mutually acceptable outside counsel (such opinion to be provided within ten (10) days of instruction) and, if the Joint Patent Subcommittee is still unable to agree following receipt of such outside counsel’s opinion, such matter shall be referred to the Senior Executives for resolution. If, after referral to the Senior Executives, notwithstanding anything to the contrary in Section 4.1.5, the matter has not been resolved, the Senior Executive of GSK shall make the final decision within ten (10) days of being referred such matter (which decision shall become the decision of the Joint Patent Subcommittee and the JSC); \*\*\*\*\* , shall make the final decision with respect to any dispute pertaining to \*\*\*\*\* (which decision shall become the decision of the Joint Patent Subcommittee and the JSC). At the discretion and upon unanimous consent of the Joint Patent Subcommittee, any of the ten (10) day time limits in this Section 4.2.1 may be shortened.

4.3 Alliance Managers. Within thirty (30) days following the Effective Date, each Party shall appoint a representative (“ Alliance Manager ”) to facilitate communications between the Parties and to act as a liaison between the Parties with respect to such matters as the Parties may mutually agree in order to maximize the efficiency of the collaboration. Each Alliance Manager shall be permitted to attend meetings of the JSC as a nonvoting observer, subject to the confidentiality provisions of Article XI. Each Party may replace its Alliance Manager with an alternative representative at any time with prior written notice to the other Party.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

4.4 General Communications . Each Party shall keep the other Party informed, by way of updates to the Joint Steering Committee at its meetings and as otherwise specified in this Agreement, or as reasonably requested by the other Party, as to its progress and activities relating to the Development and Commercialization of the Compound and Products in the Territory, including with respect to regulatory matters and meetings with Regulatory Authorities,. In connection therewith, Amicus and GSK shall provide each other through the Joint Steering Committee with such information regarding such progress and activities under the Development Plan and/or the Marketing Plan, or otherwise relating to the Product, as the other Party may request from time to time.

4.5 Scope of Governance . Notwithstanding the creation of the Joint Steering Committee, or any Subcommittee, each Party shall retain the rights, powers and discretion granted to it hereunder, and the Joint Steering Committee shall not be delegated or vested with rights, powers or discretion, unless such delegation or vesting is expressly provided herein, or the Parties expressly so agree in writing. Neither the Joint Steering Committee, nor any Subcommittee, will have the power to amend or modify, or waive compliance with, this Agreement, and no decision of the Joint Steering Committee, or any Party exercising a deciding vote as provided in Section 4.1.5 or Section 4.2.1, as applicable, shall be in contravention of any terms and conditions of this Agreement or shall result in any obligations (including any obligation to incur or assume any financial or other commitment, including without limitation allocation of additional FTEs to the Program) being imposed on Amicus or its Affiliates, without the express prior written consent of Amicus. It is understood and agreed that issues to be formally decided by the Joint Steering Committee are only those specific issues that are expressly provided in this Agreement to be decided by the Joint Steering Committee.

## **V. PRODUCT DEVELOPMENT AND REGULATORY ACTIVITIES**

5.1 Product Development . Subject to Section 4.1.5, the Joint Steering Committee will oversee Development of the Compound and Products in the Territory in accordance with the then-current Development Plan (including the associated budget).

### 5.1.1 Development Plans .

(a) Initial Development Plan . An initial Development plan and budget for the Product in the Field in the Territory is attached to this Agreement as Schedule 5.1 and sets out separately the Development activities to be conducted by each Party following the Effective Date and a budget for such activities (the “Initial Development Plan”). The Initial Development Plan shall be deemed to be the Development Plan for all purposes until such Initial Development Plan is amended in accordance with Sections 5.1.1(b) below.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

(b) Amendments. Subject to Section 5.1.4(c), the Joint Steering Committee shall review the Initial Development Plan (or as amended) (the Initial Development Plan, as amended, the “Development Plan”) on an ongoing basis and no less frequently than once each calendar year and shall amend the then-current Development Plan as necessary to include a reasonably detailed written plan of the JSC’s then-current estimate of the Development activities (and associated budget) \*\*\*\*\* of the period covered by such plan and an outline of Development activities (and the associated budget for such Development activities) \*\*\*\*\*. The JSC shall agree upon any amendments to be made to the Development Plan, including any changes to the budget or timelines for such Development Plan, \*\*\*\*\*, provided, that the JSC shall, to the extent possible, \*\*\*\*\*. Notwithstanding the foregoing, the JSC shall agree upon any extensions to the timelines in the Development Plan that result directly from (i) material changes to any activities pertaining to the goals specified in the Development Plan that are required or reasonably requested by the FDA or other Regulatory Authority in a Major Market, such that if not performed is likely, in the reasonable judgment of the JSC, to jeopardize the receipt of Marketing Approval of the Product in any such Major Market or (ii) other factors beyond a Party’s reasonable control. In addition, (i) unless otherwise mutually agreed by the Parties in writing, the then-current Development Plan will at all times provide for the allocation of \*\*\*\*\*Amicus FTEs in the performance of Development activities for Products in the Field in the Territory for \*\*\*\*\*; and (ii) the number of Amicus FTEs allocated under the then-current Development Plan to conduct Development activities for Products in the Field in the Territory in any calendar year covered by such Development Plan \*\*\*\*\* shall not be decreased by the JSC by more than \*\*\*\*\* on less than \*\*\*\*\* written notice to Amicus; provided that upon the occurrence of a major adverse event in the Development activities pursuant to the Development Plan (e.g. termination of a clinical study as a result of an adverse event), the JSC may provide such a notice to Amicus within a shorter period of time, but the JSC shall to provide Amicus as much notice as is reasonably practical in the circumstances. If the JSC is unable to agree upon any changes to be made to the Development Plan, including the budget included therein, then, until such time as a revised Development Plan is approved by the JSC, or established pursuant to Section 4.1.5(a) above: (x) the then-current Development Plan shall continue to govern the Parties’ respective Development activities under this Agreement; and (y) each Party shall be permitted to conduct and/or commence Development activities allocated to such Party in such preceding Development Plan and incur Development Costs consistent with such preceding Development Plan, which Development Costs shall be shared by the Parties in accordance with Sections 5.1.5 and 5.1.6 below and Schedule 5.1.5.

5.1.2 Conduct of Development Activities. Each Party shall conduct those activities allocated to such Party under the Development Plan in compliance in all material respects in accordance with good scientific and clinical practices, and Laws applicable in the country in which such activities are conducted.

5.1.3 Development Activities of GSK. GSK shall use Commercially Reasonable Efforts to carry out all clinical Development and other activities required to obtain Regulatory Approval for at least \*\*\*\*\* Product in the Field and in each Major Market. Such efforts by GSK shall include, but shall not be limited to, use of Commercially Reasonable Efforts (a) to achieve the specific overall Development goals as set forth in the Initial Development Plan, (b) to achieve such Development goals in accordance with the timelines specified in the Development Plan, and (c) \*\*\*\*\*.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

5.1.4 Development Activities of Amicus . Amicus shall use Commercially Reasonable Efforts to carry out all clinical Development and other activities allocated to Amicus under the then-current Development Plan, in accordance with the then-current Development Plan and as directed by the Joint Steering Committee. Such efforts by Amicus shall include, but shall not be limited to, use of Commercially Reasonable Efforts to achieve the goals of the then-current Development Plan in accordance with the timelines specified therein.

(a) Except as otherwise mutually agreed in writing by the Parties and subject to Section 5.1.4(b) below, following the Effective Date and with oversight by the Joint Steering Committee, Amicus shall continue to conduct the existing 011 Phase III Clinical Study and the 012 Phase III Clinical Study, in each case as set forth in the Development Plan.

(b) GSK may, in its sole discretion and at its sole option, elect to participate in the conduct of the 011 Phase III Clinical Study and/or the 012 Phase III Clinical Study, in each case under the direction of the Joint Steering Committee. In the event that GSK elects to participate in the conduct of the 011 Phase III Clinical Study and/or the 012 Phase III Clinical Study, as applicable, then GSK shall provide notice of its election to participate in such studies to the Joint Steering Committee. Thereafter, subject to Section 5.1.1(b) above and Section 5.1.4.(c) below, the JSC shall re-allocate responsibilities to each Party in the Development Plan as necessary for the conduct of the 011 Phase III Clinical Study and/or the 012 Phase III Clinical Study, as applicable.

(c) Without limiting Section 5.1.4(a) above, it is understood that the Development Plan will at all times provide for Amicus to have an active role in the Development activities for Products in the Field in the Territory.

5.1.5 Allocation of Funding of Development Plan . Subject to the terms and conditions of this Agreement (including Sections 5.1.6, 12.1.2, 13.6, 14.2.2 and 14.3 below) and provided that Amicus' share of the Development Costs incurred under and in accordance with the Development Plan for each of the calendar years of the Term of the Agreement shall be subject to the applicable Amicus Annual Cost Cap and, ultimately, the Amicus Aggregate Development Cost Cap, Amicus and GSK shall share in the Development Costs to jointly fund the Development of Product for the Territory pursuant to the Development Plan, as follows and in accordance with the provisions of Schedule 5.1.5 :

(a) Amicus shall fund one hundred percent (100%) of the Development Costs as set forth in the Initial Development Plan incurred in the conduct of Development activities under and in accordance with then-current Development Plan from the Effective Date through and until December 31, 2010 and the GSK 2010 FTE Costs actually incurred by GSK, but Amicus shall not be obligated to fund more than an amount equal to the Amicus Annual Cost Cap for calendar year 2010, and GSK shall fund the remaining Development Costs incurred in the conduct of activities during such period pursuant to the Development Plan for calendar year 2010. For the avoidance of doubt, Amicus shall not defer until 2011 any costs incurred by Amicus in conducting Development in 2010.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

(b) From January 1, 2011 through and including December 31, 2011, Amicus shall fund fifty percent (50%) of the Development Costs incurred under and in accordance with the then-current Development Plan during such period, but shall not be obligated to fund more than an amount equal to the applicable Amicus Annual Cost Cap, and GSK shall fund the remaining Development Costs incurred in the conduct of activities during such period pursuant to the Development Plan.

(c) Except as provided in Section 5.1.6 below, from January 1, 2012 and for each calendar year (or part thereof) thereafter until the Amicus Aggregate Development Cost Cap is reached, Amicus shall fund on an annual basis twenty-five percent (25%) of the Development Costs incurred under and in accordance with the then-current Development Plan during such period, but shall not be obligated to fund more than an amount equal to the Amicus Annual Cost Cap for the relevant calendar year, and GSK shall fund the remaining Development Costs incurred in the conduct of Development activities pursuant to the Development Plan for each such calendar year.

(d) To the extent that, for any calendar year commencing with calendar year 2011, Amicus has been required, pursuant to Section 5.1.5(b) or (c) above, as applicable, to fund an amount of Development Costs less than the Amicus Annual Cost Cap for the applicable calendar year due to the actual amount of Development Costs for such calendar year being less than the amount of Development Costs budgeted in the then-current Development Plan for such calendar year, then the difference between the Amicus Annual Cost Cap for such calendar year and the share of the Development Costs for such calendar year actually required to be paid by Amicus pursuant to Section 5.1.5(b) or (c) above, as applicable, shall be carried forward into the next calendar year and added to the Amicus Annual Cost Cap for that subsequent calendar year and each subsequent year thereafter until such “carry-forward” amounts are exhausted; provided, however that in no event shall the aggregate of the Amicus Annual Cost Caps calculated in accordance with this Section 5.1.5(d) exceed the Amicus Aggregate Development Cost Cap.

5.1.6 Funding for Excess Development Costs. If, prior to any termination of Amicus’ co-Development rights and obligations pursuant to Sections 12.1.2, 13.6, 14.2.2 or 14.3, the total Development Costs incurred under and in accordance with the Development Plan exceed the Total Program Development Costs in the Initial Development Plan by more than \*\*\*\*\*, Amicus shall be responsible for \*\*\*\*\* of such additional Development Costs that are between \*\*\*\*\* and \*\*\*\*\* of the Total Program Development Costs in the Initial Development Plan (such additional Development Costs, the “Overage”) and GSK shall be responsible for all additional Development Costs. Amicus shall pay its \*\*\*\*\* share of the Overage on a quarterly basis in accordance with the provisions of Schedule 5.1.5 until such amount is paid in full.

5.1.7 Use of Clinical Trial Data. Subject to Section 2.1 and pursuant to the procedures set out in Section 5.2.3, Amicus shall make available to GSK, and GSK shall have complete access to, at no charge to GSK, all clinical trial data and all additional data, in each case, to the extent included in the Amicus Know-How, resulting from any clinical trials performed by Amicus, its Affiliates, or licensee with respect to the Compound and Product in the Territory. GSK shall be free to use all such data and information, as necessary or as required, to support the Development, Manufacture and Commercialization of the Compound and Product in the Territory in accordance with the terms and conditions of this Agreement.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

## 5.2 Regulatory Matters.

5.2.1 Assignment of, and Responsibility for, Regulatory Filings. Promptly following the Effective Date, Amicus and GSK will establish a plan and timeline to transfer and assign ownership of all existing Marketing Approvals and other filings (if applicable) with Regulatory Authorities for the Compound and Product in the Territory to GSK, except as solely necessary for Amicus to conduct the 011 Phase III Clinical Study and/or 012 Phase III Clinical Study, as applicable. GSK thereafter shall own and shall have the sole responsibility, as overseen by the Joint Steering Committee during the Development of Product, to hold and maintain all Marketing Approvals and other filings with Regulatory Authorities for the Products in the Territory and, during the Term, GSK shall use Commercially Reasonable Efforts to hold and maintain all such Marketing Approvals and filings with Regulatory Authorities. Further, GSK will be solely responsible for filing and obtaining INDs, MAAs and/or Marketing Approvals (including pricing or reimbursement approvals) from the applicable Regulatory Authorities in connection with the Development, Manufacture, use, and Commercialization of the Compound and Products in the Territory as overseen by the Joint Steering Committee and GSK will use Commercially Reasonable Efforts to obtain any such necessary approvals. GSK shall also be responsible for obtaining any export approvals required by a relevant Regulatory Authority to import or export the Product to any country within the Territory. All such activities shall be done in consultation with the Joint Steering Committee, and GSK shall reasonably consider in good faith the comments of Amicus with respect to such activities. All such regulatory filings will be in the name of GSK or its Affiliate, except where otherwise required by applicable Law in any country within the Territory.

5.2.2 Regulatory Cooperation. GSK shall lead the liaison with, and will manage, all interactions with Regulatory Authorities in the Territory in relation to the Product during the Term of the Agreement. Subject to Section 4.1.5, GSK agrees to consult with Amicus with respect to substantive interactions with Regulatory Authorities in each of the Major Markets, provided, however, that such agreement to consult with Amicus shall not be construed or interpreted to prevent or delay GSK from making any decisions with respect to regulatory matters in a timely manner (e.g. during a meeting with a Regulatory Authority). In any event, GSK shall keep Amicus and the Joint Steering Committee informed with respect to all interactions with Regulatory Authorities in the Territory and will reasonably consider in good faith the comments of Amicus with respect to such activities. Amicus will also provide reasonable cooperation and assistance to GSK, as reasonably requested by GSK, in the event that GSK must respond to questions from Regulatory Authorities in the Territory concerning Development activities conducted by or on behalf of Amicus with the Compound and Product. If requested by GSK, Amicus will attend all relevant meetings with Regulatory Agencies in an observational and advisory role. Each Party will promptly provide the other Party and the JSC with copies of, or electronic access to, all material documents and correspondence received from, or submitted to, a Regulatory Authority in each Major Market related to the Compound or a Product, including any notices of, or requests for, any substantive meetings with a Regulatory Authority in a Major Market relating to the Compound or a Product.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

### 5.2.3 Exchange of Data and Know-How.

(a) By Amicus. Promptly following the Effective Date, Amicus will make available to GSK, at no cost or expense to GSK, all Amicus Know-How that is necessary or materially useful for GSK to Develop, Manufacture, and Commercialize the Compound and Products in the Territory, including all patent prosecution files for all GSK Prosecuted Amicus Patents, data from any and all clinical trials and preclinical studies and non-clinical development work for the Compound and Products included in the Amicus Know-How that have been obtained by Amicus, its Affiliates or licensees prior to the Effective Date. During the Term, Amicus shall provide to GSK promptly upon the request of GSK and at no cost or expense to GSK, all Amicus Know-How that has not previously been provided to GSK. Amicus shall provide all Amicus Know-How in electronic form to the extent the same exists in electronic form, and shall provide copies as reasonably requested and an opportunity for GSK or its designee to inspect (and copy) all other materials comprising such Know-How (including for example, original patient report forms and other original source data). The Parties will cooperate and reasonably agree upon formats and procedures to facilitate the orderly and efficient exchange of the Amicus Know-How during the Term.

(b) Provision of Data to the JSC. Upon request by the JSC, each Party shall promptly provide to the JSC summaries in reasonable detail of all data generated or obtained in the course of such Party's performance of activities under the Development Plan.

5.2.4 Sharing of Regulatory Filings. Without limiting Section 5.2.3 above, each Party shall, upon reasonable request of the other Party, permit the other Party to access, and shall provide the other Party with sufficient rights to reference and use in association with exercising its rights and performing its obligations under this Agreement, all of such Party's, and its Affiliates' and, to the extent it has the right to do so, its licensees' and Sublicensees' data, regulatory filings and regulatory communications associated with any submissions of MAAs or other approvals for Product in the Territory.

5.2.5 Clinical Trial Register. Notwithstanding anything in this Agreement to the contrary, GSK shall have the right to publish in its clinical trial register the results or summaries of the results of all clinical trials for the Compound and Product conducted by either Party, their Affiliates, licensees' and Sublicensees' (subject to Sections 5.2.4 and 11.5) in the Territory pursuant to this Agreement.

### 5.2.6 Adverse Event Reporting.

(a) Pharmacovigilance Agreement. The Parties shall enter into a pharmacovigilance agreement on terms no less stringent than those required by ICH guidelines, including: (i) providing detailed procedures regarding the maintenance of core safety information and the exchange of safety data relating to the Compound and Products in the Territory within appropriate timeframes and in an appropriate format to enable each Party to meet both expedited and periodic regulatory reporting requirements; and (ii) ensuring compliance with the reporting requirements of all applicable Regulatory Authorities on a worldwide basis for the reporting of safety data in accordance with standards stipulated in the ICH guidelines, and all applicable regulatory and legal requirements regarding the management of safety data.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

(b) Adverse Event Reporting. As between the Parties: GSK shall be responsible for the timely reporting of all Product quality complaints, adverse drug reactions/experiences/events, Product complaints and safety data relating to the Compound and Product to appropriate Regulatory Authorities in accordance with the applicable Laws of the relevant countries and Regulatory Authorities in the Territory. GSK shall respond in a timely manner to safety issues related to the Product in the Territory, as required pursuant to applicable Law. GSK shall ensure that its Affiliates and Sublicensees comply with such reporting obligations in the Territory.

(c) Global Safety Database. As between the Parties: GSK shall maintain a unified worldwide global safety database with respect to the Product in the Territory.

5.2.7 Termination of Ongoing Development and Committee Obligations. The Parties' obligations under Sections 5.1 and 5.2 (and the Development Plans), and to share Development Costs under Section 5.1.5 and 5.1.6 above, and Amicus' supply and Manufacturing obligations under Section 6.5 below, shall terminate \*\*\*\*\* after the Effective Date (to the extent such obligations have not already terminated or expired). At such time, the Joint Steering Committee and all Subcommittees will terminate. However, each Party will continue to have an approval right with respect to matters specified to be decided by the Joint Steering Committee or any Subcommittee under this Agreement. In such event, if the Parties are unable to reach agreement on a matter specified in this Agreement to have been decided by the Joint Steering Committee or such Subcommittee (other than the Joint Patent Subcommittee), then the matter shall be determined in accordance with Section 4.1.5(a). Further, in such event, if the Parties are unable to reach agreement on a matter specified in this Agreement to have been decided by the Joint Patent Subcommittee, then the Parties shall seek the opinion of mutually acceptable outside counsel (such opinion to be provided within ten (10) days of instruction) and, if the Parties are unable to agree following receipt of such outside counsel's opinion, then such matter shall be referred to the Senior Executives for resolution. If, after referral to the Senior Executives, notwithstanding anything to the contrary in Section 4.1.5, such matter has not been resolved, the Senior Executive of GSK shall make the final decision within ten (10) days of being referred such matter; except that the Senior Executive of Amicus, not GSK, shall make the final decision with respect to any dispute pertaining to an Amicus Prosecuted Patent.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

## **VI. COMMERCIALIZATION AND PROMOTION; TRANSFER OF MANUFACTURING RESPONSIBILITIES AND INTERIM SUPPLY**

6.1 Marketing Plan. Prior to the anticipated Launch of the first Product in the United States or a Major EU Country, GSK shall prepare the Marketing Plan, which shall include without limitation the Marketing Strategy, and shall provide such Marketing Plan to the Joint Steering Committee. GSK, not less frequently than \*\*\*\*\*, will provide to the JSC summary updates to the Marketing Plan and summary updates of Commercialization activities undertaken by GSK and its Affiliates and Sublicensees pursuant to the Marketing Plan \*\*\*\*\*. In addition, GSK will update the JSC on a rolling basis during the JSC's quarterly meetings with respect to activities conducted pursuant to the then-current Marketing Plan \*\*\*\*\*. Except for any Commercialization activities allocated to Amicus pursuant to Section 6.1.2 below, GSK shall carry out all marketing, promotion and other Commercialization activities of the Products in the Territory in accordance with the then-current Marketing Plan

6.1.1 GSK's Responsibility. GSK shall have, in GSK's sole discretion and at its sole expense, and using Commercially Reasonable Efforts, the exclusive right to Manufacture (subject to Section 6.5 below), Commercialize, distribute, market, provide sales force support for and to promote Product in the Field in the Territory, including, without limitation, the exclusive right and responsibility for the following in the Territory:

(a) negotiating with relevant governmental authorities and agencies and MCOs to establish pricing and reimbursement for Products in the Territory;

(b) managed care contracting for Products in the Territory, provided that GSK shall not engage in any Discriminatory Conduct with respect to managed care (including, Medicare) contracting or otherwise relating to the Products. For the purposes of this Section 6.1.1(b), "Discriminatory Conduct" shall be deemed to occur if GSK or its Affiliate discounts the price of or positions Product in its managed care contracting or otherwise to benefit or increase the sales of other products of GSK or its Affiliate;

(c) receiving, accepting and filling orders for Products from customers in the Territory;

(d) distributing Products to customers in the Territory;

(e) controlling invoicing, order processing and collecting accounts receivable for sales of Products in the Territory;

(f) recording sales of Products in the Territory in its books of account for sales;

(g) conducting disease awareness and education programs in the Territory; and

(h) any and all other Commercialization activities, in GSK's discretion, related to Compound or Product in the Territory.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

GSK shall use Commercially Reasonable Efforts to Commercialize the Product in each of the Major Markets.

6.1.2 Amicus' Responsibilities. Notwithstanding the foregoing, Amicus shall have, and the Marketing Plan shall provide Amicus with the following, subject to a determination by the JSC that Amicus shall have such a role, which determination shall be made on reasonable (and in no event less than three (3) months) written notice to Amicus:

(a) an active role in connection with:

(i) medical affairs activities with respect to Products in the United States;

(ii) medical liaison activities with respect to Products with key opinion leaders in the United States;

(iii) disease awareness and education and patient advocacy programs with respect to Products in the United States and such other countries within the Territory as the Parties shall mutually agree; and

(b) an advisory role in connection with strategic marketing activities and creation and maintenance of the Marketing Strategy for Products in the United States and such other countries within the Territory as the Parties shall mutually agree;

provided, however, that any activities conducted by Amicus in connection therewith would be conducted by Amicus in accordance with the then-current Marketing Plan using Commercially Reasonable Efforts and with GSK oversight and in full compliance with and adherence to all applicable GSK policies (to the extent the same and any updates thereto are disclosed to Amicus in writing) and all applicable Laws.

6.2 Promotional Materials. The determination of the content, quantity, and method of distribution of any promotional materials for the Compound or Product for the Territory shall be the sole responsibility of GSK. Subject to Section 6.3, GSK shall own all right, title and interest in and to all such promotional materials created during the Term of the Agreement, including any intellectual property rights therein or attendant thereto, excluding any Amicus Trademark(s) and the Amicus House Marks.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

6.3 Use of Trademarks and House Marks . The Joint Steering Committee will determine which Trademark or Trademarks will be used in marketing Product in the Territory. Further, all packaging, and package inserts for Product in the Territory shall, along with the GSK brand name and logo or other identifying markings of GSK or its Affiliates (collectively, the “ GSK House Marks ”), include the Amicus brand name and logo (such Amicus brand name and logo, collectively “ Amicus House Marks ”) in reasonable size and prominence as allowed by applicable Law; it being understood that the exact size, placement and prominence of such Amicus House Marks shall be determined by GSK in its reasonable discretion. Amicus hereby grants to GSK a non-exclusive, royalty-free license, with the right to grant sublicenses as provided in Section 2.2, to use the Amicus House Marks in connection with the developing, making, having made, use, sale, offering for sale, importation, packaging, distributing and promotion of the Products in the Field in the Territory. The ownership and all goodwill from the use of the Amicus House Marks shall vest in and inure to the benefit of Amicus. Solely to the extent necessary to preserve Amicus’s legal rights in the Amicus House Marks, GSK shall submit to Amicus, not less than fifteen (15) days prior to their proposed distribution, representative packaging for the Product displaying the Amicus House Marks for Amicus’s review and written approval solely with respect to GSK’s use of the Amicus House Marks, which approval will not be unreasonably withheld or delayed. If Amicus has not responded within thirty (30) days after the submission of such packaging for the Product, Amicus’s approval to GSK’s use of the Amicus House Marks on such packaging will be deemed to have been received. GSK may make any subsequent changes to packaging bearing the Amicus House Marks, other than changes to the Amicus House Marks, without the subsequent approval from Amicus.

6.4 Product Recalls . At the direction of the Joint Steering Committee and subject to Article XV, GSK will have the responsibility for any total or partial recall or market withdrawal of a Product in the Territory (whether voluntary or not). Amicus will cooperate with and assist GSK in effecting such recall or market withdrawal, including making available to GSK, upon request, all of Amicus’s pertinent records. All costs associated with any total or partial recall or market withdrawal of the Product in the Territory shall be borne by GSK; provided that to the extent that such total or partial recall or market withdrawal is as a result of Amicus’s (or Amicus’s Third Party manufacturer’s) gross negligence or failure to comply with the terms of this Agreement, all such costs shall be borne by Amicus.

6.5 Manufacturing Responsibilities . From the Effective Date, subject to Sections 6.5.1 and 6.5.2 below, GSK (itself or through an Affiliate) shall have the exclusive right to Manufacture Compound and Product for distribution in the Territory, including, without limitation, all batches of drug substance and drug product (including any such batches of drug substance or drug product planned for use to support registration and validation of Product), and will have the right, in accordance with the terms of this Agreement, to appoint one or more Third Parties to Manufacture Compound and Product for such purposes. For the avoidance of doubt, GSK shall have the ultimate decision making authority over the use of Third Parties in its manufacturing supply chain.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

6.5.1 Transition of Manufacturing and Supply Responsibilities . Promptly following the Effective Date, Amicus and GSK shall develop and reasonably agree upon a detailed plan (“ Supply Transition Plan ”) to transfer to GSK responsibility for Manufacturing of the Compound and Products for use in the Field in the Territory, such transfer of responsibility to occur no later than \*\*\*\*\* (“ Supply Transition Date ”). Amicus shall cooperate in good faith with GSK to effect the transfer of such Manufacturing responsibilities to GSK in an orderly manner in accordance with the Supply Transition Plan. Without limiting the foregoing, Amicus shall deliver to GSK in accordance with the Supply Transition Plan, all information Controlled by Amicus (including such information generated by Amicus’s Third Party manufacturer(s)) as is reasonably necessary or useful for GSK or its Affiliates or its Third Party manufacturer, to commence Manufacturing Compound or Product following the Supply Transition Date. In connection therewith, any such Supply Transition Plan shall incorporate the technology transfer requirements set forth in Schedule 6.5.1 attached hereto, to the extent applicable.

6.5.2 Activities Prior to the Supply Transition Date . During the period on and from the Effective Date until the Supply Transition Date, as between the Parties, subject to Section 6.5.2(b):

(a) Amicus shall supply, or will direct its Third Party manufacturers to supply, the requirements of Product and Compound necessary to conduct and complete the clinical studies and other Development activities under the Development Plan, and the costs of such quantities of Compound and Product shall be included in the Development Costs to be shared by the Parties pursuant to Sections 5.1.5 and 5.1.6 and Schedule 5.1.5 .

(b) Amicus shall maintain its arrangements with any Third Party manufacturers of the Compound and/or Products in effect as of the Effective Date and shall, under the direction of GSK, direct the management of its Third Party manufacturers in existence as of the Effective Date. Notwithstanding the foregoing, in the event that the JSC determines that the Parties should re-negotiate a current agreement or arrangement for the supply of the Compound and Products, or negotiate an agreement for the supply of the Compound and Products with a new Third Party supplier, including but not limited to any agreement regarding the Manufacture of validation batches of Compound or Product, then GSK shall have the right to approve such Third Party supplier, to lead the negotiation of any such agreement in such manner as the Parties may reasonably agree, and to enter into an agreement with such Third Party regarding the supply of the Compound and Products; and upon execution of any such agreement between GSK and a Third Party supplier, Amicus shall be relieved of all of its obligations with respect to all of the activities for the Compound and Product performed by such Third Party supplier, and GSK shall have no further rights, under this Section 6.5.2. As between the Parties, Amicus shall be responsible for making any payments due to any of its Third Party manufacturers with respect to the Compound and/or Products prior to the Supply Transition Date, it being understood that such amounts shall be included in the Development Costs to be shared by the Parties pursuant to Sections 5.1.5 and 5.1.6 and Schedule 5.1.5 .

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

(c) GSK shall have, and Amicus shall procure for GSK, the right for GSK to audit and inspect each Amicus Third Party manufacturer's records and those portions of each facility used in the Manufacture of the active pharmaceutical ingredient, drug substance and drug product related to the Compound or Product, in each case on reasonable notice (and in any event not less than two (2) business days) and during normal business hours for the purpose of ensuring compliance with this Section 6.5.2. Amicus acknowledges that GSK Global Quality Policies and Guidelines (a copy of which has been provided to Amicus prior to the Effective Date) represent GSK's interpretation of cGMPs and that GSK will use the GSK Global Quality Policies and Guidelines to assess whether each Third Party manufacturer has performed its obligations with respect to the active pharmaceutical ingredient, drug substance and drug product related to the Compound or Product in a manner that is consistent with cGMPs. Purposes for such inspections may include cGMPs compliance, system audits, compiling information for reporting obligations, compliance with specifications, compliance with quality agreement, financial audits to verify amounts invoiced to GSK, and/or investigations of complaints and/or compliance with any Laws, the Environmental, Health and Safety Guidelines, or the terms of this Agreement (including all representations and warranties of Amicus hereunder).

6.5.3 Activities After the Supply Transition Date . On and from the Supply Transition Date, as between the Parties:

(a) GSK (itself or through an Affiliate or a Third Party manufacturer) shall have the exclusive right to Manufacture Compound and Product for distribution in the Territory; except that, if this Agreement is terminated by Amicus pursuant to Section 13.2 or by GSK pursuant to Section 13.3 with respect to a particular Product(s) and/or in a particular country(ies) of the Territory, Amicus shall have the non-exclusive right to Manufacture Compound and/or Terminated Products at locations within the Territory solely for use in the Development of any Terminated Products and/or for sale and/or use in the Affected Area.

(b) At the written request of GSK, Amicus will assign or facilitate the transfer to GSK of any agreements between Amicus and its Third Party manufacturers of the Compound and/or Products existing as of the Effective Date, including any and all such supply and quality agreements with such Third Party manufacturers, to the fullest extent possible, provided that such assignment or transfer is permitted under the supply agreement and/or is accepted by GSK and the Third Party.

(c) GSK agrees to make available, and supply to Amicus, Amicus's and its Affiliates requirements of the Compound and the Products necessary or reasonably useful for Amicus to conduct and complete the Development activities allocated to Amicus and/or its Affiliates under and in accordance with the then-current Development Plan; and the costs of such quantities of Compound and Product shall be included in the Development Costs to be shared by the Parties pursuant to Sections 5.1.5 and 5.1.6 and Schedule 5.1.5 .

6.5.4 Supply of Compound and Product . Any quantities of Compound and Product to be supplied by one Party to the other Party pursuant to this Section 6.5 will meet applicable Compound or Product specifications. The specifications for the Compound and Products as of the Effective Date attached hereto as Schedule 6.5.4 and shall be amended only upon agreement by the JSC. In addition, all quantities of Compound and Product supplied by one Party to the other Party pursuant to this Section 6.5 will not be misbranded or adulterated. All such Compound and/or Product will be manufactured in accordance with cGMPs; provided, however, that a Party may supply Compound not manufactured in accordance with cGMP if specifically intended for non-human testing activities allocated to the other Party under the Development Plan and as agreed to in writing in advance by such other Party.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

## **VII. OWNERSHIP AND INTELLECTUAL PROPERTY**

7.1 Ownership. Subject to GSK's license rights under the License and any license under the Amicus Trademarks and/or Amicus House Marks granted to GSK pursuant to Sections 2.1, 2.4, or 6.3, as applicable, as between the Parties, Amicus will own or Control the Amicus Intellectual Property, Amicus Confidential Information, Amicus Know-How, Amicus Trademarks and Amicus House Mark owned or Controlled by Amicus in the Territory as of the Effective Date. Subject to any license or other rights granted to Amicus pursuant to the terms of Section 2.3, 2.4, or Section 14.3 below, GSK will own the GSK Confidential Information, GSK Background IP, GSK Trademarks, and GSK House Marks owned or Controlled by GSK as of the Effective Date. GSK shall own any Trademarks that are created or designated by the JSC for use on the Product in the Territory after the Effective Date, subject to Section 14.3.9.

### 7.2 Patent Applications on Licensed Technology.

7.2.1 GSK Control of Prosecution. Subject to any restrictions Amicus may have under any Third Party agreement covering the Amicus Patents included in the Licensed Technology (including the Background License Agreements) and except for those Amicus Prosecuted Patents described below, GSK will assume control of, \*\*\*\*\*, prosecuting and maintaining such Patents included in the Amicus Patents as of the Effective Date, or which may be filed in any country of the Territory after the Effective Date, to the extent the same are directed to the Compound or a Product, and/or Manufacturing and/or use thereof, in the Field in the Territory (such Patents, excluding the Amicus Prosecuted Patents, are referred to below as the "GSK Prosecuted Amicus Patents"). A list of the GSK Prosecuted Amicus Patents, as of the Effective Date, is set forth on Schedule 7.2.1, hereto. Amicus shall have the right, \*\*\*\*\*, to reasonably assist GSK in connection with the filing, prosecution and maintenance of any GSK Prosecuted Amicus Patents in the Territory. GSK shall use diligent efforts consistent with those normally employed by GSK in the course of business to prosecute and maintain the GSK Prosecuted Amicus Patents described in this Section 7.2.1 and GSK will, in a timely manner, solicit Amicus' comments regarding the prosecution and maintenance of such GSK Prosecuted Amicus Patents and review of the nature and text of any such Patent application and prosecution matters related thereto, including any correspondence between GSK and any government intellectual property or Patent authorities, agencies or other government bodies, in reasonably sufficient time prior to filing thereof, and GSK shall give due consideration to Amicus' reasonable amendments to such correspondence. Without prejudice to Section 7.2.4, in the event that GSK intends to disregard any of Amicus' amendments, GSK shall set up a meeting between Amicus's and GSK's respective patent counsels to provide explanations therefor.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

7.2.2 Amicus Prosecuted Patents. Amicus will control, \*\*\*\*\*, prosecuting and maintaining the Amicus Patents identified on Schedule 7.2.2 (together with future Patents claiming priority thereto, the “Amicus Prosecuted Patents”). GSK shall have the right, \*\*\*\*\*, to reasonably assist Amicus in connection with the filing, prosecution and maintenance of any such Amicus Prosecuted Patents in the Territory. Amicus shall use diligent efforts consistent with those normally employed by Amicus in the course of business to prosecute and maintain the Amicus Prosecuted Patents and Amicus will, in a timely manner, solicit GSK’s comments regarding the prosecution and maintenance of such Amicus Prosecuted Patents and review of the nature and text of any such Patent application and prosecution matters related thereto, including any correspondence between Amicus and any government intellectual property or Patent authorities, agencies or other government bodies, in reasonably sufficient time prior to filing thereof, and Amicus will give due consideration to GSK’s reasonable comments and amendments. Without prejudice to Section 7.2.4, \*\*\*\*\*.

7.2.3 Segregation of Patent Applications. In the event that any Amicus Patent contains claims to the Compound and/or Products (i.e. is a GSK Prosecuted Amicus Patent) as well as the Amicus Proprietary Chaperone Technology more broadly or any other compound or product owned or controlled by Amicus, the Parties shall cooperate in good faith to segregate such Patents to allow Amicus to control the prosecution and maintenance of Patent applications and Patents pertaining to subject matter other than the Compound and/or Products. A list of such Segregated Patents as of the Effective Date is set forth on Schedule 7.2.3 hereto.

7.2.4 Additional Matters. Any disagreements under this Section 7.2 shall be referred to the Joint Patent Subcommittee for resolution as provided in Section 4.2.1. For purposes of this Article 7, “prosecution and maintenance” (including variations such as “prosecute and maintain”) means, with respect to a Patent, the preparing, filing, maintenance and prosecution of such Patent, as well as the conduct of interferences, oppositions, re-examination, re-issues and other similar proceedings.

### 7.3 Program Improvements

7.3.1 To the extent that a Program Improvement is developed by or on behalf of one Party, that Party will promptly disclose such Program Improvement to the Joint Patent Subcommittee in writing with all relevant data supporting such Program Improvement.

7.3.2 Each Party will, subject to the terms of Section 7.4, be sole owner of Program Improvements invented solely by its employees and agents and the employees and agents of its respective Affiliates and will do and procure all necessary acts, and obtain all necessary assignments or other instruments as may be required to confer such sole ownership on said Party. With respect to such solely-invented Program Improvements, the Party owning such Program Improvement will own any applications for Patent with respect thereto and any Patents issued on such applications, unless such rights are assigned to the other Party pursuant to Section 7.4.

7.3.3 The Parties will be the joint owners of Program Improvements invented jointly by the employees and agents of the Parties or the employees and agents of their respective Affiliates and any Program Patents covering such jointly invented Program Improvements (each a “Joint Program Patent”). Each Party will do and procure all necessary acts, and obtain all necessary assignments or other instruments as may be required to confer such joint ownership on the Parties.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

7.3.4 Inventorship under this Agreement shall be determined in accordance with the patent laws of the United States.

7.4 Abandonment of Patents and Applications . In the event that GSK decides not to file, continue to prosecute or maintain a GSK Prosecuted Amicus Patent that falls under Section 7.2, or either Party decides not to file, maintain a Patent or to abandon a Patent application or issued Patent that falls under Section 7.3 (in either case, the “ Abandoning Party ”), such Abandoning Party will give written notice to the other Party at least sixty (60) days prior to any public disclosure, allowing such application to go abandoned, or prior to not taking a necessary step to maintain such Patent, and the other Party will have the option of taking over the prosecution or maintenance of such application or Patent at its sole expense. If the other Party elects to take over the filing, prosecution or maintenance of such application or Patent pursuant to this Section 7.4, the Abandoning Party or Party giving permission will assign all its right, title and interest in such application or Patent to the other Party, subject to the Abandoning Party or Party giving permission retaining a non-exclusive, perpetual, irrevocable, sublicensable, fully-paid-up license from the other Party to such Patent or Patent application. The Party taking over prosecution, or maintenance will, in a timely manner, solicit the Abandoning Party’s comments in prosecution matters related to such applications, including any correspondence between the Abandoning Party and any government intellectual property or Patent authorities, agencies or other government bodies, in reasonably sufficient time prior to filing thereof, and shall give due consideration to the Abandoning Party’s comments. Any disagreements hereunder shall be referred to the Joint Patent Subcommittee for resolution as provided in Section 4.2.1.

7.5 Cooperation . Each Party will cooperate, and will require its employees, Affiliates, consultants and subcontractors to cooperate, with all reasonable requests of the other Party for assistance in preparation and prosecution and maintenance of any applications for Patent and any Patent issuing therefrom and any applications for Trademark and any registration issuing therefrom that is owned by the requesting Party hereunder. GSK shall be solely responsible for any and all costs associated with the GSK Trademarks and GSK House Marks, including any Trademarks owned by GSK pursuant to Section 7.1 herein. Amicus shall be solely responsible for any and all costs associated with the Amicus Trademarks and Amicus House Marks.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

## 7.6 Patent Filing Procedures for Patents relating to Program Improvements .

### 7.6.1 Program Improvement relating to Compound or Product .

(a) If a Program Improvement relates to Compound or Product, then GSK will determine whether or not to file a Patent application in the Territory on such Program Improvement. If GSK elects to file such an application, \*\*\*\*\* prosecuting and maintaining any Patents that issue thereon and will control the prosecution of such application; however, Amicus shall have the right, \*\*\*\*\* to reasonably assist GSK in connection with the filing, prosecution and maintenance of any Patent applications filed under this Section 7.6.1(a). GSK shall use diligent efforts consistent with those normally employed by GSK in the course of business to prosecute and maintain any such Patent application or Patent described in this Section 7.6.1(a) and GSK will, in a timely manner, solicit Amicus' comments and review of the nature and text of any such application and prosecution and maintenance matters related thereto, including any correspondence between GSK and any government intellectual property or Patent authorities, agencies, or other government bodies, in reasonably sufficient time prior to filing thereof, and GSK shall give due consideration to Amicus' reasonable amendments to such correspondence. \*\*\*\*\* Any remaining disagreements hereunder, including filing, prosecution and maintenance decisions or strategies and/or any disputes by Amicus regarding GSK's determination to disregard any of Amicus' proposed amendments provided with respect to an application for Patent or Patent described in this Section 7.6.1(a), shall be referred to the Joint Patent Subcommittee for resolution as provided in Section 4.2.1; and

(b) If GSK elects not to file an application for Patent in any country in the Territory covering any such Program Improvement, GSK shall give Amicus notice thereof at least sixty (60) days prior to causing in any way such Program Improvement to become unpatentable through disclosure, sale, or otherwise, and Amicus shall thereafter have the right, at its sole expense, to prosecute and maintain such Patent application in any such country. Any disagreements hereunder shall be referred to the Joint Patent Subcommittee for resolution as provided in Section 4.2.1.

### 7.6.2 Program Improvement not relating to Compound or Product.

(a) If a Program Improvement does not relate to Compound or Product, then Amicus will determine whether or not to file a Patent application in the Territory on such Program Improvement. If Amicus elects to file such an application, \*\*\*\*\* prosecuting and maintaining any Patents that issue thereon and will control the prosecution of such application; however, GSK shall have the right, \*\*\*\*\* to reasonably assist Amicus in connection with the filing, prosecution and maintenance of any Patent applications filed under this Section 7.6.2(a). Amicus shall use diligent efforts consistent with those normally employed by Amicus in the course of business to prosecute and maintain any such Patent application or Patent described in this Section 7.6.2(a) and Amicus will, in a timely manner, solicit GSK's comments and review of the nature and text of any such application and prosecution and maintenance matters related thereto, including any correspondence between Amicus and any government intellectual property or Patent authorities, agencies, or other government bodies, in reasonably sufficient time prior to filing thereof, and Amicus shall give due consideration to GSK's reasonable amendments to such correspondence. \*\*\*\*\* Any remaining disagreements hereunder, including filing, prosecution and maintenance decisions or strategies, shall be referred to the Joint Patent Subcommittee for resolution as provided in Section 4.2.1; and

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

(b) If Amicus elects not to file an application for Patent in any country in the Territory covering any such Program Improvement, Amicus shall give GSK notice thereof at least sixty (60) days prior to causing in any way such Program Improvement to become unpatentable through disclosure, sale, or otherwise, and GSK shall thereafter have the right, at its sole expense, to prosecute and maintain such Patent application in any such country. Any disagreements hereunder shall be referred to the Joint Patent Subcommittee for resolution as provided in Section 4.2.1.

#### 7.7 Orange Book Listing; Patent Term Restoration and Supplemental Protection Certificates.

7.7.1 GSK's Obligations. GSK will be responsible for listing with the applicable Regulatory Authorities in the Territory during the Term of the Agreement, all applicable Patents included within the Licensed Technology in the U.S. FDA's Orange Book (or equivalent). Prior to such listings, the Parties will meet, through the Joint Patent Subcommittee, to evaluate and identify all applicable Patent rights, and subject to any restrictions Amicus may have under third party agreements covering the Amicus Patents included in the Licensed Technology (including the Background License Agreements), GSK will have the right to review, where reasonable, original records relating to any invention for which Patent rights are being considered for any such listing. Notwithstanding the foregoing, GSK will determine, in its sole discretion, which Patents in the Territory included within the Licensed Technology shall be listed in the Orange Book (or equivalent) for a Product, regardless of which Party owns such Patent. In addition, subject to any restrictions Amicus may have under third party agreements covering the Amicus Patents included in the Licensed Technology (including the Background License Agreements), GSK shall determine, in its sole discretion, to which Patents included within the Licensed Technology (excluding Patents relating solely to the Amicus Proprietary Chaperone Technology) GSK would apply the U.S. Hatch-Waxman extension and Supplementary Protection Certificate Extensions and other Patent Term Extensions for countries in the Territory. The Parties will cooperate with each other in gaining Patent term extension where applicable to Products. Upon GSK's reasonable request, Amicus shall timely provide any documentation or other assistance required in order to obtain such Patent term extensions, subject to any restrictions Amicus may have under third party agreements covering the applicable Amicus Patent (including the Background License Agreements).

7.8 Trademark Filing Procedures. The Joint Steering Committee may also designate, pursuant to Sections 2.4, 4.1.3 and 6.3, a Trademark for use on a Product in the Territory after the Effective Date. In the event that the Joint Steering Committee so designates a Trademark for use on a Product after the Effective Date and determines that an application for Trademark registration shall be made with respect to such designated Trademark (including determining in which countries within the Territory such Trademark applications shall be filed and maintained), GSK will undertake to thereafter file and maintain such Trademark application for registration and Trademark registration, as applicable. GSK shall be solely responsible for filing and maintaining, at its sole expense, any GSK Trademarks, GSK House Marks and any GSK domain names owned by GSK prior to the Effective Date and used on or in connection with Product. Amicus shall be solely responsible for filing and maintaining, at its sole expense, any Amicus Trademarks, Amicus House Marks and any Amicus domain names owned by Amicus prior to the Effective Date and used on or in connection with Product. In addition, each Party shall be responsible, at such Party's expense, for conducting Trademark searches, filing Trademark applications, and maintaining any Trademark registrations for any Trademarks that are considered by such Party to be a back-up Trademark for such Party's Trademarks or House Marks.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

## **VIII. ENFORCEMENT AND DEFENSE OF INTELLECTUAL PROPERTY**

8.1 Notices. Each Party will advise the Joint Steering Committee and the Joint Patent Subcommittee promptly upon its becoming aware of: (a) any unlicensed activities which such Party believes may be an actual or impending infringement of any Patent or other proprietary right owned or applied for by it or the other Party included in the Amicus Patents or Program Patents by a Product, or the Development, Manufacture, use, importation, or sale thereof; (b) any attack on or appeal of the grant of any Patent owned or applied for by it or the other Party to the extent containing claims to the Compound or a Product or the Development, Manufacture, use, or sale thereof; (c) any application for Patent by, or the grant of a Patent to, a Third Party in respect of rights which may be related to the Compound or a Product so as to potentially materially affect the Development, Manufacture, use, importation, or sale thereof; (d) any application made for a compulsory license under any Patent owned or applied for by it or the other Party and covering the Compound or Product or the Development, Manufacture, use, importation, or sale thereof in the Territory; or (e) any application for Patent by, or the grant of a Patent to, a Third Party in respect of rights which may claim the same subject matter as, or conflict with, any Patent owned or applied for by it or the other Party containing claims to the Compound or a Product, or the Development, Manufacture, use, importation, or sale thereof.

8.2 Control of Actions. Subject to any restrictions Amicus may have under a Third Party agreement covering the Amicus Patents included in the Licensed Technology (including the Background License Agreements), GSK will determine whether or not to take whatever legal or other action is required in response to activities in the Territory requiring notice under Section 8.1(a), (c) or (d) to the extent such activities specifically relate to Compound or Product (“Protective Action”). If GSK determines that such Protective Action is warranted, in its sole discretion, then, subject to any restrictions Amicus may have under a Third Party agreement covering the Amicus Patents included in the Licensed Technology (including the Background License Agreements), GSK shall, at GSK’s expense, commence, prosecute and control such Protective Action, including the settlement thereof and the granting of any licenses or sublicense within the scope of the License in the Territory under any Amicus Intellectual Property or Program Improvement licensed to GSK hereunder. Amicus will cooperate with GSK in such action, including being joined as a Party to such action if such joinder is necessary for standing. Each Party may be represented by counsel of its own selection at its own expense in such Protective Action. Any recovery obtained as a result of such Protective Action and attributable to activities in the Territory, whether by judgment, award, decree, or settlement, will, after reimbursement of the Parties for their reasonable costs and expenses associated with such Protective Action, be treated as Net Sales of Product. To the extent such recovery is insufficient to reimburse the Parties’ associated reasonable costs and expenses fully, then a Party’s share of such recovery will be the product of the total amount recovered with that Party’s reasonable costs and expenses divided by the sum of both Parties’ reasonable costs and expenses. The Party responsible pursuant to Section 7.2, 7.3 or 7.6 above, as applicable for prosecution and maintenance of the relevant Patent described in Section 8.1(b), and Section 8.1(e) shall determine whether or not to take whatever legal or other action is required with respect to the activities described in Section 8.1(b) and Section 8.1(e). For the avoidance of doubt, Amicus will determine and control any legal or other action in response to activities equivalent to those described in Section 8.1(a), Section 8.1(c) and Section 8.1(d) that do not specifically relate to Compound or Product.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

8.3 Trademark Infringement . Notice regarding potential infringement of and control of any Protective Action relating to any Amicus Trademark or GSK Trademark in any country of the Territory related to the Compound or a Product or the Development, Manufacture, use, importation, or sale thereof in the Territory will be addressed in accordance with the applicable Trademark License Agreement.

8.4 Third Party Claims . GSK and Amicus will each promptly notify the Joint Steering Committee and the Joint Patent Subcommittee of any Claim by a Third Party against GSK or Amicus, or any Affiliate or sublicensee of Amicus or GSK, alleging infringement of such Third Party's intellectual property rights as a result of the Development, Manufacture, marketing, sale, importation, or use of the Compound or Product in the Territory. As directed by the Joint Steering Committee, the Parties will cooperate and use Commercially Reasonable Efforts to resolve such claimed infringement, and GSK shall be entitled to lead in the defense and shall select its counsel, and Amicus shall have the right to participate in such action, and to select its own counsel at its own expense. If it appears reasonably likely that the claimed infringement will give rise to a Claim for indemnification hereunder, then the Party against whom such Claim for indemnification would be made will have the first right to defend against such Claim in accordance with Article XV.

## **IX. REPRESENTATIONS AND WARRANTIES**

9.1 Representations and Warranties of Both Parties . Amicus and GSK each hereby represent and warrant to the other, as of the Effective Date, as follows:

9.1.1 It is a corporation, duly organized, validly existing and in good standing under the laws of the jurisdiction of its incorporation and has all requisite power and authority, corporate or otherwise, to conduct its business as now being conducted, to own, lease and operate its properties and to execute, deliver and perform this Agreement.

9.1.2 No consent, approval, order or authorization of, or registration, declaration or filing with, any governmental agency is required to be obtained or made by or with respect to such Party in connection with its execution, delivery and performance of this Agreement.

9.1.3 The execution, delivery and performance by it of this Agreement and the transactions contemplated thereby have been duly authorized by all necessary corporate action and stockholder action and will not (i) violate any applicable Laws or (ii) result in a breach of or constitute a default under any material agreement, mortgage, lease, license, permit or other instrument or obligation to which it is a party or by which it or its properties may be bound or affected.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

9.2 Representations and Warranties of Amicus . Amicus hereby represents and warrants to GSK, as of the Effective Date, as follows:

9.2.1 It has the full right, power and authority to enter into this Agreement and to grant the License to GSK.

9.2.2 Except as otherwise may have been disclosed by Amicus to GSK prior the Effective Date, Amicus has received no notice that (a) the manufacture, sale, importation or use of the Compound within the Field as contemplated hereby infringes any Third Party rights, and (b) the Amicus Patents (to the extent representing issued Patents) are invalid or unenforceable.

9.2.3 To Amicus's knowledge, there are no errors in the inventorship set forth in any of the Patent applications comprising Amicus Patents.

9.2.4 Except as provided or limited in Article II, the Amicus Intellectual Property constitutes all intellectual property that is Controlled by Amicus and used in the Development and/or Manufacture of the Compound, and Amicus does not Control any additional Patents, Know-How or information that are necessary for GSK to Develop, Manufacture and Commercialize the Compound.

9.2.5 To Amicus's knowledge, no Third Party Controls any Patent that is necessary for GSK to Develop, Manufacture and Commercialize the Compound as such activities are currently conducted or currently proposed to be conducted.

9.2.6 It has not previously granted any right, license or interest in or to the Amicus Patents, or any portion thereof, that is in conflict with the rights or licenses granted to GSK under this Agreement.

9.2.7 There are no investigations, inquiries, actions or other proceedings pending before any Regulatory Authority with respect to the Compound, and Amicus has not received written notice threatening any such investigation, inquiry, action or other proceeding.

9.2.8 The Development of the Compound by or on behalf of Amicus has been conducted in compliance in all material respects with all applicable Laws; and neither Amicus nor to Amicus's knowledge, its Third Party contractors, have received any written notice which has led Amicus to believe that any of the Regulatory Approvals relating to the Compound or Product developed by Amicus are not currently in good standing with the FDA or EMA and Amicus has no knowledge that any of its Third Party contractors has developed Compound or Product in a manner that does not comply in all material respects with all applicable Laws.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

9.2.9 Other than the Background License Agreements, as of the Effective Date, there are no other agreements to which Amicus is a party or, to Amicus's knowledge, that would prevent Amicus from performing its obligations under this Agreement or GSK from exercising the rights under the Amicus Intellectual Property under and in accordance with the License.

9.2.10 To its knowledge, there is no pending or threatened product liability action in relation to the Compound, and it is not aware of any grounds for any such product liability action.

9.2.11 Amicus has all material permits, licenses, franchises, authorizations, orders and approvals of, and has made all filings, applications and registrations with, governmental entities that are required in order to permit Amicus to own or lease properties and assets and to carry on its business as presently conducted that are material to Amicus. Amicus has complied and is in compliance in all material respects with all statutes, laws, regulations, rules, judgments, orders and decrees of all governmental entities applicable to it that pertain to its business, including but not limited to compliance with the U.S. Foreign Corrupt Practices Act of 1977 (FCPA) (15 U.S.C. §§ 78dd-1, et seq.) and any applicable similar laws in foreign jurisdictions in which Amicus is currently, or has previously, conducted its business or is currently, or has previously, conducted clinical trials. Amicus has not received any notice from a governmental entity alleging noncompliance with any such applicable statutes, laws, regulations, rules, judgments, orders and decrees, and, to the knowledge of Amicus, Amicus is not under investigation with respect to, or threatened to be charged, with any material violation of any applicable statutes, laws, regulations, rules, judgments, orders or decrees of any governmental entities.

9.2.12 It has not, up through and including the Effective Date, knowingly withheld any material information in its possession from GSK in response to GSK's reasonable inquiries in connection with GSK's due diligence relating to the Compound, this Agreement and the underlying transaction, and to its knowledge, the information related to the Compound that Amicus has provided to GSK prior to the Effective Date is up-to-date and accurate in all material respects.

**9.3 Mutual Limitations on Warranties.** OTHER THAN THE REPRESENTATIONS AND WARRANTIES MADE BY THE PARTIES PURSUANT TO SECTIONS 9.1 AND 9.2, THE PARTIES DISCLAIM ANY AND ALL OTHER REPRESENTATIONS AND WARRANTIES WHETHER EXPRESS OR IMPLIED, INCLUDING ANY REPRESENTATIONS OR WARRANTIES OF NON-INFRINGEMENT, MERCHANTABILITY, OR FITNESS FOR A PARTICULAR PURPOSE OR ANY REPRESENTATIONS OR WARRANTY ARISING FROM COURSE OF DEALING OR USAGE OF TRADE.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

## X. COVENANTS

### 10.1 Conduct of Activities .

10.1.1 Throughout the Term, Amicus and GSK will comply in all material respects with all applicable Laws concerning the Development, Manufacture, and Commercialization of the Compound or Products.

10.1.2 Neither Amicus nor GSK, nor any of their respective employees or consultants who shall be undertaking any activities related to this Agreement or the subject matter thereof, shall have been debarred or shall be the subject of debarment or other disciplinary proceedings by the FDA or any Regulatory Authority in the Territory.

### 10.2 Background License Agreements .

10.2.1 It is understood that certain Patents and Know-How included within the Amicus Intellectual Property have been licensed pursuant to the Background License Agreements and that the obligations of Amicus and the rights of GSK under this Agreement shall be subject to, and limited by, the Background License Agreements.

10.2.2 It is further understood that each Background License Agreement may require that particular provisions be incorporated into a sublicense granted thereunder. The text of any such provisions in the Background License Agreements is set out on Schedule 10.2 attached hereto and shall be deemed incorporated by reference into this Agreement. GSK agrees to be bound by the provisions set out on Schedule 10.2 to the extent applicable to GSK in its capacity as a sublicensee under each Background License Agreement and, to the extent required by any Background License Agreement, the relevant Third Party licensor shall be deemed to be a third party beneficiary of this Agreement for the purposes of enforcing such Third Party licensor's rights against GSK in its capacity as a sublicensee under the applicable Background License Agreement. In addition, GSK, in its capacity as a sublicensee under each Background License Agreement, agrees to comply with the obligations applicable to sublicensees under such agreement, as set forth on Schedule 10.2.

10.2.3 Except as the Parties may otherwise mutually agree or as provided in Section 3.5.2(c), Amicus shall not amend, without the prior written consent of GSK (such consent not to be unreasonably withheld or delayed), or voluntarily terminate, its rights under any Background License Agreement in any manner that would materially and adversely affect GSK's rights and licenses under this Agreement. Amicus shall promptly notify GSK of any notice of breach delivered by it, or any termination or amendment of any of the Background License Agreements that materially and adversely affects GSK's rights and licenses under this Agreement.

10.3 Non-Compete . \*\*\*\*\*.

10.4 Non-Solicitation . \*\*\*\*\*.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

## **XI. CONFIDENTIAL INFORMATION**

### **11.1 Confidentiality.**

11.1.1 During the Term and for five (5) years thereafter, each Party will keep, and cause its Affiliates and Sublicensees, if any, to keep confidential all Confidential Information of the other Party, and neither Party, nor any of its Affiliates or Sublicensees, if any, will use or disclose the Confidential Information of the other Party except as expressly permitted in this Agreement. The Parties acknowledge that Confidential Information may have been disclosed by either Party or its Affiliates to the other Party or its Affiliates pursuant to the Confidentiality Agreement. All information disclosed pursuant to the Confidentiality Agreement will be deemed Confidential Information of the disclosing Party within the meaning of this Agreement and subject to the terms hereof.

11.1.2 The fact that a particular item of information is not or has ceased to be Confidential Information by virtue of one or more of the exclusions specified in the definition of Confidential Information (the “Excluded Item”) shall not relieve the Party who obtained or received the Excluded Item from that Party’s obligation of confidentiality and non-use (a) as to any other item of Confidential Information of the other Party or (b) as to the relationship of the Excluded Item to any other item of Confidential Information of the other Party.

11.1.3 Each Party hereby acknowledges that the Confidential Information of the other Party is highly valuable, proprietary, and confidential and that any use or disclosure of the other Party’s Confidential Information, including any disclosures made to any Person or governmental agency in connection with the conduct of a clinical study pursuant to Development Plan, will be made only to the extent reasonably necessary to carry out such Party’s responsibilities or exercise the rights granted to, or reserved by it, under this Agreement. Any disclosure of the other Party’s Confidential Information shall be made to an officer, employee, agent, or permitted Sublicensee or contractor of a Party or any of its Affiliates only if such officer, employee, agent, or permitted sublicensee is informed of the confidential nature thereof and shall have agreed to hold such information in confidence and not to use such Confidential Information under confidentiality provisions at least as stringent as those provided in this Agreement, and each Party shall be responsible for any breach of such obligation of confidentiality by its or its Affiliates officers, employees, agents, permitted Sublicensees and/or contractors.

11.1.4 The Parties agree that the obligations of this Section 11.1 are necessary and reasonable in order to protect the Parties’ respective businesses, and that monetary damages alone may be inadequate to compensate a Party for any breach by the other Party or any of its Affiliates or their respective officers, employees, or agents of its covenants and agreements set forth herein. The Parties agree that any breach or threatened breach of this Section 11.1 may cause irreparable injury to the injured Party for which damages may not be an adequate remedy and that, in addition to any other remedies that may be available, in Law and equity or otherwise, such Party will be entitled to seek equitable relief against the breach or threatened breach of the provisions of this Section 11.1.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

## 11.2 Disclosure of Terms; Public Announcements .

11.2.1 Initial Press Release . Notwithstanding Section 11.3 below, the Parties have agreed on an initial press release of the transaction contemplated by this Agreement which is attached hereto as Exhibit B (the “ Initial Press Release ”). The Initial Press Release may be issued or used by each Party individually or by the Parties jointly on or after the Effective Date. Thereafter, each Party may disclose the information contained in such press release without need for further approval by the other.

11.2.2 Further Publicity . The Parties acknowledge the importance of supporting each other’s efforts to publicly disclose results and significant development regarding the Compound and Products in the Territory and other activities in connection with this Agreement in the Territory that reflect information that is not otherwise permitted to be disclosed under this Article 11, beyond what is required by Law, and each Party may make such public disclosures from time to time with the approval of the other Party, which approval will not to be unreasonably withheld or delayed. Such disclosures may include, without limitation, achievement of milestones, significant events in the Development or regulatory process and/or the Launch of a Product in a Major Market. When a Party (the “ Requesting Party ”) elects to make any such public disclosure under this Section 11.2.2, it will give the other Party (the “ Cooperating Party ”) at least seven (7) business days notice to review and comment on such statement, and in any event the Cooperating Party shall work diligently and reasonably to agree on the text of any proposed disclosure in an expeditious manner. The principles to be observed in such disclosures shall be accuracy, compliance with applicable Law and regulatory guidance documents, reasonable sensitivity to potential negative reactions of the FDA (and its foreign counterparts) and the need to keep investors informed regarding the Requesting Party’s business.

11.3 Confidential Terms; Required Disclosure . Each Party agrees not to disclose to any Third Party the terms of this Agreement without the prior written consent of the other Party hereto, except each Party may disclose the terms of this Agreement to its advisors (including financial advisors, attorneys and accountants), actual or potential acquisition partners or private investors, and others on a need to know basis, in each case under appropriate confidentiality provisions substantially equivalent to those in this Agreement. A Party will be entitled to disclose the terms of this Agreement and/or Confidential Information of the disclosing Party where such disclosure is reasonably necessary to prosecute or defend any litigation or otherwise enforce its rights pursuant to this Agreement, or where demand for such disclosure is made on such Party or otherwise required pursuant to: (i) a valid order of a court or other governmental body or (ii) any other applicable Law; provided that if such Party, as the receiving Party, intends to make such disclosure or receives such demand, to the extent it may legally do so, the receiving Party shall give the disclosing Party prompt notice thereof to enable the disclosing Party to seek a protective order or other appropriate remedy concerning any such disclosure. The receiving Party will co-operate with the disclosing Party at the disclosing Party’s expense in connection with the disclosing Party’s efforts to obtain any such order or other remedy. If any such order or other remedy does not fully preclude disclosure, the receiving Party will make such disclosure only to the extent that such disclosure is legally required and subject to confidentiality, to the extent available. Notwithstanding the foregoing, the Parties agree to work together to prepare a redacted version of this Agreement to be filed by Amicus with the United States Securities Exchange Commission.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

11.4 Clinical Trial Register . Nothing herein shall limit GSK's right to publish in its clinical trial register the results or summaries of the results of all clinical trials for the Compound or Products in the Territory as set forth in Section 5.2.5 herein.

11.5 Publications . Except as otherwise expressly set forth herein and subject to JSC review and approval of plans for publications as set forth in Section 4.1.3 (h) and excluding publications made pursuant to Section 11.4, GSK shall have the right to publish manuscripts, abstracts, or other articles in scientific journals pertaining specifically to any Product in the Territory without obtaining the prior written consent of Amicus, and subject to the procedures specified in this Section 11.5; provided, however, that Amicus shall have the right to review and comment upon, such comments to be considered by GSK in good faith, such manuscripts, abstracts, or other articles in which an Amicus employee is also named as an author or which includes Know-How or other information pertaining specifically to the Compound or a Product that has not previously been published pursuant to this Section 11.5. Amicus may publish manuscripts, abstracts, or other articles in scientific journals pertaining specifically to Compound or Product in the Territory upon the prior written consent of GSK, such consent not to be unreasonably withheld. In the event that either Party desires to make a publication pursuant to this Section 11.5, such Party shall provide a copy of the proposed manuscript (including abstracts, or presentation to a journal, editor, meeting, seminar or other third party) to the other Party for its review and comments at least forty-five (45) days (or fourteen (14) days for any abstract submitted to a conference) prior to submission of such proposed manuscript for publication; the object being to prevent either the endangerment of applications for the protection of intellectual property rights by premature publications detrimental to their novelty or the disclosure of Confidential Information. If, during the forty-five (45) days (or fourteen (14) days, as applicable) specified above the non-publishing Party notifies the other Party that a proposed manuscript contains patentable subject matter which requires protection, the non-publishing Party may require the delay of the publication for a period of time not to exceed forty-five (45) days (or fourteen (14) days, for any abstract submitted to a conference) for the purpose of allowing the pursuit of such protection. The publishing Party shall delete from the proposed manuscript prior to submission all Confidential Information of the non-publishing Party that the non-publishing Party identifies in good faith and requests to be deleted. If no response is received from the non-publishing Party within forty-five (45) days (or fourteen (14) days, as applicable) of the date the proposed manuscript was submitted to the non-publishing Party, it may be conclusively presumed that the publication may proceed without delay. Notwithstanding the foregoing, but without limiting either Party's rights under Section 11.3, in the event that a Party believes in good faith that it is obligated or appropriate to disclose any information pertaining to the safety of a Product, then such Party shall immediately notify the other Party and the Senior Executives of each Party shall meet within \*\*\*\*\* thereafter to discuss disclosure of such information. In the event that the Senior Executives are unable to agree upon whether or not to disclose such information within \*\*\*\*\* after such meeting, then the matter shall be referred to the Joint Steering Committee, which shall meet shall meet as expeditiously as possible to fully and finally resolve the dispute.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

## **XII. CHANGE OF CONTROL**

12.1 **Change of Control of Amicus** . Amicus shall notify GSK in writing within fifteen (15) days of the closing of any Change of Control of Amicus. If, during the Term, a Change of Control event occurs that involves a Major Pharmaceutical Company, then:

12.1.1 Amicus' rights and obligations to participate in any Commercialization activities shall automatically terminate, effective as of the effective date of the closing of the Change of Control event, without the need for further notice of such termination;

12.1.2 GSK shall have the right, exercisable in GSK's sole discretion upon written notice to Amicus, to terminate any and all of Amicus' rights to co-Develop Product with GSK pursuant to this Agreement; and in such case, all of Amicus' rights and obligations to share in Development Costs incurred in the Development of Products under Sections 5.1.5 and 5.1.6 and Schedule 5.1.5 shall also terminate;

12.1.3 All of the rights and licenses granted to GSK pursuant to this Agreement shall continue in full force and effect, unchanged and unaffected; and

12.1.4 GSK shall have the right to terminate and dissolve the JSC and to take any actions, or make any decisions, in its sole discretion, previously reserved for the JSC. Notwithstanding the foregoing, in such event, GSK shall not have the right to terminate and dissolve the Joint Patent Subcommittee, and the Joint Patent Subcommittee shall continue in effect with the responsibilities described in, and decisions made in accordance with, Section 4.2.1 until the last Patent application included within the Licensed Technology or Program Patents Controlled by GSK has been granted or rejected in a final, unappealable decision by the relevant governmental authority after which GSK may terminate and dissolve the Joint Patent Subcommittee, and all obligations of the Joint Patent Subcommittee shall vest exclusively in GSK, including the right to make a final decision on matters originally within the scope of responsibilities of the Joint Patent Subcommittee, except that Amicus, not GSK, shall have the right to make the final decision with respect to any matter pertaining to an Amicus Prosecuted Patent.

### 12.2 **Divestment of Product** .

12.2.1 If, as a result of a Change of Control of Amicus, Amicus becomes obligated to divest rights to the Compound and/or Product in one (1) or more countries in the Territory, Amicus shall promptly notify GSK in writing. Within \*\*\*\*\* of the date of such notice from Amicus, GSK shall notify Amicus in writing whether or not it is interested in acquiring all of Amicus' rights to the Compound and Product in such country or countries in the Territory ("Election Notice"). If GSK does not provide an Election Notice to Amicus within such \*\*\*\*\* period, Amicus shall be free to grant one or more Third Parties rights to acquire the Compound and Product in the applicable country or countries of the Territory, without further obligation to GSK under this Section 12.2.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

12.2.2 If GSK does provide an Election Notice to Amicus within the \*\*\*\*\* period specified in Section 12.2.1 above, the Parties shall discuss mutually acceptable terms upon which GSK may acquire all of Amicus' rights to the Compound and Product in such country or countries in the Territory, including all of Amicus' co-Development and Commercialization participation rights with respect to the Product in such country or countries of the Territory. If the Parties agree on such terms, the Parties shall prepare and execute a definitive agreement setting forth the agreed terms (“Product Acquisition Agreement”). If the Parties have not entered into a Product Acquisition Agreement within \*\*\*\*\* after the date of such Election Notice (“Negotiation Period”), then Amicus shall be free to complete a sale of the Compound and Product in such country or countries in the Territory to a Third Party without further obligation to GSK under this Section 12.2; provided that Amicus shall not complete a sale of the Compound and Product in such country or countries in the Territory to a Third Party on financial terms more favorable than the last financial terms upon which Amicus offered such rights to GSK during the Negotiation Period without first following the procedures set forth in Section 12.2.3 below.

12.2.3 If Amicus wishes to offer to a Third Party the right to acquire the Compound and Product in the applicable country or countries of the Territory on financial terms that are more favorable than the last financial terms upon which Amicus offered such rights to GSK, then to the extent that Amicus has a continuing obligation to GSK under Section 12.2.2, Amicus shall provide notice of the same to GSK (“Re-Offer Notice”) and provide a revised term sheet of proposed terms (“Revised Terms”). In such event, the Parties shall repeat the procedures of Section 12.2.2, except that the Negotiation Period shall be \*\*\*\*\* from the date of the Re-Offer Notice and GSK's Election Notice must be provided within \*\*\*\*\* of its receipt of the Re-Offer Notice. If GSK provides an Election Notice pursuant to this Section 12.2.3 and desires to accept the Revised Terms, it shall so agree in writing within the \*\*\*\*\* Negotiation Period, in which case the Parties shall enter into a Product Acquisition Agreement reflecting such Revised Terms and such other terms as are reasonable.

12.2.4 The only obligations of GSK and Amicus under this Section 12.2 are as expressly stated herein, and there are no further implied obligations relating to the matters contemplated herein. Without limiting the foregoing, Amicus is not obligated at any time to disclose the identity of any Third Party with whom it is discussing a Third Party agreement. Notwithstanding the foregoing, in no event shall GSK's rights and licenses to the Product be altered by any such divestiture by Amicus to a Third Party of Amicus's rights to the Product in a country or countries of the Territory.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

12.3 Amicus Intellectual Property. Notwithstanding any provision of this Agreement, in the event of a Change of Control of Amicus, the scope of the Licensed Technology (including the Amicus Patents, Amicus Know-How and other Amicus Intellectual Property) and the Compound and Product and the rights and licenses granted to GSK with respect to the Compound and Products under this Agreement (including those in Sections 2.1, 2.2, 5.1.7 and 5.2.4 and Article 11), shall not include, and nothing herein shall be construed to include, any of the Patents, Know-How or other intellectual property or subject matter that (i) was owned or Controlled by the acquiring entity or its Affiliates prior to the closing of such Change of Control of Amicus, or (ii) any intellectual property rights that the acquiring entity or any of its Affiliates develops following a Change of Control of Amicus independently without using any of the Amicus Intellectual Property.

12.4 For the avoidance of doubt, except as set out in this Article 12, a Change of Control of Amicus shall not otherwise affect the rights or obligations of the Parties with respect to the Compound and Products in the Territory under this Agreement, and shall not be deemed to modify or expand the scope of GSK's rights under Section 2.1 above.

### **XIII. TERM AND TERMINATION**

13.1 Term. This Agreement shall commence on the Effective Date, and unless terminated earlier as provided in this Article XIII, shall continue in full force and effect on a country-by-country and Product-by-Product basis until the expiration of the Royalty Term in each country in the Territory (the "Term"). Upon the expiration of the Royalty Term in each country of the Territory, subject to Section 3.5.2(b), GSK shall have a perpetual, exclusive, fully-paid up, royalty-free license in such country under the Licensed Technology to make, have made, use, sale, offer for sale and import Product in such country.

13.2 Termination for Material Breach. Either Party may terminate this Agreement in its entirety, or at the non-breaching Party's discretion, on a country-by-country or Product-by-Product basis, by written notice to the other Party in the event that the other Party is in material default or material breach of any of its obligations hereunder, and fails to remedy such default or breach within a period of \*\*\*\*\* after written notice thereof was provided to the breaching Party by the non-breaching Party. Any such termination shall become effective at the end of such \*\*\*\*\* period unless the breaching Party has cured any such breach or default prior to the expiration of the \*\*\*\*\* period.

13.3 Termination for Convenience. GSK may terminate this Agreement in its entirety, or on a country-by-country or Product-by-Product basis, for any reason whatsoever, upon \*\*\*\*\* prior written notice to Amicus.

13.4 Bankruptcy. Either Party may terminate this Agreement in its entirety at any time during the Term by giving written notice to the other Party if the other Party files in any court or agency pursuant to any statute or regulation of any state or country, a petition in bankruptcy or insolvency or for reorganization or for an arrangement or for the appointment of a receiver or trustee of the Party or of its assets, or if the other Party is served with an involuntary petition against it, filed in any insolvency proceeding, and such petition will not be dismissed within \*\*\*\*\* after the filing thereof, or if the other Party makes a general assignment for the benefit of creditors.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

13.5 By Mutual Consent. The Parties may terminate this Agreement in its entirety or on a Product-by-Product or country-by-country basis at any time and for any reason during the Term upon their mutual written agreement.

13.6 Termination of Amicus' Co-Development Right for Product. After \*\*\*\*\*, Amicus may, in its sole discretion, elect to terminate its right to co-Develop Products with GSK pursuant to Section 5.1 and to share Development Costs incurred in the Development of Products pursuant to Sections 5.1.5 and 5.1.6 and Schedule 5.1.5 (such option, the "Co-Development Opt-Out"). To exercise the Co-Development Opt-Out, Amicus shall provide written notice to GSK ("Co-Development Opt-Out Notice") at least \*\*\*\*\*prior to the beginning of the first Quarter in which Amicus wishes such Co-Development Opt-Out to take effect, which notice shall not be given prior to \*\*\*\*\* ("First Opt-Out Quarter"). Upon delivery of the Co-Development Opt-Out Notice, Amicus's Co-Development rights with respect to the Products pursuant to Section 5.1, and Amicus' obligations to pay Development Costs incurred in the Development of Products pursuant to Sections 5.1.5 and 5.1.6 and Schedule 5.1.5, shall terminate as of the first day of the First Opt-Out Quarter and the royalty rates applicable to Net Sales of Products specified in Section 3.4.1 shall be reduced in accordance with the specific methodology for such reduction set forth in Schedule 13.6.

#### **XIV. EFFECTS OF TERMINATION**

14.1 Accrued Obligations. The expiration or termination of this Agreement for any reason shall not release either Party from any liability that, at the time of such expiration or termination, has already accrued to the other Party or that it is attributable to a period prior to such expiration or termination, nor will any termination of this Agreement preclude either Party from pursuing any and all rights and remedies it may have under this Agreement, or at law or in equity, with respect to breach of this Agreement.

14.2 Rights upon Termination by GSK for Amicus Breach. If GSK terminates this Agreement in its entirety, or terminates this Agreement with respect to a Product or country, pursuant to Section 13.2, then the provisions of this Section 14.2 shall apply. As used in this Section 14.2 and Section 14.3 below, "Affected Area" shall mean the Territory in the case of termination of this Agreement in its entirety, or the terminated country(ies) in the case of termination of this Agreement with respect to such country(ies) and "Terminated Product(s)" shall mean the Compound and all Products in the case of termination of this Agreement in its entirety, or the terminated Product(s) in the case of termination of this Agreement with respect to such Product(s).

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

#### 14.2.1 Licenses .

(a) The License granted to GSK pursuant to Section 2.1 above will survive any such termination and will convert to an irrevocable exclusive license, with the right to sublicense in accordance with Section 2.2, under the Licensed Technology solely with respect to the Terminated Products in the Affected Area. The licenses granted to GSK under any Amicus Trademark pursuant to a Trademark License Agreement and/or the Amicus House Marks pursuant to Section 6.3 will also survive any such termination and will convert to an irrevocable license solely with respect to the Terminated Products in the Affected Area, with the right to sublicense in accordance with the applicable Trademark License Agreement or Section 6.3, as applicable; unless Amicus assigns any Amicus terminated Product Trademark to GSK in accordance with Section 14.2.2 below, in which event GSK's license under the applicable Amicus Terminated Product Trademark pursuant to the Trademark License Agreement shall terminate upon the assignment of such Amicus Terminated Product Trademark to GSK.

(b) The licenses granted by GSK to Amicus pursuant to Section 2.3(a) to Develop the Compound and Product in the Field in the Territory in accordance with Article V shall survive any termination of this Agreement by GSK pursuant to Section 13.2; unless GSK terminates this Agreement pursuant to Section 13.2 in its entirety. If such termination by GSK occurs prior to the Supply Transition Date, the Parties will work together in good faith to complete all activities under the Supply Transition Plan as soon as possible after the date of such termination.

14.2.2 Assignment of Amicus Trademarks . Within \*\*\*\*\* after the effective date of termination, upon request by GSK, Amicus shall either return to GSK or destroy all tangible items pertaining to a Terminated Product in any country of the Affected Area and comprising, bearing or containing any GSK Trademark and/or GSK House Marks that is in Amicus' possession. Effective upon the effective date of termination, Amicus shall cease to use all Trademarks and trade names of GSK (including the GSK House Marks and all GSK Trademarks) with respect to the Terminated Products in the Affected Area, and all rights granted to Amicus hereunder to use such Trademarks and trade names of GSK with respect to the Compound and Terminated Products in the Affected Area shall terminate. In addition, at GSK's option, which shall be exercised by written notice to Amicus, and upon payment by GSK of the out-of-pocket costs of assignments and of the out-of-pocket costs incurred to identify, design and register (including but not limited to clearance filing and maintaining) any Amicus Trademark selected by the JSC for use with each Terminated Product in the Affected Area (each, an "Amicus Terminated Product Trademark"), Amicus shall promptly assign to GSK, at no additional cost to GSK (including no royalty obligations), all rights of Amicus and its Affiliates in and to such Amicus Terminated Product Trademarks, including all registrations and applications for registration for such Amicus Terminated Product Trademarks in the Affected Area and all associated goodwill. For the avoidance of doubt, the foregoing shall not include any Amicus House Marks. Further, upon payment by GSK of the out-of-pocket costs of assignment and registration, Amicus shall transfer to GSK all domain names established by Amicus for use with a Terminated Product in any country of the Affected Area.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

14.2.3 Development . If GSK terminates this Agreement in its entirety, at GSK's election exercisable on written notice to Amicus no later than \*\*\*\*\* following the effective date of any such termination, GSK may assume the conduct of all Development activities allocated to Amicus under the then-current Development Plan and all Commercialization activities allocated to Amicus under the then-current Marketing Plan (if any) in respect of the Products and thereafter, GSK will perform any and all such Development and/or Commercialization activities thereunder in accordance with the terms of this Agreement. Following GSK's termination and election to assume such Development and Commercialization activities, Amicus shall have no further rights or obligations to Develop or Commercialize the Products in the Territory, or to share in the Development Costs incurred in the performance of the Development Plan after the date of such notice of election from GSK. If GSK so elects, then GSK may off-set Amicus' share (determined in accordance with Section 5.1.5) of any documented, out-of-pocket expenses that GSK incurs directly as a result of the assumption, and the conduct of any Development activities allocated to Amicus under the Development Plan in effect as of the date of any such termination that are assumed by GSK pursuant to this Section 14.2.3 against any milestone or royalty payments owed to Amicus but not yet paid by GSK under Section 3.3 prior to the date of GSK's notice to Amicus of the applicable breach issued pursuant to Section 13.2. If GSK terminates this Agreement pursuant to Section 13.2 only with respect to a particular Product (s) and/or particular country(ies), the rights and obligations of the Parties with respect to the Development of Products and related matters set forth in Article V of this Agreement shall survive with respect to the Terminated Products in the Affected Area.

14.2.4 Payment Obligations . Subject to Section 14.2.3, the obligations of GSK, and the rights of Amicus, under Article III with respect to Terminated Products in the Affected Area will survive any such termination.

14.2.5 Committees . For the avoidance of doubt, upon termination by GSK of this Agreement in its entirety pursuant to the terms of Section 13.2 or 13.4, the Joint Steering Committee shall cease to exist and, subject to this Section 14.2.5 below, all obligations of the Joint Steering Committee shall vest exclusively in GSK, including the right to make a final decision on matters originally within the scope of responsibilities of the Joint Steering Committee, subject to Section 4.4. Notwithstanding the foregoing, GSK shall not have the right to terminate and dissolve the Joint Patent Subcommittee, and the Joint Patent Subcommittee shall continue in effect with the responsibilities described in, and decisions made in accordance with, Section 4.2.1 until the last Patent application included within the Licensed Technology or Program Patents Controlled by GSK has been granted or rejected in a final, unappealable decision by the relevant governmental authority after which GSK may terminate and dissolve the Joint Patent Subcommittee, and all obligations of the Joint Patent Subcommittee shall vest exclusively in GSK, including the right to make a final decision on matters originally within the scope of responsibilities of the Joint Patent Subcommittee, except that Amicus, not GSK, shall have the right to make the final decision with respect to any matter pertaining to an Amicus Prosecuted Patent.

14.2.6 Additional Matters .

(a) Upon any termination of this Agreement by GSK pursuant to Section 13.2, all of the Parties rights and obligations under Articles VII and VIII with respect to the Licensed Intellectual Property, Program Patents and Program Improvements shall survive.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

(b) Upon termination of this Agreement by GSK pursuant to Section 13.2: (i) with respect to all Products in a particular country(ies), all of the Parties' rights and obligations under the Agreement with respect to all Products in all countries outside of such Affected Area shall survive; or (ii) with respect to a Product(s), but not all Products, all of the Parties' rights and obligations under the Agreement with respect all such Products (i.e., the non-Terminated Products) in the Territory shall survive.

14.2.7 Non-Compete. \*\*\*\*\*.

14.2.8 Transition. Without limiting the foregoing, following termination as set forth herein, Amicus shall use Commercially Reasonable Efforts to cooperate with GSK and/or its designee to effect a smooth and orderly transition of any Development, Manufacturing or Commercialization activities with respect to a Terminated Product(s) in the Affected Area that were, prior to such termination, allocated to Amicus under the Development Plan, the Supply Transition Plan and/or the then-current Marketing Plan, respectively.

14.3 Rights upon Termination by Amicus for GSK Breach; or Termination by GSK for Convenience. If Amicus terminates this Agreement in its entirety pursuant to Section 13.2, or if GSK terminates this Agreement in its entirety or terminates this Agreement with respect to a particular Product(s) or country(ies) in the Territory, in each case pursuant to Section 13.3, then the provisions of this Section 14.3 shall apply.

14.3.1 Licenses. Effective as of the date of termination, Amicus shall have and is hereby granted by GSK a non-exclusive, irrevocable license, with the right to grant sublicenses, under any Program Improvements and Program Patents that are Controlled by GSK or its Affiliates, and any other Patents Controlled (to the extent such Patents are in-licensed, solely to the extent that GSK has the right to grant sublicenses under its licensed rights) by GSK or its Affiliates on the effective date of termination that are necessary and actually practiced prior to termination of this Agreement by GSK or its Affiliates in Developing, Manufacturing, Commercializing and otherwise exploiting the Compound and Terminated Products, for the purposes of Developing, Manufacturing, Commercializing and otherwise exploiting the Compound and Terminated Products for the Affected Area. Following the effective date of termination, the License granted by Amicus to GSK under the Licensed Technology with respect to the Terminated Product(s) in the Affected Area shall convert to a non-exclusive license and shall be limited: (i) to the use of the Licensed Technology for the purposes of permitting GSK to comply with its obligations under this Section 14.3 for the applicable periods described in Sections 14.3.2, 14.3.3 and 14.3.6 below; except that (ii) GSK shall continue to have a non-exclusive license under the Licensed Technology to make and/or have made the Terminated Product(s) in the Affected Area solely for use and sale within the Territory, unless or until this Agreement is terminated in its entirety. Except as provided in the preceding sentence for the applicable periods described in such sentence, the License, and all of GSK's rights, under the Licensed Technology with respect to the Terminated Products in the Affected Area shall terminate and shall automatically revert to Amicus upon any such termination of this Agreement. Further, any licenses granted by Amicus to GSK under the Amicus Trademarks and/or Amicus House Marks granted pursuant to a Trademark License Agreement and/or Section 6.3, respectively, for the Terminated Products in the Affected Area shall immediately terminate, except to the extent necessary for the purposes of permitting GSK to comply with its obligations under this Section 14.3 for the applicable periods described in Sections 14.3.2, 14.3.3 and 14.3.6 below; and subject to the foregoing, all right, title and interest in and to such Amicus Trademarks and Amicus House Marks with respect to the Terminated Product(s) in the Affected Area shall automatically revert to Amicus.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

14.3.2 Development. In the event that, on the date of notice of such termination, there are any ongoing clinical trials of any Terminated Product in the Affected Area (or, if the Affected Area is less than the entire world, any ongoing clinical trials of any Terminated Product that are specifically directed to the requirements of a country within the Affected Area) (each, an “Ongoing Trial”), to the extent and as requested by Amicus, following the effective date of termination, GSK will promptly transition to Amicus or its designee, or complete such Ongoing Trial(s). During the period in which GSK is performing activities in accordance with this Section 14.3.2, GSK will remain responsible for GSK’s share under Sections 5.1.5 and 5.1.6 of (i) any Development Costs incurred in the continued conduct of such Ongoing Trials and (ii) any Out-of-Pocket Expenses incurred by Amicus or GSK to transition any Ongoing Trials (or portion thereof) to Amicus or its designee, as requested by Amicus.

14.3.3 Commercialization. To avoid disruption of supply of any Terminated Products to patients if this Agreement is terminated after the Launch of a Terminated Product in the Affected Area, GSK, its Affiliates and Sublicensees shall continue to sell the Terminated Products in each country of the Affected Area for which Marketing Approval of such Terminated Product has been obtained, in accordance with the terms and conditions of this Agreement, until the date on which Amicus notifies GSK that Amicus has secured an alternative distributor or licensee for such Terminated Product in such country of the Affected Area, but in no event more than \*\*\*\*\* after the effective date of any such termination of this Agreement (“Wind-Down Period”); provided that Amicus may terminate the Wind-Down Period in any country(ies) of the Affected Area upon \*\*\*\*\* written notice to GSK; provided further that GSK shall not be obligated to promote the sale of Terminated Products in the Affected Area during the Wind-Down Period. Notwithstanding any other provision of this Agreement, during the Wind-down Period, GSK’s and its Affiliates’ and Sublicensees’ rights with respect to the Terminated Products in the Affected Area shall be non-exclusive and, without limiting the foregoing, Amicus shall have the right to engage one or more other distributor(s) and/or licensee(s) of any Terminated Product in all or part of the Affected Area; provided, however, that in the event that Amicus does so engage one or more other distributor(s) and/or licensee (s) of any Terminated Product in all or part of the Affected Area, GSK shall have no further obligation to continue to sell the Terminated Products in the Affected Area or such part thereof, as applicable. Any Terminated Product sold or disposed by GSK in the Affected Area during the Wind-down Period shall be subject to applicable royalty payment obligations under Section 3.4 above, and for such purposes, Sections 3.4, 3.5, 3.7, 3.8, 3.9 and 3.11 shall survive. Within \*\*\*\*\* following the expiration of the Wind-Down Period, GSK shall notify Amicus of any quantities of Compound or Terminated Product(s) remaining in GSK’s or its Affiliate’s inventory, as well as any components necessary for the Manufacture of the Compound and Terminated Product(s) in GSK’s or its Affiliate’s inventory, and Amicus shall have the option, upon notice to GSK, to repurchase any such quantities of the Compound and/or Terminated Product(s) and/or components from GSK at a price to be mutually agreed by the Parties. If Amicus so elects to purchase any remaining quantities of Compound or Terminated Products or components from GSK as set forth herein, GSK will transfer to Amicus such quantities of inventory of Compound or Terminated Product(s) or components.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

14.3.4 Data and Know-How. GSK will, at the request of Amicus, provide Amicus complete access to, and/or copies of, documentation pertaining to all preclinical and clinical data and all regulatory data, and all other Program Improvements, in each case as necessary for Amicus to Develop, Manufacture and Commercialize the Compound and the Terminated Product(s) as of the date of termination (including all Know-How pertaining to the Manufacture of the Compound and Terminated Products, including Know-How corresponding to that described in Schedule 6.5.1), that are Controlled by GSK or its Affiliates, and Amicus shall have, and is hereby granted by GSK, an irrevocable, non-exclusive, royalty-free right and license, with the right to sublicense, to use and disclose all such data and other Program Improvements following any such termination of this Agreement in accordance with the license granted to Amicus pursuant to Section 14.3.1 above. In addition, all such data and other Program Improvements generated by or under authority of GSK or its Affiliates hereunder during the term of the Agreement shall, to the extent specifically pertaining to a Terminated Product in the Affected Area (as well as, if any such termination applies to this Agreement in its entirety, such items to the extent specifically pertaining to the Compound), be deemed Confidential Information of Amicus to be used solely in connection with the Compound and/or Products and not Confidential Information of GSK (and will not be subject to the exclusions under Section 1.35 (a) and (d) above).

14.3.5 Regulatory Filings. At Amicus' option, which shall be exercised by written notice to GSK, GSK will assign and transfer (or cause to be assigned and transferred) to Amicus or its designee (or to the extent not so assignable, GSK shall take all reasonable action to make available to Amicus or its designee the benefits of) all regulatory submissions and filings and marketing approvals (including all INDs, MAAs and Marketing Approvals) related to the Compound or the Terminated Product(s) in the Affected Area, including such regulatory submissions and registrations made or owned by GSK's Affiliates and Sublicensees. In each case, unless otherwise required by any applicable Law, GSK shall use all reasonable efforts to make such foregoing assignment (or availability), within \*\*\*\*\* after the effective date of any such termination (or, with respect to any such regulatory filings pertaining to an Ongoing Trial that GSK is continuing to conduct pursuant to Section 14.3.2 above, within \*\*\*\*\* after the completion of such Ongoing Trial), provided, however, that in the event that GSK is unable to make such assignment or to make such regulatory filings available to Amicus within \*\*\*\*\* after the effective date of any such termination (or the completion of such Ongoing Trial, as applicable) due to factors beyond GSK's reasonable control, then GSK shall so notify Amicus and (including the reason for any such delay) prior to the expiration of such \*\*\*\*\* period and the Parties shall mutually agree (such agreement not to be unreasonably withheld by either Party) an appropriate extension to such \*\*\*\*\* period.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

14.3.6 Supply. If GSK is Manufacturing, itself or through a Third Party, Compound or any Terminated Product (each a “GSK Supplied Material”), upon request by Amicus, GSK will, or will cause such Third Party to, supply Amicus with its (and its Affiliates’ and licensees’) reasonable requirements of each GSK Supplied Material at GSK’s Manufacturing Costs therefor (provided that such Manufacturing Costs of GSK shall not differ substantially from GSK’s Manufacturing Costs for the applicable GSK Supplied Material in the year immediately prior to the Wind Down Period) and on customary terms with respect to quality, ordering and delivery, until Amicus, on a material-by-material basis, using Commercially Reasonable Efforts, is able, itself or through a Third Party, to Manufacture such GSK Supplied Material to meet such reasonable requirements for the Terminated Products in the Affected Area, but in no event shall GSK be obligated to supply Amicus with GSK Supplied Material for more than \*\*\*\*\* as provided in this Section 14.3.6. If GSK is Manufacturing the Terminated Products for the Affected Area through a Third Party, upon Amicus’ request, GSK shall use Commercially Reasonable Efforts to transition to Amicus its arrangement with such Third Party contractor.

14.3.7 Transition. Without limiting the foregoing, GSK shall use Commercially Reasonable Efforts to cooperate with Amicus and/or its designee to effect a smooth and orderly transition in the Development, sale and ongoing marketing, promotion and Commercialization of all Terminated Product(s) in the Affected Area following termination as set forth herein.

14.3.8 Sublicensees. Any contracts with Sublicensees of any Terminated Product in the Affected Area engaged by GSK, other than GSK’s Affiliates, shall be assigned to Amicus to the extent GSK has the right to do so and Amicus so requests. In the event such assignment is not requested by Amicus or GSK does not have the right to do so, then the rights of such Sublicensees with respect to the Terminated Product in the Affected Area shall terminate upon termination of GSK’s rights with respect to the Terminated Products in the Affected Area. GSK shall ensure that its Affiliates and such Sublicensees (if not assigned to Amicus pursuant to this Section 14.3.8) shall transition all Terminated Products in the Affected Area back to Amicus in the manner set forth in this Section 14.3 as if such Affiliate or Sublicensee were named herein.

14.3.9 Trademarks .

(a) Within \*\*\*\*\* after the end of the Wind-Down Period upon request by Amicus, GSK shall either return to Amicus or destroy all tangible items pertaining to a Terminated Product in each country of the Affected Area and comprising, bearing or containing any Amicus Trademark and/or the Amicus House Marks that is in GSK’s possession. Effective upon the end of the Wind-Down Period, GSK shall cease to use all Trademarks and trade names of Amicus (including the Amicus House Marks and all Amicus Trademarks) with respect to the Terminated Products in the Affected Area, and all rights granted to GSK hereunder to use such Trademarks and trade names of Amicus with respect to the Compound and Terminated Products in the Affected Area shall terminate. In addition, at Amicus’ option, which shall be exercised by written notice to GSK, and upon payment by Amicus of the out-of-pocket costs of assignments and of the out-of-pocket costs incurred to identify, design and register (including but not limited to clearance filing and maintaining) of any GSK Trademark selected by the JSC that had been used with each Terminated Product in the Affected Area (each, a “GSK Terminated Product Trademark”), GSK shall promptly assign to Amicus, \*\*\*\*\*, all rights of GSK and its Affiliates in and to such GSK Terminated Product Trademarks, including all registrations and applications for registration for such GSK Terminated Product Trademarks in the Affected Area and all associated goodwill. For the avoidance of doubt, the foregoing shall not include any GSK House Marks. Further, upon payment by Amicus of the out-of-pocket costs of assignment and registration, GSK shall transfer to Amicus all domain names established by GSK for use with a Terminated Product in any country of the Affected Area.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

(b) Subject to, and without limiting Amicus' rights under, Sections 14.3.1, 14.3.4 and 14.5, if GSK terminates this Agreement pursuant to Section 13.3, then within \*\*\*\*\* after the end of the Wind Down Period and upon the request of GSK, Amicus shall either return to GSK or destroy all tangible items comprising, bearing or containing any GSK Trademark (other than the GSK Terminated Product Trademarks) and/or the GSK House Marks that are in Amicus' possession. Effective upon the end of the Wind Down Period, Amicus shall cease to use all Trademarks and trade names of GSK (including the GSK House Marks and all GSK Trademarks, other than the GSK Terminated Product Trademarks) with respect to the Terminated Products in the Affected Area.

14.3.10 Termination solely with respect to a Product(s) or country(ies). Upon termination of this Agreement by GSK pursuant to Section 13.3 or by Amicus pursuant to Section 13.2 with respect to a Terminated Product(s) and/or the Affected Area only, the Parties' rights and obligations under the Agreement with respect to all other Products in the Territory shall survive, subject to the following provisions:

(a) Each country of the Affected Area shall cease to be a country within the Territory and the definition of "Territory" in Section 1.130 shall be deemed to be amended accordingly and all references to a Major Market shall be deemed to be references to a "Major Market within the Territory"; similarly, each Terminated Product shall cease to be a "Product" covered by this Agreement and the definition of "Product" in Section 1.105 shall be deemed to be amended accordingly;

(b) Notwithstanding any other provision of this Agreement, including the definition of "Amicus Know-How" in Section 1.19 and Sections 2.1, 5.1.7 and 5.2.3, Amicus shall not have any obligation to make available to GSK any data or other Know-How with respect to a Terminated Product generated by or on behalf of Amicus, its Affiliates and/or licensees for the Affected Area following any such termination and GSK shall have no rights to, and the License shall not include, any such data or Know-How; in addition, all of GSK's approval rights as set forth in the second sentence of Section 11.5 shall immediately terminate, provided, however, that nothing in this Section 14.3.10(b) shall otherwise modify GSK's rights or Amicus' obligations set forth in Section 11.5, including GSK's right of pre-publication review and Amicus' obligations to remove, at the request of GSK, any GSK Confidential Information from any such publication;

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

(c) Upon notice by one Party to the other Party, the Parties shall promptly meet and negotiate in good faith appropriate downward adjustments to the levels of Calendar Year Net Sales that trigger each milestone payment corresponding to a sales milestone that includes Net Sales in the Affected Area as set forth in Section 3.3.3 and to which each of the royalty rates applies as set forth in Section 3.4.1; provided that if the Parties are unable to agree on such adjustments within thirty (30) days following the date of any such notice from Amicus, then either Party may, upon written notice to the other, refer such dispute for resolution pursuant to the alternative dispute resolution as contemplated by Section 16.2.3 (with the Parties expressly acknowledging that any such modifications or revisions to the levels of Calendar Year Net Sales will not, with reference to any other provision of this Agreement, be construed as consequential or otherwise impermissible damages);

(d) As between the Parties, GSK shall continue to be responsible for pharmacovigilance and adverse event reporting within the Territory with respect to the Compound and Products as provided in Section 5.2.6 and Amicus shall be responsible for pharmacovigilance with respect to the Terminated Products in the Affected Area, and the Parties shall cooperate such that Amicus (or Amicus' designee) is able to maintain a worldwide safety database for the Terminated Products. The Parties shall promptly negotiate and implement any appropriate amendments to the pharmacovigilance agreement described in Section 5.2.6(a) in accordance with this Section 14.3.10(c); and

(e) The Parties rights and obligations under Section 7.1 shall survive; and GSK's rights and obligations under Article 7 regarding the prosecution and maintenance of all GSK Prosecuted Amicus Patents and Program Patents in the Affected Area to the extent pertaining to the Terminated Products shall terminate from and after the date of any such termination and all such Patents shall be deemed to be Amicus Prosecuted Patents; provided, however, that Amicus will, in a timely manner, solicit GSK's comments regarding the prosecution and maintenance of such Amicus Patents and Program Patents and review of the nature and text of any such Patent application and prosecution matters related thereto, including any correspondence between Amicus and any government intellectual property or Patent authorities, agencies or other government bodies, in reasonably sufficient time prior to filing thereof, and Amicus will give due consideration to GSK's reasonable comments and amendments.

#### 14.3.11 Non-Compete .

(a) Upon termination of this Agreement in its entirety by Amicus pursuant to Section 13.2 or by GSK pursuant to Section 13.3: \*\*\*\*\*.

(b) Upon termination of this Agreement by Amicus pursuant to Section 13.2 or by GSK pursuant to Section 13.3 with respect to particular Product(s) and/or country(ies) of the Territory: \*\*\*\*\*.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

14.4 Rights upon Termination for Bankruptcy. Notwithstanding the bankruptcy of Amicus, or the impairment of performance by Amicus of its obligations under this Agreement as a result of bankruptcy or insolvency of Amicus as described in Section 13.4, upon the termination of this Agreement by GSK pursuant to Section 13.4, GSK will be entitled to retain all rights and licenses granted to GSK by Amicus under this Agreement. All rights and licenses granted under or pursuant to this Agreement by Amicus to GSK are, and will otherwise be deemed to be, for purposes of Article 365(n) of the Bankruptcy Code, licenses of rights to “intellectual property” as defined under Article 101(52) of the Bankruptcy Code. The Parties agree that GSK, as a licensee of such rights under this Agreement, will retain and may fully exercise all of its rights and elections under the Bankruptcy Code. The Parties further agree that, in the event of the commencement of a bankruptcy proceeding by or against Amicus under the Bankruptcy Code, GSK will be entitled to a complete duplicate of (or complete access to, as appropriate) any such intellectual property and all embodiments of such intellectual property, and same, if not already in its possession, will be promptly delivered to GSK (i) upon any such commencement of a bankruptcy proceeding upon written request therefore by GSK, unless Amicus elects to continue to perform all of its obligations under this Agreement, or (ii) if not delivered under (i) above, upon the rejection of this Agreement by or on behalf of Amicus upon written request therefore by GSK. The provisions of this Section 14.4 shall apply *mutatis mutandis* to Amicus in the event of any bankruptcy or insolvency of GSK as described in Section 13.4.

14.5 Return of Materials. No later than thirty (30) days after the expiration or termination of this Agreement, each Party shall return or cause to be returned to the other Party (or, at such other Party’s request, destroy and certify such destruction) all Confidential Information received from the other Party and all copies thereof that are in such Party’s possession, as well as all biological or chemical materials delivered or provided by the other Party; provided, however, that each Party may retain one (1) copy of such Confidential Information received from the other Party for record purposes. Notwithstanding the foregoing, to the extent that a Party has a continuing license pursuant to Section 14.2 or Section 14.3 above, as applicable, after the termination of this Agreement, such Party may retain the Confidential Information of the other Party and use such Confidential Information solely to the extent necessary and for the purpose of the continued practice of such license and in such event, notwithstanding Section 11.1 above, such Party’s obligations under Article XI above, shall continue for so long as such Party continues to practice such license.

14.6 Survival. Upon the expiration or termination of this Agreement, all rights and obligations of the Parties under this Agreement shall terminate except those described in the following provisions (which such provisions shall survive for the term specified therein and, if no such term is specified, then indefinitely): Articles I, XIV, XV and XVI; Sections 3.9, 3.10, 11.1 and 11.3; and GSK’s rights under Section 11.4 (solely to the extent GSK’s clinical trial register includes the results of clinical trials for the Compound or Products prior to any such termination).

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

## **XV. INDEMNIFICATION AND LIMITATION OF LIABILITY**

15.1 Indemnification of Amicus. GSK shall indemnify and hold harmless each of Amicus, its Affiliates and the directors, officers, stockholders and employees of such entities and the successors and assigns of any of the foregoing (the “Amicus Indemnitees”), from and against any and all liabilities, damages, penalties, fines, costs, expenses (including, reasonable attorneys’ fees and other expenses of litigation) (“Liabilities”) from any claims, actions, suits or proceedings brought by a Third Party (a “Third Party Claim”) incurred by any Amicus Indemnitee, arising from, or occurring as a result of: (a) activities relating to the Development, use, marketing, distribution, importation or sale of any Compound and Product by GSK, its Affiliates, Sublicensees, or subcontractors in the Territory; (b) any material breach of any representations, warranties or covenants by GSK in Articles IX and X above; and/or (c) activities relating to the Manufacture (after the Supply Transition Date) of any Compound or Product by GSK, its Affiliates, Sublicensees, or subcontractors for distribution in the Territory; except to the extent such Third Party Claims fall within the scope of Amicus’s indemnification obligations set forth in Section 15.2 below or result from the gross negligence or intentional misconduct of a Amicus Indemnitee. For the avoidance of doubt, Product Liability Claims are not subject to this Section 15.1 and are governed by the provisions of Section 15.4 below.

15.2 Indemnification of GSK. Amicus shall indemnify and hold harmless each of GSK, its Affiliates and Sublicensees and the directors, officers and employees of GSK, its Affiliates and Sublicensees and the successors and assigns of any of the foregoing (the “GSK Indemnitees”), from and against any and all Liabilities from any Third Party Claims incurred by any GSK Indemnitee, arising from, or occurring as a result of (a) activities related to the Development, or use of any Compound and Product by Amicus, its Affiliates, Sublicensees or subcontractors in the Territory; (b) any material breach of any representations, warranties or covenants by Amicus in Article IX and X above; or (c) the Manufacture of any Compound or Product by Amicus, its Affiliates, Sublicensees, or subcontractors, prior to GSK’s assumption of responsibility for Manufacturing the Compound and Product pursuant to Section 6.5 or Section 14.2.2(b); except to the extent such Third Party Claims (i) fall within the scope of GSK’s indemnification obligations set forth in Section 15.1 above or (ii) result from the gross negligence or intentional misconduct of an GSK Indemnitee or (iii) with respect to clause (c) above, result from Amicus’ compliance with any direction of GSK pursuant to Section 6.5.2(a) above. For the avoidance of doubt, Product Liability Claims are not subject to this Section 15.2 and are governed by the provisions of Section 15.4 below.

15.3 Procedure. A Party that intends to claim indemnification under this Article XV (the “Indemnitee”) shall promptly notify the other Party (the “Indemnitor”) in writing of the assertion or the commencement of Third Party Claim and will provide the Indemnitor such information with respect thereto that the Indemnitor may reasonably request. The Indemnitor shall be entitled to control and appoint lead counsel for such defense, in each case at its expense. If the Indemnitor shall assume the control of the defense of any Third Party Claim in accordance with the provisions of this Section 15.3, the Indemnitor shall obtain the prior consent of the Indemnitee (which shall not be unreasonably withheld) before entering into any settlement of such Third Party Claim. The failure to deliver written notice to the Indemnitor within a reasonable time after the commencement of any action with respect to a Third Party Claim shall not relieve the Indemnitor of its obligations under this Article XV unless the delay or failure is prejudicial to its ability to defend such action. The Indemnitee under this Section 15.3 shall cooperate fully with the Indemnitor and its legal representatives in the investigation of any action with respect to a Third Party Claim covered by this indemnification.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

#### 15.4 Product Liability.

15.4.1 Each Party shall notify the other Party as promptly as practicable if any Third Party Claim is commenced or threatened against such Party in the Territory alleging product liability, product defect, design, packaging or labeling defect, failure to warn or any similar action relating to the use or safety of Compound and Products in the Territory (a "Product Liability Claim"). For clarity, a Product Liability Claim will not be deemed to include any Third Party Claims relating to a Manufacturing defect of Compound and Products and Sections 15.1 and 15.2 shall apply to any such Third Party Claims.

15.4.2 To the extent that either the GSK Indemnitees or the Amicus Indemnitees incur, suffer, or are faced with any Product Liability Claims with respect to the Product in the Territory, then \*\*\*\*\*.

15.4.3 GSK shall have the right to control and appoint lead counsel for the defense of any such Product Liability Claims and to settle any such Product Liability Claims, in its discretion, provided, that GSK shall reasonably consult with and consider the input of Amicus with respect to such matters.

15.5 Insurance. In addition to its duty to indemnify, each Party will procure product liability insurance in commercially reasonable amounts in view of its activities. Alternatively, either Party may establish a program of self insurance for the same risks. In either event, as reasonably requested in writing by the other Party not more than once every twelve (12) months, each Party will supply the other Party with evidence of such coverage during the time any Product is being Developed, Manufactured or Commercialized by such Party or any of its Affiliates, Sublicensees, designees or agents.

15.6 Disclaimer of Consequential Damages. IN NO EVENT WILL EITHER AMICUS OR GSK BE LIABLE TO THE OTHER FOR ANY SPECIAL, INDIRECT, CONSEQUENTIAL, INCIDENTAL, OR PUNITIVE DAMAGES ARISING UNDER OR AS A RESULT OF THIS AGREEMENT (OR THE TERMINATION HEREOF) INCLUDING, BUT NOT LIMITED TO, THE LOSS OF PROSPECTIVE PROFITS OR ANTICIPATED SALES.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

## **XVI. MISCELLANEOUS**

16.1 Governing Law . For all matters other than the scope and validity of Patents, this Agreement shall be deemed to have been made in the State of Delaware and its form, execution, validity, construction and effect shall be determined in accordance with the laws of the State of Delaware, without giving effect to the principles of conflicts of law thereof and the Parties agree to the personal jurisdiction of and venue in any federal or state court located in Delaware. The application of the United Nations Convention for Contracts for the International Sales of Goods is hereby expressly excluded.

### 16.2 Dispute Resolution .

16.2.1 The Parties agree that with respect to any disputes arising with respect to the interpretation, breach, enforcement, termination or validity of this Agreement (for the purposes of this Section 16.2, each a “Dispute”), the Dispute shall first be presented to the Chief Executive Officer of Amicus and the GSK Chairman of Research and Development, or their respective designees for resolution. If the Amicus Chief Executive Officer and GSK Chairman or Research and Development, or their respective designees, cannot resolve the Dispute within \*\*\*\*\* of the request to do so, either Party may initiate arbitration proceedings with respect thereto as provided in Section 16.2.2 below. Prior to the establishment of an arbitration tribunal, Amicus and GSK shall each have the right to apply to any court of competent jurisdiction for appropriate interim or provisional relief, as necessary to protect the rights or property of that Party.

16.2.2 Any Dispute shall be finally resolved by arbitration in accordance with the Commercial Arbitration Rules of the American Arbitration Association (“AAA”) then in effect (the “Rules”), except as modified herein. The place of arbitration shall be Wilmington, Delaware. If the amount in controversy \*\*\*\*\* , there shall be one (1) neutral and impartial arbitrator who shall be agreed upon by the Parties within twenty (20) days of receipt by respondent of a copy of the demand for arbitration. If the amount in controversy \*\*\*\*\* , there shall be three (3) arbitrators, of whom each Party shall appoint one (1) within thirty (30) days of the receipt by the respondent of the demand for arbitration. The two (2) arbitrators so appointed shall select a third (3<sup>rd</sup>) arbitrator as the chair of the arbitral tribunal within thirty (30) days of the appointment of the second arbitrator. If any arbitrator is not appointed within the time limit provided herein, such arbitrator shall be appointed by the AAA in accordance with the listing, striking, and ranking procedures in the Rules. Any arbitrator appointed by the AAA shall be an attorney with no less than fifteen (15) years of experience with commercial cases and an experienced arbitrator, who shall, if practicable, have substantial experience with transactions or disputes related to the field of pharmaceutical products and/or, if applicable, intellectual property.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

16.2.3 In the case of any Dispute arising under Section 1.93(f)(v) or Section 14.3.10(c) or under Schedule 5.1.5, the procedures of this Section 16.2.3 shall apply. Arbitration with respect to all such Disputes under Section 1.93(f) (v) or Section 14.3.10 (c) or under Schedule 5.1.5, as applicable, shall be a “baseball” type arbitration, meaning that, following all permitted discovery and in accordance with procedures otherwise determined by the arbitrator, each Party shall prepare and submit to the arbitrator and the other Party a written report setting forth its final position with respect to the substance of the dispute, and each party may submit a revised report and position within 15 (fifteen) days of receiving the other party’s report. The arbitrator shall then select one of the Party’s positions as his or her final decision and shall not have authority to render any substantive decision other than to so select the position of either GSK or Amicus. The Parties and the arbitrator shall use all reasonable efforts to complete any such arbitration with respect to a Dispute arising under Section 1.93(f)(v) or Section 14.3.10(c) or under Schedule 5.1.5, as applicable, within ninety (90) days.

16.2.4 The arbitral tribunal is not empowered to award damages in excess of compensatory damages, and each Party hereby irrevocably waives any right to recover punitive, exemplary, multiple or similar damages with respect to any Dispute. Any arbitration proceedings, decision, or award rendered hereunder and the validity, effect, and interpretation of this arbitration provision shall be governed by the Federal Arbitration Act, 9 U.S.C. §1 et seq. The decision of the arbitral tribunal shall be in writing and, if applicable, shall state the findings of fact and conclusions of law on which it is based. The decision of the arbitral tribunal shall be final and binding upon the Parties regarding the applicable Dispute presented to the arbitral tribunal. Judgment upon the decision of the arbitral tribunal may be entered in any court having jurisdiction. The arbitration proceedings and the decision of the arbitral tribunal shall not be made public without the joint consent of the Parties and each Party shall maintain the confidentiality of such proceedings and decision unless each Party otherwise agrees in writing; provided that either Party may make such disclosures as are permitted for Confidential Information of the other Party under Article XI above. The Parties agree that they shall share equally the cost of the arbitration filing and hearing fees, and the cost of the arbitral tribunal and administrative fees of the AAA. Each Party shall bear its own costs and attorneys’ and witnesses’ fees and associated costs and expenses. The arbitral tribunal shall have full authority to grant provisional remedies and to direct the Parties to request that any court modify or vacate any temporary or preliminary relief issued by such court.

16.2.5 The Parties hereby submit to the exclusive jurisdiction of the federal and state courts located in Delaware for the purpose of an order to compel arbitration, for preliminary relief in aid of arbitration, or for a preliminary injunction to maintain the status quo or prevent irreparable harm prior to the appointment of the arbitrators, and to the non-exclusive jurisdiction of such courts for the enforcement of any award issued hereunder. The Parties hereby agree to accept service of process pursuant to the notice provisions of this Agreement.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

### 16.3 Assignment and Binding Effect.

16.3.1 This Agreement may not be assigned, by operation of law or otherwise, by either Party without the prior written consent of the other, except as otherwise permitted under this Section 16.3:

(a) Amicus may assign this Agreement to an Affiliate or to a Third Party without such prior written consent as part of a merger, consolidation, sale, or transfer of all or substantially all its assets, but only if the assignee has or simultaneously acquires all of the necessary rights and other assets to perform Amicus's obligations under this Agreement. A Change of Control or ownership of Amicus by merger or otherwise will not constitute an impermissible assignment of this Agreement by Amicus, provided, however, that such Change of Control event shall be subject to the terms of Article XII herein.

(b) GSK may assign this Agreement to any Affiliate without the prior written consent of Amicus. GSK may also assign this Agreement to a Third Party as part of a merger, consolidation, sale, or transfer of all or substantially all its assets, without the prior written consent of Amicus, but only if the assignee has or simultaneously acquires all of the necessary rights and other assets to perform GSK's obligations under this Agreement.

16.3.2 No assignment under this Section 16.3 shall be effective unless the intended assignee executes and delivers to the Party which is not the assignor a writing whereby the assignee expressly undertakes to perform and comply with all of its assignor's obligations hereunder. Notwithstanding such undertaking, such assignor shall continue to be primarily liable for such assignee's performance hereof and compliance herewith.

16.3.3 Any assignment in violation of this Section 16.3 shall be void and of no effect.

16.3.4 This Agreement, and the rights and obligations of the Parties herein contained, shall be binding upon, and shall inure to the benefit of, the Parties and their respective legal representatives, successors and permitted assigns.

16.4 Independent Contractor Status. The relationship of the Parties is that of independent contractors. Nothing in this Agreement will be construed to constitute, create, give effect or otherwise imply a joint venture, agency, partnership or other formal business organization or any employer/employee relationship of any kind between the Parties.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.



The address of either Party set forth above may be changed from time to time by written notice in the manner prescribed herein from the Party requesting the change.

16.6 Further Assurances. The Parties will execute and deliver any further or additional instruments or documents and perform any acts which may be reasonably necessary in order to effectuate and carry out the purposes of this Agreement.

16.7 Waivers. The waiver by either Party of a default or a breach of any provision of this Agreement by the other Party will not operate or be construed to operate as a waiver of any subsequent default or breach. The continued performance by either Party with knowledge of the existence of a default or breach will not operate or be construed to operate as a waiver of any default or breach. Any waiver by a Party of a particular provision or right will be in writing, will be as to a particular matter and, if applicable, for a particular period of time and will be signed by such Party.

16.8 Entire Agreement. This Agreement (including the Exhibits and Schedules hereto), the Equity Agreement, any and all Trademark License Agreements and the pharmacovigilance agreement described in Section 5.2.6(a) (in each case, if and when executed by the Parties) constitute the entire agreement between the Parties with respect to the subject matter hereof, and supersede all prior agreements and negotiations with respect to such subject matter.

16.9 Severability. If any provision in this Agreement is deemed to be, or becomes, invalid, illegal, void or unenforceable under applicable Laws, then: (i) it will be deleted with respect to the applicable jurisdiction(s) to which such Law pertains and the validity, legality and enforceability of the remaining provisions of this Agreement shall not be impaired or affected in any way, and (ii) the Parties will use Commercially Reasonable Efforts to substitute for the invalid, illegal or unenforceable provision a valid, legal and enforceable provision which conforms as nearly as possible with the original intent of the Parties. \*\*\*\*\*. Any termination in accordance with the foregoing shall be deemed a termination of this Agreement in its entirety pursuant to Section 13.3 if the Party who made the assertion was GSK, and shall be deemed a termination of this Agreement in its entirety under Section 13.2 by reason of a breach by Amicus, if Amicus is the Party who made such assertion.

16.10 Counterparts. This Agreement may be executed in more than one counterpart, each of which shall be deemed to be an original but all of which taken together shall be deemed a single instrument. A facsimile transmission of the signed Agreement will be legal and binding on both Parties.

16.11 Force Majeure. Neither Party to this Agreement will be liable for failure or delay in the performance of any of its obligations hereunder (other than the failure to pay monies owed), if such failure or delay is due to acts of God, earthquakes, fires, strikes, acts of war (whether declared or not), terrorism, civil unrest, or intervention of any governmental authority or any other event or occurrence beyond the reasonable control of such Party (a “Force Majeure Event”), but any such delay or failure will be remedied by such Party as soon as practicable after the removal of the cause of such failure or delay. Upon the occurrence of Force Majeure Event, the Party failing or delaying performance will promptly notify the other Party in writing, setting forth the nature of the occurrence, its expected duration and how such Party’s performance is affected, and the Party failing or delaying performance will use its Commercially Reasonable Efforts to avoid or remove the causes of non-performance and shall continue performance with the utmost dispatch whenever such causes are removed.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

16.12 Interest on Late Payments . If any Party fails to pay in full on or before the date due any royalty, fee or other amount that is required to be paid to the other Party under this Agreement, the paying Party will also pay to the other Party (or its designee) interest at a rate equal to: (i) the prime rate as reported by Citibank N.A., plus \*\*\*\*\*; or (ii) if lower, the maximum rate permitted by law; calculated on the number of days such payment is delinquent, compounded annually and computed on the basis of a three hundred sixty five (365) day year.

16.13 Cumulative Remedies . Unless otherwise set forth in this Agreement, all rights and remedies of the Parties, including all rights to payment, rights of termination, rights to injunctive relief, and other rights provided under this Agreement, shall be cumulative and in addition to all other remedies provided for in this Agreement, in law, and in equity.

16.14 Amendment . This Agreement may not be amended, supplemented or otherwise modified except by an instrument in writing signed by both Parties that specifically refers to this Agreement.

16.15 Headings and References . All section headings contained in this Agreement are for convenience of reference only and will not affect the meaning or interpretation of this Agreement.

16.16 No Strict Construction . This Agreement has been prepared jointly and will not be strictly construed against either Party.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

IN WITNESS WHEREOF, the Parties hereto, intending to be legally bound hereby, have caused this License and Collaboration Agreement to be executed by their duly authorized representatives as of the date first written above.

**AMICUS THERAPEUTICS, INC.**

**GLAXO GROUP LIMITED**

By: /s/ John F. Crowley

By: /s/ Paul Williamson

Name: John F. Crowley  
Title: Chairman and CEO

Name: Paul Williamson  
Title: Corporate Director

\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

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**EXHIBIT A**

**EQUITY AGREEMENT**

(See Exhibit 10.31 to Annual Report on Form 10-K filed on March 4, 2011)

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## **EXHIBIT B**

### **INITIAL PRESS RELEASE**

#### **GSK and Amicus Therapeutics Enter Exclusive Worldwide Agreement to Develop and Commercialize Amigal™ for Fabry Disease**

**- Amicus to receive \$60M in upfront license payment and equity investment and eligible for approximately \$170M million in future potential milestone payments-**

LONDON and CRANBURY, N.J., Oct 29, 2010 /PRNewswire via COMTEX News Network/ — GlaxoSmithKline PLC (GSK) and Amicus Therapeutics (Nasdaq: FOLD) today announced a definitive agreement to develop and commercialize Amigal™ (migalastat HCl), currently in Phase 3 for the treatment of Fabry disease, a rare inherited disorder. Under the terms of the agreement, GSK will receive an exclusive worldwide license to develop, manufacture and commercialize migalastat HCl. Additionally, as part of the agreement GSK and Amicus also intend to advance clinical studies exploring the co-administration of migalastat HCl with enzyme replacement therapy (ERT) for the treatment of Fabry disease.

Under the terms of the Agreement, Amicus will receive an upfront, license payment of \$30M from GSK and is eligible to receive further payments of approximately \$170M upon the successful achievement of development and commercialization milestones, as well as tiered double-digit royalties on global sales of migalastat HCl. GSK and Amicus will jointly fund development costs in accordance with an agreed upon development plan. Additionally, as part of the collaboration, GSK is purchasing 6.9 million shares of Amicus common stock at a price of \$4.56 per share. The total value of this equity investment to Amicus is \$31 million and represents a 19.9% ownership position for GSK in the Company. The total cash up-front to Amicus from GSK for the upfront license payment and equity investment is approximately \$60 million.

“This strategic collaboration is another significant milestone in delivering our vision for GSK Rare Diseases. Amicus’ scientific and clinical expertise in human genetic diseases is among the best in the industry, and we are pleased to be collaborators and investors in this exceptional company,” said Marc Dunoyer, Global Head of GSK Rare Diseases and a member of the GSK Corporate Executive Team. “Our focus now is to continue to advance Amigal for Fabry disease and it is our hope to deliver a first-in-class, oral medicine to the thousands of people worldwide living with this devastating rare disease.”

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics said, “The completion of this agreement with GSK is a transformational event for Amicus. It provides a strong validation of the potential for Amigal to become an important new treatment option for people living with Fabry disease and for our pharmacological chaperone technology broadly. GSK has extremely impressive global clinical, regulatory and commercial expertise and a strong commitment to the development of treatments for rare diseases. We look forward to working in close partnership with them.” Crowley continued, “With this key strategic alliance with GSK and the added financial strength it provides, Amicus is now uniquely positioned to build shareholder value through our expertise in rare disease drug development.”

#### **About Amigal™ (migalastat HCl) for the Treatment of Fabry Disease**

Migalastat HCl is an investigational treatment for Fabry disease and has the potential to be the first in a new class of oral, small molecule medicines called pharmacological chaperones. It is designed to selectively bind to and stabilize the target enzyme alpha-galactosidase A (alpha-Gal A), which facilitates proper trafficking of the enzyme to the lysosomes, where it is needed to break down the target substrate globotriaosylceramide (GL-3).

Results from Phase 2 studies of migalastat HCl, which has orphan designation in both the US and EU, demonstrated that in subjects identified as responders to migalastat HCl treatment resulted in increased levels of alpha-Gal A, reduced levels of GL-3 as measured in renal interstitial capillary cells from kidney biopsies and in urine, and a potential positive impact on renal function. Treatment with migalastat HCl has been generally well-tolerated, with no drug-related serious adverse events. The most common adverse events were headache, arthralgia and diarrhea.

A Phase 3 study (Study 011) commenced in the second quarter of 2009 and treatment of the first patient began in the fourth quarter of 2009. This ongoing study is a 6-month, randomized, double-blind trial comparing migalastat HCl to placebo in 60 subjects in approximately 40 investigational sites worldwide. The surrogate primary endpoint is the change in the amount of kidney interstitial capillary GL-3. Subjects being enrolled are Fabry patients who have never received enzyme replacement therapy (ERT), or who have not received ERT for at least 6 months, and who have a mutation responsive to migalastat HCl.

GSK and Amicus today provided an update to the enrollment timeline for Study 011. Enrollment is now expected to be completed in the first quarter of 2011 and preliminary results are expected to be announced in the second half of 2011.

Furthermore, a separate Phase 3 study (Study 012) is expected to commence before year end. The study will be an 18-month, randomized, open-label study comparing migalastat HCl to ERT in approximately 60 subjects. The primary outcome of efficacy will be renal function as measured by glomerular filtration rate (GFR).

#### **About Fabry Disease**

Fabry disease is an inherited lysosomal storage disorder caused by deficiency of an enzyme called alpha-galactosidase A (alpha-Gal A). The role of alpha-Gal A within the body is to break down a complex lipid called globotriaosylceramide (GL-3). Reduced or absent levels of alpha-Gal A activity leads to the accumulation of GL-3 in the affected tissues, including the central nervous system, heart, kidneys, and skin. This accumulation of GL-3 is believed to cause the various symptoms of Fabry disease, including pain, kidney failure, and increased risk of heart attack and stroke.

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It is currently estimated that Fabry disease affects approximately 5,000 to 10,000 people worldwide.

**GlaxoSmithKline** - one of the world's leading research-based pharmaceutical and healthcare companies - is committed to improving the quality of human life by enabling people to do more, feel better and live longer. For further information please visit [www.gsk.com](http://www.gsk.com)

### **Amicus Therapeutics**

Amicus Therapeutics is a biopharmaceutical company focused on developing treatments for rare diseases. The Company is developing orally-administered, small molecule drugs called pharmacological chaperones, a novel, first-in-class approach to treating a broad range of diseases including lysosomal storage disorders and CNS diseases. Amicus' lead program is in Phase 3 for the treatment of Fabry disease. For further information, please visit [www.amicustherapeutics.com](http://www.amicustherapeutics.com).

#### Amicus Enquiries:

Analyst/Investor and Media enquiries: Jenene Thomas (609) 662-5084

#### GlaxoSmithKline Enquiries:

UK Media enquiries: David Mawdsley (020) 8047 5502  
Claire Brough (020) 8047 5502  
Stephen Rea (020) 8047 5502  
Alexandra Harrison (020) 8047 5502  
Jo Revill (020) 8047 5502

#### US Media enquiries:

Nancy Pekarek (919) 483 2839  
Mary Anne Rhyne (919) 483 2839  
Kevin Colgan (919) 483 2839  
Sarah Alspach (919) 483 2839

#### European Analyst/Investor enquiries:

Sally Ferguson (020) 8047 5543  
Gary Davies (020) 8047 5503

#### US Analyst/ Investor enquiries:

Tom Curry (215) 751 5419  
Jen Hill Baxter (215) 751 7002

### **Amicus Forward-Looking Statements**

This press release contains, and the accompanying conference call will contain, "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995 relating to preclinical and clinical development of Amicus' candidate drug products, the timing and reporting of results from preclinical studies and clinical trials evaluating Amicus' candidate drug products and the projected cash position for the Company, including achievement of development and commercialization milestone payments and sales royalties under our collaboration with GlaxoSmithKline. Words such as, but not limited to, "look forward to," "believe," "expect," "anticipate," "estimate," "intend," "plan," "targets," "likely," "will," "would," "should" and "could," and similar expressions or words identify forward-looking statements. Such forward-looking statements are based upon current expectations that involve risks, changes in circumstances, assumptions and uncertainties. The inclusion of forward-looking statements should not be regarded as a representation by Amicus that any of its plans will be achieved. Any or all of the forward-looking statements in this press release may turn out to be wrong. They can be affected by inaccurate assumptions Amicus might make or by known or unknown risks and uncertainties. For example, with respect to statements regarding the goals, progress, timing and outcomes of discussions with regulatory authorities and the potential goals, progress, timing and results of preclinical studies and clinical trials, actual results may differ materially from those set forth in this release due to the risks and uncertainties inherent in the business of Amicus, including, without limitation: the potential that results of clinical or pre-clinical studies indicate that the product candidates are unsafe or ineffective; the potential that it may be difficult to enroll patients in our clinical trials; the potential that regulatory authorities may not grant or may delay approval for our product candidates; the potential that preclinical and clinical studies could be delayed because we identify serious side effects or other safety issues; the potential that we will need additional funding to complete all of our studies and, our dependence on third parties in the conduct of our clinical studies. Further, the results of earlier preclinical studies and/or clinical trials may not be predictive of future results. With respect to statements regarding projections of the Company's cash position, actual results may differ based on market factors and the Company's ability to execute its operational and budget plans, including achievement of development and commercialization milestone payments and sales royalties under our collaboration with GlaxoSmithKline. In addition, all forward looking statements are subject to other risks detailed in our Annual Report on Form 10-K for the year ended December 31, 2009. You are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date hereof. All forward-looking statements are qualified in their entirety by this cautionary statement, and Amicus undertakes no obligation to revise or update this news release to reflect events or circumstances after the date hereof. This caution is made under the safe harbor provisions of Section 21E of the Private Securities Litigation Reform Act of 1995.

### **GSK's cautionary statement regarding forward-looking statements**

Under the safe harbor provisions of the U.S. Private Securities Litigation Reform Act of 1995, GSK cautions investors that any forward-looking statements or projections made by GSK, including those made in this announcement, are subject to risks and uncertainties that may cause actual results to differ materially from those projected. Factors that may affect GSK's operations are described under 'Risk Factors' in the 'Business Review' in the company's Annual Report on Form 20-F for 2009.

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## EXHIBIT C

### **TRADEMARK LICENSE AGREEMENT**

THIS TRADEMARK LICENSE AGREEMENT (“Agreement”) is made as of the \_\_\_\_\_ day of \_\_\_\_\_, 2010 (the “Effective Date”) by and between Amicus Therapeutics, Inc., a Delaware corporation having a place of business at 6 Cedar Brook Drive, Cranbury, New Jersey, 08512 (“Licensor”), as licensor, and Glaxo Group Limited, a company organized under the laws of England and Wales with its registered office at Glaxo Wellcome House, Berkeley Avenue, Greenford, Middlesex, UB6 0NN, England (“Licensee”), as licensee. Licensee and Licensor are sometimes collectively referred to herein as the “Parties” and separately as a “Party.”

WHEREAS, pursuant to that certain License and Collaboration Agreement by and between Licensee and Licensor, dated as of the \_\_\_\_\_ day of October, 2010 (the “License and Collaboration Agreement”), Licensor agreed to license to Licensee certain trademarks in the Territory (as defined in the License and Collaboration Agreement) as set forth on Exhibit A attached hereto, including all common law rights to such trademarks in the Territory (the “Licensed Marks”);

WHEREAS, Licensor is willing to grant, and Licensee is willing to receive, a license to use the Licensed Marks in connection with Compound and Products (as those terms are defined in the License and Collaboration Agreement) in the Territory pursuant to the terms and conditions set forth in this Agreement.

NOW, THEREFORE, in consideration of the mutual covenants, agreements and stipulations set forth herein and in the License and Collaboration Agreement, the receipt and legal sufficiency of which are hereby mutually acknowledged, Licensor and Licensee hereby agree as follows:

1. DEFINITIONS. Capitalized terms not otherwise defined in this Agreement shall have the meanings set forth in the License and Collaboration Agreement.
  2. GRANT OF LICENSE. During the Term of this Agreement, and pursuant to the terms and conditions contained herein, Licensor hereby grants to Licensee and its Affiliates, and Licensee and its Affiliates hereby accept, a non-exclusive, royalty-free, sublicensable (subject to Section 8 hereof) license to use the Licensed Marks in the Territory in connection with the making, having made, use, sale, offering for sale, importation, packaging, distributing and promoting of Product in the Field and in the country or countries of the Territory. In addition, Licensee shall have the right to use the Licensed Marks (excluding Amicus House Marks) as part of a domain name, subject to Licensor’s prior written approval and provided that Licensee remains responsible for all costs associated with development and operation of the associated website.
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### 3. USE OF THE LICENSED MARKS

- 3.1. Upon reasonable advance written request, Licensee agrees to submit to Licensor representative packaging for the Product displaying the Licensed Marks for Licensor's inspection. If Licensor reasonably determines that Licensee has failed to maintain a level of quality consistent with those normally employed by the Licensor in the use of the Licensor's Trademarks, then Licensor may request that Licensee take reasonable steps to remedy any such deficiencies and Licensee agrees to take commercially reasonable actions to comply with such requests, and in any event the Licensee shall not use or distribute any packaging for the Product displaying the Licensed Marks until it has complied with such requests.
- 3.2. Licensee and its Affiliates and Sub-licensees shall comply with all applicable Laws pertaining to the Commercialization of Products bearing the Licensed Marks.

### 4. MAINTENANCE OF LICENSED MARKS .

- 4.1. Licensor shall prosecute and maintain trademark applications and registrations existing as of the Effective Date for the Licensed Marks used on or in connection with Product in the Territory. All costs and expenses (including but not limited to attorneys' fees and expenses and official fees) of prosecuting and maintaining applications and registrations existing as of the Effective Date for the Licensed Marks shall be borne by Licensor. For the avoidance of doubt, Licensee shall prepare, file, prosecute, maintain and own any Trademarks (other than Amicus House Marks) that are created or designated by the JSC for use on Product in the Territory after the Effective Date.
  - 4.2. Licensor shall not (i) abandon any rights in the Licensed Marks in the Territory, (ii) abandon or allow to lapse any pending application for the Licensed Marks in the Territory, or (iii) permit any active registration for the Licensed Marks to lapse, expire or be cancelled in the Territory, without first notifying Licensee.
5. TERM. This Agreement shall be effective commencing on the Effective Date and shall continue perpetually unless terminated as set forth in Section 6 below.

### 6. TERMINATION .

- 6.1. This Agreement shall terminate automatically, without notice or any further action hereunder by either Party: (a) in its entirety upon the expiration or termination of the License and Collaboration Agreement by a Party in its entirety by Amicus pursuant to Section 13.2, by GSK pursuant to Section 13.3, or by either Party pursuant to Section 13.4; or (b) with respect to a particular Licensed Mark: (i) upon assignment of such Licensed Mark (other than an Amicus House Mark) to Licensee if the License and Collaboration Agreement is terminated by GSK pursuant to Section 13.2 with respect to the Product with which such Licensed Mark is used in the country(ies) of the Territory in which such Licensed Mark is registered or in use by a Party; or (ii) upon termination of the License and Collaboration Agreement by GSK pursuant to Section 13.3, or by Amicus pursuant to Section 13.2, of the License and Collaboration Agreement with respect to the Product with which such Licensed Mark is used and/or in the country(ies) of the Territory in which such Licensed Mark is registered or in use by a Party.
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- 6.2. The Parties may terminate this Agreement in its entirety or on a Licensed Mark-by-Licensed Mark basis at any time and for any reason during the Term upon their mutual written agreement; provided that the Parties shall agree to terminate this Agreement, in its entirety, or on a Licensed Mark-by-Licensed Mark or country-by-country basis, as applicable, if the JSC determines in accordance with Section 2.4, 4.3.1 and 6.3 of the License and Collaboration Agreement that such Licensed Mark(s) shall no longer be used with respect to a Product(s) in a particular country(ies) of the Territory.
- 6.3. Subject to and in accordance with Section 14.3 of the License and Collaboration Agreement, upon termination or expiration of this Agreement, Licensee agrees (i) immediately to discontinue, and to cause all of Licensee's Affiliates and any Sub-licensees of Licensee thereof immediately to discontinue, use of the Licensed Marks; (ii) upon Licensor's request, to return to Licensor or destroy all tangible embodiments of any Licensed Marks; and (iii) if such items or materials are destroyed by Licensee at Licensor's request, to furnish Licensor with certification and evidence of such destruction.

7. OWNERSHIP. Licensor represents and warrants, and Licensee acknowledges, that the Licensed Marks are the sole and exclusive property of Licensor or its Affiliates and all goodwill accrued through use of the Licensed Marks shall be deemed to be the absolute property of Licensor or its Affiliates. Licensee further acknowledges that nothing in this Agreement confers upon Licensee any right of ownership in and to the Licensed Marks. Licensee agrees to reasonably cooperate with Licensor to execute, deliver, and otherwise provide to Licensor all information and documents reasonably requested for the purpose of establishing, registering, evidencing or defending Licensor's complete and exclusive ownership of all rights, titles, and interests of every kind and nature whatsoever in and to the Licensed Marks. Licensee agrees not to register, use or authorize the use of any trademark or designation confusingly similar to the Licensed Marks, and Licensee agrees not to challenge Licensor's or its Affiliates' ownership of the Licensed Marks.

8. SUB-LICENCES. Licensee shall have the right to grant sublicenses under this Agreement solely as and to the extent Licensee is permitted to grant Sublicenses under and in accordance with Section 2.2 of the License and Collaboration Agreement. In any event, Licensee shall ensure that each of its sublicensees is bound by a written agreement containing provisions at least as protective of the Licensed Marks and Licensor as this Agreement; and Licensee shall remain responsible to Licensor for all activities of its Affiliates and sublicensees to the same extent as if such activities had been undertaken by Licensee itself. Promptly following the execution of each sublicense, Licensee shall provide Licensor with a complete copy of such sublicense.

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9. ASSIGNMENTS. Neither this Agreement, nor any of the rights or obligations of a Party may be directly or indirectly assigned, sold, delegated or otherwise disposed except by a Party in connection with an assignment of, and to the same assignee as, the License and Collaboration Agreement in accordance with Section 16.3 of the License and Collaboration Agreement.

9.1. No assignment under this Section 9 shall be effective unless the intended assignee executes and delivers to the Party which is not the assignor a writing whereby the assignee expressly undertakes to perform and comply with all of its assignor's obligations hereunder. Notwithstanding such undertaking, such assignor shall continue to be primarily liable for such assignee's performance hereof and compliance herewith.

9.2. Any assignment in violation of this Section 9 shall be void and of no effect.

9.3. This Agreement, and the rights and obligations of the Parties herein contained, shall be binding upon, and shall inure to the benefit of, the Parties and their respective legal representatives, successors and permitted assigns.

10. AMENDMENTS. No waiver, amendment or modification of any provision hereof or of any right or remedy hereunder shall be effective unless in writing and signed by the Party against whom such waiver, amendment or modification is sought to be enforced.

11. COUNTERPARTS. This Agreement may be executed in any number of counterparts, each of which shall be deemed to be an original, but all of which together shall constitute but one and the same instrument. Delivery of an executed counterpart signature page of this Agreement by facsimile transmission shall be as effective as delivery of a manually executed signature page.

12. APPLICABLE LAW AND DISPUTE RESOLUTION. This Agreement shall be governed by, interpreted and construed, and all claims and disputes, whether in tort, contract or otherwise be resolved in accordance with the substantive laws of the State of Delaware without reference to any rules of conflict of laws. Any and all disputes under this Agreement shall be resolved in accordance with Section 16.2 of the License and Collaboration Agreement.

13. FURTHER ASSURANCES. Each of Party shall, at any time or from time to time after the Effective Date, at the request and expense of the other Party, execute and deliver to the other Party all such instruments and documents or further assurances as the other Party may reasonably request in order to give effect to the transactions contemplated by this Agreement, including but not limited to Licensee's request for Licensor's cooperation to record or register this Agreement with any applicable governmental entity.

14. REPRESENTATIONS AND WARRANTIES. Each Party represents and warrants that (i) this Agreement has been duly and validly executed and delivered by such Party and constitutes a legal and binding obligation of such Party, enforceable against it in accordance with its terms, and (ii) it has all necessary right, power and authority to execute and perform its obligations under this Agreement and to grant the rights granted herein. Licensor further represents and warrants that it is the owner of all right, title, and interest in and to the Licensed Marks and that the execution, delivery, and performance of its obligations under this Agreement will not conflict with or violate any agreement or other obligation of Licensor or binding upon Licensor's assets, including but not limited to the Licensed Marks.

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15. SEVERABILITY. If any provision of this Agreement is held to be illegal, invalid or unenforceable under any present or future law, and if the rights or obligations of either Party under this Agreement will not be materially and adversely affected thereby, (a) such provision will be fully severable, (b) this Agreement will be construed and enforced as if such illegal, invalid or unenforceable provision had never comprised a part hereof, (c) the remaining provisions of this Agreement will remain in full force and effect and will not be affected by the illegal, invalid or unenforceable provision or by its severance herefrom, and (d) in lieu of such illegal, invalid or unenforceable provision, there will be added automatically as a part of this Agreement a legal, valid and enforceable provision as similar to such illegal, invalid or unenforceable provision as may be possible and reasonably acceptable to the Parties.

16. WAIVER. No waiver by any Party in one or more instances of any of the provisions of this Agreement or the breach thereof shall establish a precedent for any other instance with respect to that or any other provision. Furthermore, in case of waiver of a particular provision, all other provisions of this Agreement will continue in full force and effect.

17. INTEGRATION. This Agreement (including Exhibits hereto), and the License and Collaboration Agreement, embodies the entire agreement of the Parties hereto with respect to the subject matter hereof and supersedes any and all prior agreements with respect thereto.

*[Remainder of page intentionally left blank]*

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IN WITNESS WHEREOF, the Parties hereto have caused this Agreement to be executed as of the Effective Date by their duly authorized representatives.

AMICUS THERAPEUTICS, INC.

By: \_\_\_\_\_

Name:

Title:

GLAXO GROUP LIMITED

By: \_\_\_\_\_

Name:

Title:



**EXHIBIT A**  
**TO TRADEMARK LICENSE AGREEMENT**  
**LICENSED MARKS**

<b>Mark</b>	<b>Registration/Application No.</b>	<b>Territory/Country</b>
AMIGAL	3652668	US
AMIGAL	128551900 (Application)	Canada
AMIGAL	969558	Mexico
AMIGAL	828170193 (Application)	Brazil
AMIGAL	879558	EU (Austria, Benelux (Belgium, the Netherlands and Luxembourg), Bulgaria, Cyprus, the Czech Republic, Denmark, Estonia, Finland, France, Germany, Greece, Hungary, Ireland, Italy, Latvia, Lithuania, Malta, Poland, Portugal, Romania, the Slovak Republic, Slovenia, Spain, Sweden and the United Kingdom)  Australia  Japan  Norway  Singapore

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## EXHIBIT D

### TRADEMARK LICENSE AGREEMENT

THIS TRADEMARK LICENSE AGREEMENT (“Agreement”) is made as of the \_\_\_\_\_ day of \_\_\_\_\_, 2010 (the “Effective Date”) by and between Glaxo Group Limited, a company organized under the laws of England and Wales with its registered office at Glaxo Wellcome House, Berkeley Avenue, Greenford, Middlesex, UB6 0NN, England (“Licensor”), as licensor, and Amicus Therapeutics, Inc., a Delaware corporation having a place of business at 6 Cedar Brook Drive, Cranbury, New Jersey, 08512 (“Licensee”), as licensee. Licensee and Licensor are sometimes collectively referred to herein as the “Parties” and separately as a “Party.”

WHEREAS, pursuant to that certain License and Collaboration Agreement by and between Licensee and Licensor, dated as of the \_\_\_\_\_ day of October, 2010 (the “License and Collaboration Agreement”), Licensor agreed to license to Licensee certain trademarks in the Territory as set forth on Exhibit A attached hereto, including all common law rights to such trademarks in the Territory (the “Licensed Marks”);

WHEREAS, Licensor is willing to grant, and Licensee is willing to receive, a license to use the Licensed Marks in connection with Compound and Products (as those terms are defined in the License and Collaboration Agreement) in Territory pursuant to the terms and conditions set forth in this Agreement.

NOW, THEREFORE, in consideration of the mutual covenants, agreements and stipulations set forth herein and in the License and Collaboration Agreement, the receipt and legal sufficiency of which are hereby mutually acknowledged, Licensor and Licensee hereby agree as follows:

1. DEFINITIONS. Capitalized terms not otherwise defined in this Agreement shall have the meanings set forth in the License and Collaboration Agreement.
  2. GRANT OF LICENSE. During the Term of this Agreement, and pursuant to the terms and conditions contained herein, Licensor hereby grants to Licensee and its Affiliates, and Licensee and its Affiliates hereby accept, a non-exclusive, royalty-free license to use the Licensed Marks in the country or countries of the Territory solely in connection with Licensee’s right to:
    - 2.1. Develop Compound and Product in the Field as provided in Article V of the License and Collaboration Agreement;
    - 2.2. Manufacture Compound or Product in the Field in accordance with Section 6.5 of the License and Collaboration Agreement; and
    - 2.3. engage in Commercialization activities in accordance with the then-current Marketing Plan in the Field in accordance with Article VI of the License and Collaboration Agreement.
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Licensee shall have the right to use the Licensed Marks (excluding GSK House Marks) as part of a domain name, subject to Licensor's prior written approval and provided that Licensee remains responsible for all costs associated with development and operation of the associated website.

### **3. USE OF THE LICENSED MARKS**

- 3.1. Upon reasonable advance written request, Licensee agrees to submit to Licensor samples of the Product (to the extent Manufactured by Licensee or its designee) and samples of packaging of the Product displaying the Licensed Marks for Licensor's inspection. If Licensor reasonably determines that Licensee has failed to maintain a level of quality consistent with those normally employed by the Licensor in the use of the Licensor's Trademarks, then Licensor may request that Licensee take reasonable steps to remedy any such deficiencies and Licensee agrees to take commercially reasonable actions to comply with such requests, and in any event, the Licensee shall not use or distribute Product or any packaging for the Product displaying the Licensed Marks until it has complied with such requests.
- 3.2. Licensee and its Affiliates and sub-licensees shall comply with all applicable laws and regulations pertaining to the Commercialization of Products bearing the Licensed Marks, to the extent that Licensee shall perform any such Commercialization activities under the License and Collaboration Agreement.

### **4. MAINTENANCE OF LICENSED MARKS.**

- 4.1. Licensor shall prepare, file, prosecute and maintain trademark applications and registrations for the Licensed Marks used on or in connection with Product in the Territory. All costs and expenses (including but not limited to attorneys' fees and expenses and official fees) of preparing, filing, prosecuting and maintaining the Licensed Marks shall be borne by Licensor.
- 4.2. Licensor shall not (i) abandon any rights in the Licensed Marks in the Territory, (ii) abandon or allow to lapse any pending application for the Licensed Marks in the Territory, or (iii) permit any active registration for the Licensed Marks to lapse, expire or be cancelled in the Territory, without first notifying Licensee.

5. **TERM.** This Agreement shall be effective commencing on the Effective Date and shall continue perpetually unless terminated as set forth in Section 6 below.

### **6. TERMINATION.**

- 6.1. This Agreement shall terminate automatically, without notice or any further action hereunder by either Party: (a) in its entirety upon the expiration or termination of the License and Collaboration Agreement in its entirety by GSK pursuant to Section 13.2 of the License and Collaboration Agreement or by either Party pursuant to Section 13.4 of the License and Collaboration Agreement; or (b) with respect to a particular Licensed Mark, (i) upon assignment of such Licensed Mark (other than a GSK House Mark) to Licensee, if the License and Collaboration Agreement is terminated by GSK pursuant to Section 13.3, or by Amicus pursuant to Section 13.2, of the License and Collaboration Agreement, or (ii) upon termination of the License and Collaboration Agreement by GSK pursuant to Section 13.2, with respect to the Product with which such Licensed Mark is used and/or in the country(ies) of the Territory in which such Licensed Mark is registered or in use by a Party.
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6.2. The Parties may terminate this Agreement in its entirety or on a Licensed Mark-by-Licensed Mark basis at any time and for any reason during the Term upon their mutual written agreement; provided that the Parties shall agree to terminate this Agreement, in its entirety, or on a Licensed Mark-by-Licensed Mark basis, as applicable, if the JSC determines in accordance with Section 2.4, 4.3.1 and 6.3 of the License and Collaboration Agreement that such Licensed Mark(s) shall no longer be used with respect to a Product(s) in a particular country(ies) of the Territory.

6.3. Subject to and in accordance with Section 14.3 of the License and Collaboration Agreement, upon termination or expiration of this Agreement, Licensee agrees, with respect to any Licensed Marks that are not Terminated Product Trademarks assigned (or to be assigned) to Amicus in accordance with Section 14.3.9 of the License and Collaboration Agreement: (i) immediately to discontinue, and to cause all of Licensee's Affiliates and any sub-licensees of Licensee thereof immediately to discontinue, use of such Licensed Marks; (ii) upon Licensor's request, to return to Licensor or destroy all tangible embodiments of any such Licensed Marks; and (iii) if such items or materials are destroyed by Licensee at Licensor's request, to furnish Licensor with certification and evidence of such destruction.

7. **OWNERSHIP.** Licensor represents and warrants, and Licensee acknowledges, that the Licensed Marks are the sole and exclusive property of Licensor or its Affiliates and all goodwill accrued through use of the Licensed Marks shall be deemed to be the absolute property of Licensor or its Affiliates. Licensee further acknowledges that nothing in this Agreement confers upon Licensee any right of ownership in and to the Licensed Marks. Licensee agrees to reasonably cooperate with Licensor to execute, deliver, and otherwise provide to Licensor all information and documents reasonably requested for the purpose of establishing, registering, evidencing or defending Licensor's complete and exclusive ownership of all rights, titles, and interests of every kind and nature whatsoever in and to the Licensed Marks. Licensee agrees not to register, use or authorize the use of any trademark or designation confusingly similar to the Licensed Marks, and Licensee agrees not to challenge Licensor's or its Affiliates' ownership of the Licensed Marks.

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8. ASSIGNMENTS . Neither this Agreement, nor any of the rights or obligations of a Party may be directly or indirectly assigned, sold, delegated or otherwise disposed of by a Party except in connection with such Party's assignment of, and to the same assignee as, the License and Collaboration Agreement in accordance with Section 16.3 of the License and Collaboration Agreement.

8.1. No assignment under this Section 8 shall be effective unless the intended assignee executes and delivers to the Party which is not the assignor a writing whereby the assignee expressly undertakes to perform and comply with all of its assignor's obligations hereunder. Notwithstanding such undertaking, such assignor shall continue to be primarily liable for such assignee's performance hereof and compliance herewith.

8.2. Any assignment in violation of this Section 8 shall be void and of no effect.

8.3. This Agreement, and the rights and obligations of the Parties herein contained, shall be binding upon, and shall inure to the benefit of, the Parties and their respective legal representatives, successors and permitted assigns.

9. AMENDMENTS . No waiver, amendment or modification of any provision hereof or of any right or remedy hereunder shall be effective unless in writing and signed by the Party against whom such waiver, amendment or modification is sought to be enforced.

10. COUNTERPARTS . This Agreement may be executed in any number of counterparts, each of which shall be deemed to be an original, but all of which together shall constitute but one and the same instrument. Delivery of an executed counterpart signature page of this Agreement by facsimile transmission shall be as effective as delivery of a manually executed signature page.

11. APPLICABLE LAW AND DISPUTE RESOLUTION . This Agreement shall be governed by, interpreted and construed, and all claims and disputes, whether in tort, contract or otherwise be resolved in accordance with the substantive laws of the State of Delaware without reference to any rules of conflict of laws. Any and all disputes under this Agreement shall be resolved in accordance with Section 16.2 of the License and Collaboration Agreement.

12. FURTHER ASSURANCES . Each of Party shall, at any time or from time to time after the Effective Date, at the request and expense of the other Party, execute and deliver to the other Party all such instruments and documents or further assurances as the other Party may reasonably request in order to give effect to the transactions contemplated by this Agreement, including but not limited to Licensee's request for Licensor's cooperation to record or register this Agreement with any applicable governmental entity.

13. REPRESENTATIONS AND WARRANTIES . Each Party represents and warrants that (i) this Agreement has been duly and validly executed and delivered by such Party and constitutes a legal and binding obligation of such Party, enforceable against it in accordance with its terms, and (ii) it has all necessary right, power and authority to execute and perform its obligations under this Agreement and to grant the rights granted herein. Licensor further represents and warrants that it is the owner of all right, title, and interest in and to the Licensed Marks and that the execution, delivery, and performance of its obligations under this Agreement will not conflict with or violate any agreement or other obligation of Licensor or binding upon Licensor's assets, including but not limited to the Licensed Marks.

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14. SEVERABILITY. If any provision of this Agreement is held to be illegal, invalid or unenforceable under any present or future law, and if the rights or obligations of either Party under this Agreement will not be materially and adversely affected thereby, (a) such provision will be fully severable, (b) this Agreement will be construed and enforced as if such illegal, invalid or unenforceable provision had never comprised a part hereof, (c) the remaining provisions of this Agreement will remain in full force and effect and will not be affected by the illegal, invalid or unenforceable provision or by its severance herefrom, and (d) in lieu of such illegal, invalid or unenforceable provision, there will be added automatically as a part of this Agreement a legal, valid and enforceable provision as similar to such illegal, invalid or unenforceable provision as may be possible and reasonably acceptable to the Parties.

15. WAIVER. No waiver by any Party in one or more instances of any of the provisions of this Agreement or the breach thereof shall establish a precedent for any other instance with respect to that or any other provision. Furthermore, in case of waiver of a particular provision, all other provisions of this Agreement will continue in full force and effect.

16. INTEGRATION. This Agreement (including Exhibits hereto), and the License and Collaboration Agreement, embodies the entire agreement of the Parties hereto with respect to the subject matter hereof and supersedes any and all prior agreements with respect thereto.

*[Remainder of page intentionally left blank]*

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IN WITNESS WHEREOF, the Parties hereto have caused this Agreement to be executed as of the Effective Date by their duly authorized representatives.

GLAXO GROUP LIMITED

By: \_\_\_\_\_

Name:

Title:

AMICUS THERAPEUTICS, INC.

By: \_\_\_\_\_

Name:

Title:

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**EXHIBIT A**  
**TO TRADEMARK LICENSE AGREEMENT**  
**LICENSED MARKS**

<b>Mark</b>	<b>Registration No.</b>	<b>Territory/Country</b>
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**Schedule 1.25**

**BACKGROUND LICENSE AGREEMENTS**

- 1) \*\*\*\*\*
- 2) \*\*\*\*\*
- 3) Amended and Restated Agreement between Mount Sinai School of Medicine of New York University and Amicus Therapeutics, Inc., dated October 31, 2008.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

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**Schedule 1.34**

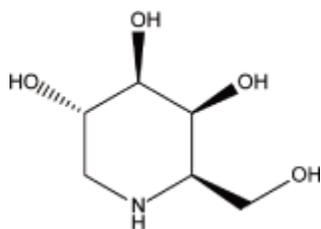
**DESCRIPTION OF COMPOUND**

United States Adopted Name: migalastat

Other Chemical Names:

- 1) 1-deoxygalactonojirimycin
- 2) (2R,3S,4R,5S)-2-(hydroxymethyl)piperidine-3,4,5-triol

Chemical Structure:



MOLECULAR FORMULA: C<sub>6</sub>H<sub>13</sub>NO<sub>4</sub>

MOLECULAR WEIGHT: 163.17

CAS REGISTRY NUMBER: 108147-54-2

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

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**Schedule 2.2**

**RESTRICTED SUBLICENSEES**

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

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### **Schedule 3.5.2**

#### **THIRD PARTY ROYALTIES OWED BY AMICUS**

As of the Effective Date, pursuant to Amended and Restated Agreement between Mount Sinai School of Medicine of New York University (“MSSM”) and Amicus Therapeutics, Inc., dated October 31, 2008 (“MSSM License”) royalties are payable by Amicus to MSSM at the rates specified below and otherwise in accordance with the terms of the MSSM License:

- \*\*\*\*\*; and
- \*\*\*\*\*.

Capitalized terms used in this Schedule 3.5.2 and not defined in the License and Collaboration Agreement to which this Schedule 3.5.2 is attached have the meanings given to such terms in the MSSM License, a copy of which has been provided by Amicus to GSK prior to the Effective Date.

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**Schedule 5.1**

**INITIAL DEVELOPMENT PLAN**

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

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**Schedule 5.1.5**

**DEVELOPMENT COST SHARING**

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**Schedule 6.5.1**

**TECHNOLOGY TRANSFER REQUIREMENTS**

Technology Transfer Requirements

Contacts

1. Name, address, phone, FAX, and e-mail address key technical contacts at **Amicus** and all third parties involved in process development, manufacture, analysis, or release.

Materials

2. Entire inventory of drug substance, and intermediates, along with their batch histories, batch records and analytical results (to the extent such histories, records and analytical results can be reproduced and transferred from the site of the contract manufacturer).
3. All drug substance primary reference standard and any reports describing its characterization and assignment of purity.
4. Working references standards for drug substance, intermediates and impurities along with any report on their comparability, characterization, or assignment of purity.

API (chemical synthesis)

5. An updated schematic of the chemical synthesis, including typical yields for each stage.
6. Copies of detailed complete manufacturing instructions for all stages and operations of the API chemical synthesis processes on the largest scale to date, including all in-process analytical tests and methods.
7. Available documented process knowledge established through development and commercial supply.
8. Cleaning method and validation reports for each stage of the chemical processes.
9. For all key raw materials, a table of suppliers, cost/kg, ordering lead times, and buying specifications, including detailed specifications of any components to the extent access to such information is provided to Amicus by the contract manufacturer.
10. A summary report describing the history of chemical synthesis process development, changes and their reason, and optimization of the processes.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

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## Analytical

11. A report summarizing available data describing the physical properties of the drug substance, including MW, solubility, pI, etc as applicable.
12. The Drug Substance Stability Report, including all data and methods.
13. Current specifications for drug substance and starting materials, including justification for the specifications.
14. A complete drug substance batch history table, including lot number, amount, Certificates of Analyses, and use or intended use (particularly for safety assessment or clinical studies).
15. A statement or certificate of available api inventory is free from TSEs/BSEs.
16. All analytical methods employed for analysis of the drug substance used in safety assessment and clinical trial supplies. This should include methods/limits of detection/limits of quantification for heavy metals and low level potential genotoxic impurities if such analysis is performed.
17. A table listing all impurities (by Retention Time of the major, Relative Retention Time and {% (a/a) or (w/w)} of all impurities present in the drug substance used in safety assessment studies and in the clinical trial supplies.
18. A table or report describing drug substance impurities including critical and typical levels, probable origins, and methods for control, particularly for any known or potential highly-toxic or mutagenic impurities or degradants.
19. A report summarizing effort to characterize drug substance impurities.
20. A report summarizing the history of analytical methods development.
21. A table of isolated intermediate acceptance criteria or buying specifications, and their analytical methods (including purity profile), including limits for any potentially mutagenic or highly toxic impurities.
22. Recommended storage conditions for the drug substance and intermediates suitable for international shipping.
23. Any shipping studies data for drug substance and intermediates including container specifications used for storage and shipping.

## Drug Product: Formulation and Manufacturing

24. Full analytical data on all batches of drug substance that have been converted to drug product.
  25. Reports on and details of pre-formulation studies
  26. Reports on and details of development pharmaceuticals, including all formulation approaches considered and evaluated
  27. Any analytical methods developed or modified subsequent to formulation development, method validation and drug product specifications.
  28. Results of any drug-excipient compatibility studies that have been conducted if applicable.
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29. Any available drug product stability data
30. Details of the manufacturing process
31. Full manufacturing records to the extent access to such information is provided to Amicus by the contract manufacturer (\*\*\*\*\*)
32. Statement or certificate that the drug product capsules do not contain TSEs/BSEs.
33. Inventory of all drug batches, with CoA, shelf life, input drug substance and other details
34. Formulation and process details of toxicology formulations and approaches including crystal form of input drug substance
35. Any shipping studies or data product including container specifications used for storage and shipping.

#### Regulatory

36. All CMC regulatory documentation including all regulatory filings, including agency questions and responses, especially those related to any aspect of primary drug substance manufacture, analysis, batch histories, impurity profiles, or stability.
37. Any regulatory data to support international shipment or shaking of drug substance or process materials.
38. Any drug substance process data or reports needed to support GSK regulatory filings.

#### Environmental, Health and Safety

39. Any worker safety information on the drug substance, formulation, reagents/excipients, including toxicity (including exposure limits, where known), thermal, gaseous or other hazards.
    - MSDS (where applicable)
    - Occupational exposure limits or exposure guidelines (where defined).
    - Occupational Hygiene monitoring and analytical methods (where available)
-

40. A report summarizing any environmental process safety assessment for the API process, including

- Environmental fate and effects data (e.g., aquatic toxicity, biodegradability, bioaccumulation potential) for API/materials/excipients.
- Chemical hazard data for the process used to manufacture drug substance (e.g., material stability, hazardous incompatibilities, etc.)

#### Intellectual Property

- Reference to any Amicus intellectual property (e.g. patents, patent applications) covering the drug substance, intermediates, or synthetic processes.
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**Schedule 6.5.4**

**AMICUS API AND DRUG PRODUCT SPECIFICATIONS**

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

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**Schedule 7.2.1**

**GSK PROSECUTED AMICUS PATENTS**

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

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**Schedule 7.2.2**

**AMICUS PROSECUTED PATENTS**

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

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**Schedule 7.2.3**

**PATENT APPLICATIONS TO BE SEGREGATED PER SECTION 7.2.3**

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

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**Schedule 10.2**

**BACKGROUND LICENSE AGREEMENT PROVISIONS**

**1. MSSM LICENSE**

Pursuant to Section 2.d. of the MSSM License, GSK agrees: (i) to be bound by, and comply with, Sections 6 (Confidential Information), 9 (Liability and Indemnification) and 10 (Security for Indemnification) of the MSSM License (substituting “GSK” for “AMICUS” in such provisions), the text of which is included below and incorporated herein by reference, to the extent applicable to GSK in its capacity as sublicensee; and (ii) that MSSM is an intended third party beneficiary of the Agreement for purposes of enforcing such indemnification and insurance provisions.

Pursuant to Section 2.c. of the MSSM License, GSK agrees: (a) the sublicense granted by Amicus to GSK under the MSSM License shall be subject and subordinate to the terms and conditions of the MSSM License; (b) such sublicense shall expire automatically on the termination of the MSSM License; (c) such sublicense shall not be assignable, in whole or in part; provided, however, that GSK may, with written notice to MSSM, assign the sublicense in connection with a merger or acquisition of GSK or the sale by the sublicensee of substantially all of its assets; (d) GSK shall be entitled to grant further sublicenses, provided that GSK complies with the obligations of Amicus under Section 2.c., Section 2.d. and all other provisions of MSSM License relating to the grant of sublicenses by Amicus under the MSSM License; and (e) both during the term of such sublicense and thereafter GSK shall be bound by a secrecy obligation similar to that imposed on Amicus in Section 6 of the MSSM License, and that GSK shall bind its employees and agents, both during the terms of their employment and thereafter, with a similar undertaking of secrecy. In addition, GSK, in its capacity as a sublicensee under the MSSM License, specifically agrees to comply with the audit rights applicable to sublicensees and the obligation to maintain books and records to enable the determination of the amounts payable by Amicus, as a result of the activities of GSK in its capacity as a sublicensee under the MSSM License.

**Provisions Extracted from MSSM License:**

Capitalized terms in the following provisions of the MSSM License, but not defined therein, shall have the meanings given to such terms in the MSSM License.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

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“6. Confidential Information.

- a. In the course of research to be performed under this Agreement, it will be necessary for each party to disclose “Confidential Information” to the other. For purposes of this Agreement, “Confidential Information” is defined as all information, data and know-how disclosed by one party (the “Disclosing Party”) to the other (the “Receiving Party”), either embodied in tangible materials (including writings, drawings, graphs, charts, photographs, recordings, structures, technical and other information) marked “Confidential” or, if initially disclosed orally, which is reduced to writing marked “Confidential” within 21 days after initial oral disclosure, other than that information which is,
    - i) known by the Receiving Party at the time of its receipt, and not through a prior disclosure by the Disclosing Party, as documented by the Receiving Party’s business records; or
    - ii) at the time of disclosure, or thereafter becomes, published or otherwise part of the public domain without breach of this Agreement by the Receiving Party; or
    - iii) obtained from a third party who has the legal right to make such disclosure and without any confidentiality obligation to the Disclosing Party; or
    - iv) independently developed by the Receiving Party without the use of Confidential Information received from the Disclosing Party and such independent development can be documented by the Receiving Party; or
    - v) disclosed to governmental or other regulatory agencies in order to obtain patents, provided that such disclosure may be made only to the extent reasonably necessary to obtain such patents or authorizations, and further provided that any such patent applications shall be filed in accordance with the terms of this Agreement; or
    - vi) required by law, regulation, rule, act or order of any governmental authority to be disclosed.
  - b. The Receiving Party agrees that at all times and notwithstanding any termination, expiration, or cancellation hereunder, it will hold the Confidential Information of the Disclosing Party in strict confidence, will use all reasonable safeguards to prevent unauthorized disclosure by its employees and agents. Notwithstanding the foregoing, the parties recognize that industry standards with respect to the treatment of Confidential Information may not be appropriate in an academic setting. However, MSSM agrees to retain Confidential Information of AMICUS in the same manner and with the same level of confidentiality as MSSM retains its own Confidential Information.
  - c. The Receiving Party will maintain reasonable procedures to prevent accidental or other loss, including unauthorized publication of any Confidential Information of the Disclosing Party. The Receiving Party will promptly notify the Disclosing Party in the event of any loss or unauthorized disclosure of the Confidential Information.
  - d. Upon termination or expiration of this Agreement, and upon written request, the Receiving Party will promptly return to the Disclosing Party all documents or other tangible materials representing Confidential Information and all copies thereof.
  - e. The Receiving Party will immediately notify the Disclosing Party in writing, if it is requested by a court order, a governmental agency, or any other entity to disclose Confidential Information in the Receiving Party’s possession. The Disclosing Party will have an opportunity to intervene by seeking a protective order or other similar order, in order to limit or prevent disclosure of the Confidential Information. The Receiving Party will disclose only the minimum Confidential Information required to be disclosed in order to comply, whether or not a protective order or other similar order is obtained by the Disclosing Party.”
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“9. Liability and Indemnification.

- a. AMICUS shall indemnify, defend and hold harmless MSSM and its trustees, officers, directors, medical and professional staff, employees, students and agents and their respective successors, heirs and assigns (the “Indemnitees”), against any liability, damage, loss or expense (including reasonable attorneys’ fees and expenses of litigation) incurred by or imposed upon the Indemnitees or any one of them in connection with any claims, suits, actions, demands or judgments: (i) arising out of the production, manufacture, sale, use in commerce or in human clinical trials, lease, or promotion by AMICUS or by a licensee, Affiliate or agent of AMICUS of any Licensed Product, process or service relating to, or developed pursuant to, this Agreement, or (ii) arising out of any other activities to be carried out pursuant to this Agreement.
  - b. AMICUS’s indemnification under subsection a(i), above, shall apply to any liability, damage, loss or expense whether or not it is attributable to the negligent activities of the Indemnitees. AMICUS’s indemnification under subsection a(ii) above, shall not apply to any liability, damage, loss or expense to the extent that it is attributable to the negligence, gross negligence or intentional misconduct of the Indemnitees.
  - c. AMICUS shall, at its own expense, provide attorneys reasonably acceptable to MSSM to defend against any actions brought or filed against any party indemnified hereunder with respect to the subject of indemnity contained herein, whether or not such actions are rightfully brought.
  - d. EXCEPT AS PROVIDED IN THIS SECTION 9, NEITHER PARTY SHALL BE LIABLE TO THE OTHER FOR INCIDENTAL, CONSEQUENTIAL, SPECIAL, EXEMPLARY OR PUNITIVE DAMAGES.
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10. Security for Indemnification.

- a. At such time as any Licensed Product is being commercially distributed or sold (other than for the purpose of obtaining regulatory approvals) by AMICUS or by a sub-licensee, Affiliate or agent of AMICUS and to the extent that it is available on commercially reasonable terms, AMICUS shall at its sole cost and expense, procure and maintain policies of comprehensive general liability insurance in amounts not less than \*\*\*\*\* per incident and \*\*\*\*\* annual aggregate and naming the indemnitees as additional insureds. Such comprehensive general liability insurance shall provide (i) product liability coverage and (ii) broad form contractual liability coverage for AMICUS's indemnification under Section 9 of this Agreement. The minimum amounts of insurance coverage required under this Section 10 shall not be construed as a limit of AMICUS's liability with respect to its indemnification under Section 9 of this Agreement.
  - b. AMICUS shall provide MSSM with written evidence of such insurance upon request of MSSM. AMICUS shall provide MSSM with written notice at least 60 days prior to the cancellation, non-renewal or material change in such insurance; if AMICUS does not obtain replacement insurance providing comparable coverage within such 60 day period effective immediately upon notice to AMICUS, MSSM shall have the right to terminate this Agreement effective at the end of such 60 day period without notice or any additional waiting periods.
  - c. AMICUS shall maintain such comprehensive general liability insurance beyond the expiration or termination of this Agreement during: (i) the period that any product, process or service, relating to, or developed pursuant to, this Agreement is being commercially distributed or sold (other than for the purpose of obtaining regulatory approvals) by AMICUS or by a licensee, Affiliate or agent of AMICUS and (ii) a reasonable period after the period referred to in (c)(i) above which in no event shall be less than seven years."
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**Schedule 13.6**

**CALCULATION OF ROYALTY RATE REDUCTION**

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

Portions of this exhibit have been omitted and filed separately with the Secretary of the Securities and Exchange Commission (the “Commission”) pursuant to an application for confidential treatment filed with the Commission pursuant to Rule 24b-2 under the Securities Exchange Act of 1934, as amended. Such portions are marked as indicated below.

### STOCK PURCHASE AGREEMENT

THIS STOCK PURCHASE AGREEMENT (this “Agreement”) is made as of the 28<sup>th</sup> day of October, 2010 (the “SPA Effective Date”) by and between Amicus Therapeutics, Inc. (“Amicus”), a Delaware corporation with its principal place of business at 6 Cedar Brook Drive, Cranbury, New Jersey 08512, and Glaxo Group Limited, a company organized under the laws of England and Wales with its registered office address at Glaxo Wellcome House, Berkeley Avenue, Greenford, Middlesex, UB6 0NN, England (“GSK”). Amicus and GSK are each referred to herein by name or as a “Party” or, collectively, as the “Parties”.

### RECITALS

WHEREAS, Amicus and GSK entered into that certain License and Collaboration Agreement dated as of October 28th, 2010 (the “License and Collaboration Agreement”); and

WHEREAS, in connection with the execution of the License and Collaboration Agreement, Amicus desires to sell to GSK and GSK desires to purchase from Amicus shares of Common Stock of Amicus on the terms and subject to the conditions set forth in this Agreement.

NOW, THEREFORE, in consideration of the foregoing recitals and the mutual covenants and agreements contained herein, the Parties hereto, intending to be legally bound, do hereby agree as follows:

1. **Definitions**. The capitalized terms used herein shall have the meanings ascribed to them below, provided that capitalized terms used herein that are not defined herein shall have the meanings ascribed to them in the License and Collaboration Agreement:

1.1. “Affiliate” means, with respect to any specified Person, at any time, a Person that, directly or indirectly, through one or more intermediaries, controls, or is controlled by, or is under common control with, such specified Person at such time. For purposes of this definition, “control,” when used with respect to any specified Person, shall mean (a) the direct or indirect ownership of more than fifty percent (50%) (or such lesser percentage which is the maximum allowed to be owned by a foreign corporation in a particular jurisdiction) of the total voting power of securities or other evidences of ownership interest in such Person or (b) the power to direct or cause the direction of the management and policies of such Person, directly or indirectly, whether through ownership of voting securities, by contract or otherwise.

1.2. “Closing” has the meaning ascribed to it in Section 3.1.

1.3. “Closing Date” means the day on which the transaction that is the subject of such Closing is consummated as set forth in Section 3.1.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

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- 1.4. “Common Stock” means the common stock of Amicus, par value \$0.01 per share.
- 1.5. “Exchange Act” means the Securities Exchange Act of 1934, as amended, together with the rules and regulations promulgated thereunder.
- 1.6. “FDA Documents” has the meaning ascribed to such term in Section 4.7.
- 1.7. “GAAP” means generally accepted accounting principles in the United States.
- 1.8. “GSK Indemnitee” has the meaning ascribed to such term in Section 7.5.
- 1.9. “Holder” means each person owning of record Registrable Securities that have not been sold to the public.
- 1.10. “Investor Rights Agreement Investor” means any Person, other than Amicus and GSK, that owns shares of Common Stock and is party to the Third Amended and Restated Investor Rights Agreement dated as of September 13, 2006 by and among Amicus and the investors named therein.
- 1.11. “Knowledge” means the knowledge of such Person, assuming that such Person engaged in reasonable inquiry or investigation with respect to the relative subject matter.
- 1.12. “Lock-Up Period” has the meaning ascribed to such term in Section 8.5.
- 1.13. “Material Adverse Effect” on or with respect to an entity (or group of entities taken as a whole) means any state of facts, event, change or effect that has had, or that would reasonably expected to have, a material adverse effect on the business, properties, results of operations or financial condition of such entity (or of such group of entities taken as a whole).
- 1.14. “Nasdaq” means the Nasdaq Stock Market, Inc.
- 1.15. “Party” means a party to this Agreement.
- 1.16. “Per Share Price” has the meaning ascribed to it in Section 2.
- 1.17. “Purchase Price” has the meaning ascribed to it in Section 2.
- 1.18. “Person” means any individual, firm, corporation, partnership, limited liability company, trust, incorporated or unincorporated association, joint venture, joint stock company, government (or an agency or political subdivision thereof) or other entity of any kind, and shall include any successor (by merger or otherwise) of such entity.
- 1.19. “Register,” “Registered,” and “Registration” refer to a registration effected by preparing and filing a Registration Statement in compliance with the Securities Act, and the declaration or ordering of effectiveness of such Registration Statement or document by the SEC.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

1.20. “Registrable Securities” means (a) the Shares, and (b) any shares of Common Stock of Amicus or other securities issued as (or issuable upon the conversion or exercise of any warrant, right or other security which is issued as) a dividend or other distribution with respect to, or in exchange for or in replacement of, the Shares by way of stock dividend, stock split or in connection with a combination of shares, recapitalization or other reorganization or otherwise. Notwithstanding the foregoing, as to any particular Shares or other securities described above, once issued they shall cease to be Registrable Securities when (1) a registration statement with respect to the sale of such securities shall have become effective under the Securities Act and such securities shall have been disposed of in accordance with such registration statement, (2) they shall have been distributed pursuant to Rule 144 (or any successor provision) under the Securities Act, (3) such securities may be sold without volume restrictions pursuant to Rule 144, as determined by the counsel to Amicus pursuant to a written opinion letter to such effect, addressed and acceptable to Amicus’s transfer agent, or (4) such securities shall have been otherwise transferred in a private transaction in which the rights under Section 7 hereof have not been assigned in connection with such transfer.

1.21. “Registration Statement” means a Registration Statement filed pursuant to the Securities Act.

1.22. “Rule 144” means Rule 144 promulgated under the Securities Act, or any successor rule.

1.23. “SEC Documents” has the meaning ascribed to such term in Section 4.7.

1.24. “SEC Guidance” means (i) any publicly-available written guidance, or rule of general applicability of the SEC staff, or (ii) written comments, requirements or requests of the SEC staff to Amicus in connection with the review of a Registration Statement.

1.25. “SEC” means the U.S. Securities and Exchange Commission.

1.26. “Securities Act” means the Securities Act of 1933, as amended, together with the rules and regulations promulgated thereunder.

1.27. “Shares” means the shares of Common Stock to be acquired by GSK hereunder as set forth in Section 2.

1.28. “Standstill Term” has the meaning ascribed to such term in Section 8.1.

1.29. “Transaction Documents” means this Agreement and the License and Collaboration Agreement.

1.30. “Voting Stock” means securities of any class or series of a corporation or association the holders of which are ordinarily, in the absence of contingencies, entitled to vote generally in matters put before the shareholders or members of such corporation or association, or securities convertible or exchangeable into or exercisable for any such securities.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

2. **Purchase and Sale.**

At the Closing, on terms and conditions as set forth herein, Amicus will sell to GSK and GSK will purchase from Amicus, 6,866,244 shares of Common Stock to be issued by Amicus after the SPA Effective Date in connection with this transaction which, as a percentage of Amicus' total number of shares of Common Stock issued and outstanding immediately following the Closing, will equal nineteen and nine tenths percent (19.9%) of the total number of shares of Common Stock issued and outstanding immediately following the Closing (the "Shares"), which number shall be subject to appropriate and equitable adjustment for any stock split, stock dividend or reclassification of the Common Stock or similar event between the date hereof and the Closing, for an aggregate consideration equal to the product of the number of Shares times the Per Share Price (the "Purchase Price"). For the purposes of this Section 2, the "Per Share Price" shall be equal to \*\*\*\*\*. Schedule A sets forth the expected capitalization of the Company immediately following Closing.

3. **Closing.**

3.1. Closing. Subject to the satisfaction or waiver of the conditions set forth in Article 6, the completion of the sale and purchase of the Shares (the "Closing") shall occur within ten (10) Business Days of the SPA Effective Date; provided that if any conditions have not been so satisfied or waived on such date, the Closing shall occur on the first Business Day after the satisfaction or waiver (by the Party entitled to grant such waiver) of the conditions to the Closing set forth in Article 6 herein (other than those conditions that by their nature are to be satisfied at the Closing, but subject to fulfillment or waiver of those conditions), or on such other date as the parties shall mutually agree (the "Closing Date").

3.2. Delivery. At the Closing, subject to the terms and conditions hereof:

(a) GSK shall deliver to Amicus:

(i) the Purchase Price by wire transfer within ten (10) Business Days after the SPA Effective Date to the following account:

Bank: xxxxxxxxxxxxxxxxxxxx  
Bank Address: xxxxxxxxxxxxxxxxxxxx  
xxxxxxxxxxxxxxxxxxxxx

Beneficiary: Amicus Therapeutics, Inc.  
Beneficiary Address: 6 Cedar Brook Drive  
Cranbury, NJ 08512

ABA: xxxxxxxxxxxxxxxxxxxx  
Account: xxxxxxxxxxxxxxxxxxxx  
SWIFT Code: xxxxxxxxxxxxxxxxxxxx

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

(ii) together with any other documents as are required to be delivered by GSK to Amicus pursuant to the terms of this Agreement; and

(b) Amicus will deliver to GSK a stock certificate, in the name of GSK, representing the Shares purchased at the Closing, dated as of the Closing Date, against payment of such Purchase Price, and any other documents as are required to be delivered by Amicus to GSK pursuant to the terms of this Agreement, including resolutions of the Board of Directors of Amicus approving the transactions contemplated by this Agreement.

3.3. Location. The Closing shall occur at the offices of Amicus, located at 6 Cedar Brook Drive, Cranbury, New Jersey 08512 (or remotely via the exchange of signatures and documents) unless otherwise agreed to by the Parties.

4. **Representations and Warranties of Amicus**. Amicus hereby represents and warrants to GSK as of the date hereof and as of the Closing Date (except as set forth below), as follows:

4.1. Capitalization. As of October 25, 2010, the authorized capital stock of Amicus consisted of (a) 50,000,000 shares of Common Stock, of which (i) 27,637,495 shares were issued and outstanding, (ii) up to 1,854,946 shares have been reserved for issuance upon exercise of outstanding common stock warrants, (iii) 4,966,667 shares have been reserved for issuance under Amicus's Amended and Restated 2007 Equity Incentive Plan, (iv) 390,797 shares have been reserved for issuance under Amicus's Amended and Restated 2007 Director Option Plan, (v) 1,644,268 shares have been reserved for issuance under Amicus's 2002 Equity Incentive Plan, and (vi) 200,000 shares have been reserved for issuance under Amicus's 2007 Employee Stock Purchase Plan; and (b) 10,000,000 shares of preferred stock, none of which is outstanding. All issued and outstanding shares of Amicus's capital stock have been duly authorized and validly issued, and are fully paid and nonassessable, and were issued in compliance with all applicable federal and state securities laws. As of the SPA Effective Date, there are no preemptive or similar rights on the part of any holder of any class or securities of Amicus. As of the SPA Effective Date, except as set forth in the SEC Documents or as described or referred to above, there are no securities convertible into or exchangeable for, or options, warrants, calls, subscriptions, rights, contracts, commitments, or understandings of any kind to which Amicus is a party or by which it is bound obligating Amicus to issue, deliver or sell, or cause to be issued, delivered or sold additional shares of its capital stock or other voting securities of Amicus. As of the SPA Effective Date, there are no outstanding agreements of Amicus to repurchase, redeem or otherwise acquire any shares of its capital stock. At the Closing, Amicus shall provide GSK with a certificate of a duly authorized officer of Amicus setting forth: (a) the capitalization of Amicus immediately following the Closing including the number of shares of the following: (i) issued and outstanding Common Stock, including, with respect to restricted Common Stock, vesting schedule and repurchase price; (ii) issued stock options, including vesting schedule and exercise price; (iii) stock options not yet issued but reserved for issuance; (iv) each series of preferred stock; and (v) warrants or stock purchase rights, if any; and (b) that as of the Closing Date (i) except as set forth in the SEC Documents or as described or referred to above, there are no securities convertible into or exchangeable for, or options, warrants, calls, subscriptions, rights, contracts, commitments, or understandings of any kind to which Amicus is a party or by which it is bound obligating Amicus to issue, deliver or sell, or cause to be issued, delivered or sold additional shares of its capital stock or other voting securities of Amicus; and (ii) there are no outstanding agreements of Amicus to repurchase, redeem or otherwise acquire any shares of its capital stock.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

4.2. Litigation. There are no actions, suits, proceedings or, to its Knowledge, any investigations, pending or currently threatened against Amicus that questions the validity of this Agreement or the issuance of the Common Stock contemplated hereby, nor to its Knowledge, is there any basis therefor. As of the Closing, there is no other material action, suit, or proceeding pending or, to the Knowledge of Amicus, currently threatened against Amicus. As of the Closing, there are no material outstanding consents, orders, decrees or judgments of any governmental entity naming Amicus.

4.3. Organization and Good Standing. Amicus is a corporation duly organized, validly existing and in good standing under the laws of the State of Delaware and has all requisite corporate power and authority to own, lease and operate its properties and carry on its business as now conducted. Amicus is duly qualified and is in good standing as a foreign corporation in each jurisdiction in which the properties owned, leased or operated, or the business conducted, by it requires such qualification except where the failure to be so qualified or in good standing, individually or in the aggregate, would not have a Material Adverse Effect.

4.4. Authorization. All corporate actions on the part of Amicus, its officers, directors and stockholders necessary for the authorization, execution and delivery of the Transaction Documents and for the issuance of the Shares have been taken. Amicus has the requisite corporate power to enter into the Transaction Documents and to carry out and perform its obligations thereunder. The Transaction Documents have been duly authorized, executed and delivered by Amicus and, upon due execution and delivery by GSK, each Transaction Document will be a valid and binding agreement of Amicus, except as enforceability may be limited by bankruptcy, insolvency, reorganization, moratorium or similar laws affecting creditors' rights generally or by equitable principles.

4.5. Subsidiaries. Other than Amicus Therapeutics UK Limited, Amicus does not currently own or control, directly or indirectly, any interest in any other corporation, partnership, trust, joint venture, limited liability company, association, or other business entity. Amicus is not a participant in any joint venture, partnership or similar arrangement.

4.6. No Conflict With Other Instruments. Neither the execution, delivery nor performance of the Transaction Documents, nor the consummation by Amicus of the transactions contemplated hereby will result in any violation of, be in conflict with, cause any acceleration or any increased payments under, or constitute a default under, with or without the passage of time or the giving of notice: (a) any provision of Amicus's Restated Certificate of Incorporation or Bylaws as in effect on the date hereof or at the Closing; (b) any provision of any judgment, decree or order to which Amicus is a party or by which it is bound, (c) any note, mortgage, material contract, material agreement, license, waiver, exemption, order or permit.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

#### 4.7. Disclosure Documents .

(a) For the two years preceding the SPA Effective Date, Amicus has filed, on a timely basis or has received a valid extension as of such time of filing and has thereafter made such filings prior to the expiration of any such extension, all reports, schedules, forms, statements and other documents required to be filed by Amicus with the SEC under the Securities Act and the Exchange Act, including pursuant to Section 13(a) or 15(d) thereof, (the foregoing materials, including the exhibits thereto and documents incorporated by reference therein, being collectively referred to herein as the “SEC Documents”) and with the U.S. Food and Drug Administration (“FDA”) under its applicable regulations (“FDA Documents”), and Amicus has paid all fees and assessments due and payable in connection with the SEC Documents and the FDA Documents. As of their respective dates, the SEC Documents and the FDA Documents complied in all material respects with all statutes and applicable rules and regulations of the SEC or FDA, as applicable, including the requirements of the Securities Act or the Exchange Act, as applicable, and none of the SEC Documents or FDA Documents, when filed, contained any untrue statement of a material fact or omitted to state a material fact required to be stated therein or necessary in order to make the statements therein, in the light of the circumstances under which they were made, not misleading.

(b) The audited financial statements of Amicus included in Amicus’s SEC Documents comply in all material respects with the published rules and regulations of the SEC with respect thereto, and such audited financial statements (i) were prepared from the books and records of Amicus, (ii) were prepared in accordance with GAAP applied on a consistent basis (except as may be indicated therein or in the notes or schedules thereto) and (iii) present fairly the financial position of Amicus as of the dates thereof and the results of operations and cash flows for the periods then ended. The unaudited financial statements included in the SEC Documents comply in all material respects with the published rules and regulations of the SEC with respect thereto, and such unaudited financial statements (i) were prepared from the books and records of Amicus, (ii) were prepared in accordance with GAAP, except as otherwise permitted under the Exchange Act and the rules and regulations thereunder, applied on a consistent basis (except as may be indicated therein or in the notes or schedules thereto) and (iii) present fairly the financial position of Amicus as of the dates thereof and the results of operations and cash flows (or changes in financial condition) for the periods then ended, subject to normal year-end adjustments and any other adjustments described therein or in the notes or schedules thereto.

4.8. Absence of Certain Events and Changes . Since the date of Amicus’s Quarterly Report on Form 10-Q for the quarter ended on June 30, 2010: (i) Amicus has conducted its business in the ordinary course consistent with past practice, (ii) there has not been any event, change or development which, individually or in the aggregate, would have a Material Adverse Effect, taken as a whole, (iii) Amicus has not incurred any material liabilities (contingent or otherwise) other than expenses incurred in the ordinary course of business consistent with past practice, (iv) Amicus has not altered its method of accounting in any material respect, and (v) Amicus has not declared or made any dividend or distribution of cash or other property to its shareholders or purchased, redeemed or made any agreements to purchase or redeem any shares of its capital stock.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

4.9. Intellectual Property. Amicus owns, or has an exclusive right pursuant to a valid, written license agreement to use and exploit, all material Intellectual Property used in or necessary for the conduct of the business of Amicus as conducted as of the Closing. No claims have been asserted by a third party in writing (a) alleging that the conduct of the business of Amicus has infringed or misappropriated any Intellectual Property rights of such third party, or (b) challenging or questioning the validity or effectiveness of any Intellectual Property right of Amicus, and, to the knowledge of Amicus, there is no valid basis for any such claim (a) or (b). To the knowledge of Amicus, no third party is misappropriating or infringing any Intellectual Property right of Amicus. No loss or expiration of any of Amicus' material Intellectual Property is pending, or, to the knowledge of Amicus, threatened. Amicus has taken reasonable steps in accordance with standard industry practices to protect its rights in its Intellectual Property and at all times has maintained the confidentiality of all information used in connection with the business that constitutes or constituted a trade secret of Amicus.

4.10. Compliance with Applicable Law. Amicus has all material permits, licenses, franchises, authorizations, orders and approvals of, and has made all filings, applications and registrations with, governmental entities that are required in order to permit Amicus to own or lease properties and assets and to carry on its business as presently conducted that are material to Amicus. Amicus has complied and is in compliance in all material respects with all statutes, laws, regulations, rules, judgments, orders and decrees of all governmental entities applicable to it that relate to its business, including but not limited to compliance with the U.S. Foreign Corrupt Practices Act of 1977 (FCPA) (15 U.S.C. §§ 78dd-1, et seq.) and any applicable similar laws in foreign jurisdictions in which Amicus is currently, or has previously, conducted its business or is currently, or has previously, conducted clinical trials. Amicus has not received any notice alleging noncompliance, and, to the Knowledge of Amicus, Amicus is not under investigation with respect to, or threatened to be charged, with any material violation of any applicable statutes, laws, regulations, rules, judgments, orders or decrees of any governmental entities.

4.11. Valid Issuance of Shares. When issued, sold and delivered in accordance with the terms hereof for the consideration expressed herein, the Shares will be duly and validly authorized and issued, fully paid and non-assessable, free and clear of all liens, and, based in part on the representations of GSK in Section 5 of this Agreement, will be issued in compliance with all applicable federal and state securities laws.

4.12. Governmental Consents. No consent, approval, order or authorization of, or registration, qualification, designation, declaration or filing with, any federal, state or local governmental authority on the part of Amicus is required in connection with the consummation of the transactions contemplated by the Transaction Documents, except for notices required or permitted to be filed with certain state and federal securities commissions, which notices will be filed on a timely basis.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

4.13. No Brokers. No broker, finder or investment banker is entitled to any brokerage, finder's or other fee or commission in connection with the transactions contemplated by this Agreement based on arrangements made by Amicus.

4.14. No Undisclosed Liabilities. Amicus does not have any liabilities (contingent or otherwise), except for (i) liabilities reflected or reserved against in financial statements of Amicus included in the SEC Documents filed with the SEC prior to the date of this Agreement, and (b) liabilities that have not been and would not reasonably be expected to be material.

4.15. Internal Controls. The records, systems, controls, data and information of Amicus are recorded, stored, maintained and operated under means (including any electronic, mechanical or photographic process, whether computerized or not) that are under the exclusive ownership and direct control of Amicus (including all means of access thereto and therefrom), except for any non-exclusive ownership and non-direct control that would not reasonably be expected to have a material adverse effect on the system of internal accounting controls described herein.

5. **Representations And Warranties Of GSK**. GSK hereby represents and warrants to Amicus as of the date hereof and as of the Closing Date as follows:

5.1. Legal Power. GSK has the requisite corporate power to enter into the Transaction Documents, to carry out and perform its obligations under the terms of the Transaction Documents.

5.2. Due Execution. The Transaction Documents have been duly authorized, executed and delivered by GSK, and, upon due execution and delivery by Amicus, each of the Transaction Documents will be a valid and binding agreement of GSK, except as enforceability may be limited by bankruptcy, insolvency, reorganization, moratorium or similar laws affecting creditors' rights generally or by equitable principles.

5.3. Ownership. As of the date hereof, GSK and its Affiliates do not currently own any of the outstanding Voting Stock of Amicus.

5.4. Investment Representations. In connection with the offer, purchase and sale of the Shares, GSK makes the following representations:

(a) GSK is acquiring the Shares for its own account, not as nominee or agent, for investment and not with a view to, or for resale in connection with, any distribution or public offering thereof within the meaning of the Securities Act.

(b) GSK understands that:

(i) the Shares have not been registered under the Securities Act by reason of a specific exemption therefrom, that such securities may be required to be held by it indefinitely under applicable securities laws, and that GSK must, therefore, bear the economic risk of such investment indefinitely, unless a subsequent disposition thereof is registered under the Securities Act or is exempt from such registration;

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

(ii) each certificate representing such Shares will be endorsed with the following legend:

“THE SECURITIES REPRESENTED HEREBY HAVE NOT BEEN REGISTERED UNDER THE SECURITIES ACT OF 1933, AS AMENDED (THE “ACT”), OR UNDER THE SECURITIES LAWS OF CERTAIN STATES. THESE SECURITIES ARE SUBJECT TO RESTRICTIONS ON TRANSFERABILITY AND RESALE AND MAY NOT BE TRANSFERRED OR RESOLD EXCEPT AS PERMITTED UNDER THE ACT AND ANY APPLICABLE STATE SECURITIES LAWS, PURSUANT TO REGISTRATION OR EXEMPTION THEREFROM OR UNLESS THE COMPANY HAS RECEIVED AN OPINION OF COUNSEL SATISFACTORY TO THE COMPANY AND ITS COUNSEL THAT SUCH REGISTRATION IS NOT REQUIRED.”; and

(iii) Amicus will instruct its transfer agent not to register the transfer of the Shares (or any portion thereof) unless the conditions specified in the foregoing legends are satisfied.

(c) GSK has such knowledge and experience in financial or business matters that it is capable of evaluating the merits and risks of the investment in the Shares to be purchased hereunder.

(d) GSK is an “accredited investor” as such term is defined in Rule 501(a) of the rules and regulations promulgated under the Securities Act.

## 6. Conditions To Closing.

6.1. Conditions to Obligations of GSK at the Closing. GSK’s obligation to purchase the Shares at the Closing is subject to the fulfillment to its reasonable satisfaction, on or prior to the Closing, of all of the following conditions, any of which may be waived by GSK:

(a) Representations and Warranties True. The representations and warranties made by Amicus in Section 4 hereof shall be true and correct in all material respects on the SPA Effective Date and (except for the representations and warranties made in the first sentence of Section 4.1) the Closing Date with the same force and effect as if they had been made on and as of such date, and a certificate duly executed by an officer of Amicus, to the effect of the foregoing, shall be delivered to GSK.

(b) Performance of Obligations. Amicus shall have performed and complied with all obligations and conditions herein required to be performed or complied with by it on or prior to the Closing and a certificate duly executed by an officer of Amicus, to the effect of the foregoing, shall be delivered to GSK.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

(c) Proceedings and Documents . All corporate and other proceedings in connection with the transactions contemplated at the Closing and all documents and instruments incident to such transactions shall be reasonably satisfactory in substance and form to GSK, and GSK shall have received all such counterpart originals or certified or other copies of such documents as it may reasonably request.

(d) Qualifications; Legal Investment . All authorizations, approvals, or permits, if any, of any governmental authority or regulatory body of the United States or of any state that are required in connection with the lawful sale and issuance of the Shares shall have been duly obtained and shall be effective on and as of the Closing. No stop order or other order enjoining the sale of the Shares shall have been issued and no proceedings for such purpose shall be pending or, to the Knowledge of Amicus, threatened by the SEC.

(e) Nasdaq Listing . If required by Nasdaq, the Shares shall have been approved for listing on the Nasdaq Stock Market, subject only to official notice of issuance.

6.2. Conditions to Obligations of Amicus at the Closing . Amicus's obligation to issue and sell the Shares at the Closing is subject to the fulfillment to its reasonable satisfaction, on or prior to the Closing, of the following conditions, any of which may be waived by Amicus:

(a) Representations and Warranties True . The representations and warranties made by GSK in Section 5 hereof shall be true and correct in all material respects on the Closing Date with the same force and effect as if they had been made on and as of such date, and a certificate duly executed by an officer of GSK, to the effect of the foregoing, shall be delivered to Amicus.

(b) Performance of Obligations . GSK shall have performed and complied with all agreements and conditions herein required to be performed or complied with by it on or before the Closing, and a certificate duly executed by an officer of GSK, to the effect of the foregoing, shall be delivered to Amicus.

(c) Qualifications; Legal Investment . All authorizations, approvals, or permits, if any, of any governmental authority or regulatory body of the United States or of any state that are required in connection with the lawful sale and issuance of the Shares shall have been duly obtained and shall be effective on and as of the Closing. No stop order or other order enjoining the sale of the Shares shall have been issued and no proceedings for such purpose shall be pending or, to the Knowledge of Amicus, threatened by the SEC.

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6.3. Condition to Obligations of each Party at the Closing. The obligations of Amicus and GSK to consummate the transactions contemplated to occur at the Closing shall be subject to the satisfaction prior to Closing of the following conditions, each of which may be waived by the other party only if it is legally permitted to do so.

(a) HSR and Other Approvals. Any applicable waiting period under the Hart-Scott-Rodino Antitrust Improvement Act of 1976 (the “HSR Act”) relating to the transactions contemplated hereby shall have expired or been terminated, and all other material authorizations, consents, orders or approvals of, or regulations, declarations or filings with, or expirations of applicable waiting periods imposed by, any governmental entity (including, without limitation, any foreign antitrust filing) necessary for the consummation of the transactions contemplated hereby, shall have been obtained or filed or shall have occurred.

(b) No Litigation, Injunctions or Restraints. No statute, rule, regulation, executive order, decree, temporary restraining order, preliminary or permanent injunction or other order enacted, entered, promulgated, enforced or issued by any governmental entity or other legal restraint or prohibition preventing the consummation of the transactions contemplated by this Agreement shall be in effect.

(c) License and Collaboration Agreement. The License and Collaboration Agreement shall continue to be in full force and effect.

## 7. Registration Rights.

7.1. Registration. As soon as reasonably practicable, but no event later than sixty (60) days after the Closing, Amicus shall prepare and file with the SEC a Registration Statement covering the resale of all, or such portion as permitted by SEC Guidance (provided that, Amicus shall use commercially reasonable efforts to advocate with the SEC for the registration of the maximum number of the Registrable Securities permitted by SEC Guidance), of the Registrable Securities and use commercially reasonable efforts to cause a Registration Statement to be declared effective (including, without limitation, the execution of any required undertaking to file post-effective amendments) as promptly as possible after the filing thereof, but in any event prior to the date which is: (i) one hundred twenty (120) days after the Closing if the Registration Statement is not reviewed by the SEC, or (ii) one-hundred fifty days (150) days after the Closing, if the Registration Statement is reviewed by the SEC. The Registration Statement shall be on Form S-3 (except if Amicus fails to meet one or more of the registrant requirements specified in General Instruction I.A. on Form S-3, such registration shall be on another appropriate form in accordance herewith).

7.2. Expenses Of Registration. Amicus shall pay all fees and expenses incurred in connection with any registration, qualification, exemption or compliance by Amicus in the performance of its obligations pursuant to this Section 7, whether or not any Registrable Securities are sold pursuant to a Registration Statement, and including all registration and filing fees, exchange listing fees, and the fees and expenses of counsel and accountants for Amicus.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

7.3. Obligations Of Amicus. In the case of registration, qualification, exemption or compliance effected by Amicus pursuant to this Agreement, Amicus will, upon request of GSK, inform GSK as to the status of such registration, qualification, exemption and compliance. Amicus shall, at its expense and in addition to its obligations under Section 7.1, as expeditiously as reasonably possible:

(a) except for such times as Amicus is permitted hereunder to suspend the use of the prospectus forming part of the Registration Statement, use its commercially reasonable efforts to keep such registration, and any required qualification, exemption or compliance under state securities laws, continuously effective with respect to GSK and its permitted assignees, until the date all Shares held by GSK may be sold during any ninety (90) day period under Rule 144 and any contractual agreements with Amicus. The period of time during which Amicus is required hereunder to keep the Registration Statement effective is referred to herein as the “**Registration Period** .”

(b) advise GSK promptly (and, in any event, within five (5) business days):

(i) when the Registration Statement or any amendment thereto has been filed with the SEC and when the Registration Statement or any post-effective amendment thereto has become effective;

(ii) of the receipt by Amicus of any notification from the SEC of any stop order suspending the effectiveness of the Registration Statement or the initiation of any proceedings for such purpose;

(iii) of the receipt by Amicus of any notification with respect to the suspension of the qualification of the Registrable Securities included therein for sale in any jurisdiction or the initiation or threatening of any proceeding for such purpose; and

(iv) of the occurrence of any event that requires the making of any changes in the Registration Statement or the prospectus so that, as of such date, the statements therein are not misleading and do not omit to state a material fact required to be stated therein or necessary to make the statements therein (in the case of the prospectus, in the light of the circumstances under which they were made) not misleading;

(c) use its commercially reasonable efforts to obtain the withdrawal of any order suspending the effectiveness of any Registration Statement as soon as reasonably practicable;

(d) if GSK so requests in writing, promptly furnish to GSK, without charge, at least one copy of such Registration Statement and any post-effective amendment thereto, including financial statements and schedules, and, if explicitly requested, all exhibits in the form filed with the SEC;

(e) during the Registration Period, promptly deliver to GSK, without charge, at least one copy of the prospectus included in such Registration Statement and any amendment or supplement thereto and as many additional copies as GSK may reasonably request; and Amicus consents to the use, consistent with the provisions hereof, of the prospectus or any amendment or supplement thereto by GSK in connection with the offering and sale of the Registrable Securities covered by the prospectus or any amendment or supplement thereto;

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

(f) during the Registration Period, if GSK so requests in writing, deliver to GSK, without charge, (i) one copy of the following documents, other than those documents available via EDGAR (and excluding, in each case, exhibits thereto): (A) its annual report to its stockholders, if any (which annual report will contain financial statements audited in accordance with generally accepted accounting principles in the United States of America by a firm of certified public accountants of recognized standing), (B) if not included in substance in its annual report to stockholders, its annual report on Form 10-K (or similar form), (C) its definitive proxy statement with respect to its annual meeting of stockholders, (D) each of its quarterly reports to its stockholders, and, if not included in substance in its quarterly reports to stockholders, its quarterly report on Form 10-Q (or similar form), and (E) a copy of the Registration Statement; and (ii) if explicitly requested, any exhibits filed with respect to the foregoing;

(g) upon the occurrence of any event contemplated by Section 7.3(b)(iv) above, except for such times as Amicus is permitted hereunder to suspend the use of the prospectus forming part of the Registration Statement, Amicus will use its commercially reasonable efforts to as soon as reasonably practicable prepare a post-effective amendment to the Registration Statement or a supplement to the related prospectus, or file any other required document so that, as thereafter delivered to GSK, the prospectus will not include any untrue statement of a material fact or omit to state any material fact necessary to make the statements therein, in the light of the circumstances under which they were made, not misleading;

(h) comply in all material respects with all applicable rules and regulations of the SEC which could affect the sale of the Registrable Securities;

(i) use its commercially reasonable efforts to cause all Registrable Securities to be listed on each securities exchange or market, if any, on which equity securities issued by Amicus have been listed;

(j) use its commercially reasonable efforts to take all other steps necessary to effect the registration of the Registrable Securities contemplated hereby and to enable GSK to sell Registrable Securities under Rule 144; and

(k) permit counsel for GSK to review the Registration Statement and all amendments and supplements thereto, within two (2) business days prior to the filing thereof with the Commission;

*provided* that, in the case of clause (k) above, Amicus will not be required to delay the filing of the Registration Statement or any amendment or supplement thereto to incorporate any comments to the Registration Statement or any amendment or supplement thereto by or on behalf of GSK if such comments would require a delay in the filing of such Registration Statement, amendment or supplement, as the case may be.

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If at any time during the Registration Period there is not an effective Registration Statement covering all of the Registrable Securities and Amicus determines to prepare and file with the SEC a registration statement relating to an offering for its own account or the account of any other stockholder upon demand (a “Demanding Stockholder”) under the Securities Act of any of its equity securities, other than on Form S-4 or Form S-8 (each as promulgated under the Securities Act) or their then equivalents (an “Incidental Registration”), then Amicus will send to GSK written notice of such determination and, if within ten (10) business days after receipt of such notice, GSK will so request in writing, Amicus will use commercially reasonable efforts to include in such registration statement or, in the case of an underwritten offering, cause the managing underwriter or underwriters to include, all or any part of such Registrable Securities GSK requests to be registered, on the same terms and conditions as the securities of Amicus or of the Demanding Stockholder included therein. In connection with any Incidental Registration, Amicus shall not be required to include any Registrable Securities in such underwritten offering unless GSK accepts the terms of the underwritten offering as agreed upon between Amicus, the Demanding Stockholder, if any, and the underwriter, and then only in such quantity as the underwriter believes will not have a material adverse effect on the success of such offering. If the underwriter determines that the registration of all or part of the Registrable Securities which GSK has requested to be included would have a material adverse effect on the success of such offering, then Amicus shall be required to include in such Incidental Registration, to the extent of the amount that the underwriter believes may be sold without causing such adverse effect, first, all of the securities to be offered for the account of Amicus or the account of the Demanding Stockholder; second, any securities to be offered for the account of the Investor Rights Agreement Investors, if any, and third, the Registrable Securities; provided, that (i) if at any time after giving written notice of its intention to register any securities and prior to the effective date of the registration statement filed in connection with such registration, Amicus will determine for any reason not to register or to delay registration of such securities, Amicus may, at its election, give written notice of such determination to GSK and, thereupon, (A) in the case of a determination not to register, will be relieved of its obligation to register any Registrable Securities to this paragraph in connection with such registration (but not from its obligation to pay expenses in accordance with this Agreement), and (B) in the case of a determination to delay registering, will be permitted to delay registering any Registrable Securities being registered pursuant to this paragraph for the same period as the delay in registering such other securities.

#### 7.4. Furnishing Information .

(a) It shall be a condition precedent to the obligations of Amicus to take any action pursuant to Section 7.1 that the selling Holders shall furnish to Amicus such information regarding themselves, the Registrable Securities held by them and the intended method of disposition of such securities as shall be legally required under the Securities Act or otherwise required by the SEC to effect the registration of their Registrable Securities.

#### 7.5. Indemnification; Contribution .

(a) Amicus shall indemnify and hold harmless each Holder (including the employees, agents, representatives, officers and directors of GSK and its Affiliates) (each a “GSK Indemnitee”) from and against any and all losses, claims, damages, liabilities and expenses (including reasonable costs of investigation) arising out of or based upon any untrue, or allegedly untrue, statement of a material fact contained in any Registration Statement, prospectus or preliminary prospectus or notification or offering circular (as amended or supplemented if Amicus shall have furnished any amendments or supplements thereto) or arising out of or based upon any omission or alleged omission to state therein a material fact required to be stated therein or necessary to make the statements therein not misleading, except insofar as the same are caused by or contained in any information concerning such Holder furnished in writing to Amicus by such Holder expressly for use in such Registration Statement.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

(b) Each Holder shall indemnify and hold harmless Amicus, and its respective directors, officers, employees and each Person who controls Amicus (within the meaning of the Securities Act and the Exchange Act) from and against any and all losses, claims, damages, liabilities and expenses (including reasonable costs of investigation) arising out of or based upon any untrue, or allegedly untrue, statement of a material fact contained in any Registration Statement, prospectus or preliminary prospectus or notification or offering circular (as amended or supplemented if Amicus shall have furnished any amendments or supplements thereto) or arising out of or based upon any omission or alleged omission to state therein a material fact required to be stated therein or necessary to make the statements therein not misleading, if such statement or omission was made in reliance upon and in conformity with any information concerning such Holder furnished in writing to Amicus by such Holder specifically for use in the preparation of such Registration Statement or prospectus.

(c) Each Person entitled to indemnification hereunder (the “Indemnified Party”) agrees to give prompt written notice to the indemnifying party (the “Indemnifying Party”) after the receipt by the Indemnified Party of any written notice of the commencement of any action, suit, proceeding or investigation or threat thereof made in writing for which the Indemnified Party intends to claim indemnification or contribution pursuant to this Agreement; provided, however, that the failure to so notify the Indemnifying Party shall not relieve the Indemnifying Party of any liability that it may have to the Indemnified Party hereunder unless, and only to the extent that, such failure results in the Indemnifying Party’s forfeiture of substantive rights or defenses. If notice of commencement of any such action is given to the Indemnifying Party as above provided, the Indemnifying Party shall be entitled to participate in and, to the extent it may wish, jointly with any other Indemnifying Party similarly notified, to assume the defense of such action at its own expense, with counsel chosen by it and reasonably satisfactory to such Indemnified Party. The Indemnified Party shall have the right to employ separate counsel in any such action and participate in the defense thereof, but the fees and expenses of such counsel (other than reasonable costs of investigation) shall be paid by the Indemnified Party unless (i) the Indemnifying Party agrees to pay the same, (ii) the Indemnifying Party fails to assume the defense of such action with counsel reasonably satisfactory to the Indemnified Party in its reasonable judgment or (iii) the named parties to any such action (including any impleaded parties) have been advised by such counsel that either (x) representation of such Indemnified Party and the Indemnifying Party by the same counsel would be inappropriate under applicable standards of professional conduct or (y) there may be one or more legal defenses available to it which are different from or additional to those available to the Indemnifying Party. In either of such cases, the Indemnifying Party shall not have the right to assume the defense of such action on behalf of such Indemnified Party. No Indemnifying Party shall be liable for any settlement entered into without its written consent (other than in the case where the Indemnifying Party is unconditionally released from liability and its rights are not adversely effected), which consent shall not be unreasonably withheld.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

(d) If the indemnification provided for in this Section 7.5 from the Indemnifying Party pursuant to applicable law is unavailable to an Indemnified Party hereunder in respect of any losses, claims, damages, liabilities or expenses referred to therein, then the Indemnifying Party, in lieu of indemnifying such Indemnified Party, shall contribute to the amount paid or payable by such Indemnified Party as a result of such losses, claims, damages, liabilities or expenses in such proportion as is appropriate to reflect the relative fault of the Indemnifying Party and Indemnified Party in connection with the actions which resulted in such losses, claims, damages, liabilities or expenses, as well as any other relevant equitable considerations. The relative faults of such Indemnifying Party and Indemnified Party shall be determined by reference to, among other things, whether any action in question, including any untrue or alleged untrue statement of a material fact or omission or alleged omission to state a material fact, has been made by, or relates to information supplied by, such Indemnifying Party or Indemnified Party, and the parties' relative intent, knowledge, access to information and opportunity to correct or prevent such action. The amount paid or payable by a party as a result of the losses, claims, damages, liabilities and expenses referred to above shall be deemed to include, subject to the limitations set forth in Sections 7.5(a), (b) and (c), any legal or other fees, charges or expenses reasonably incurred by such party in connection with any investigation or proceeding. The parties hereto agree that it would not be just and equitable if contribution pursuant to this Section 7.5(d) were determined by pro rata allocation or by any other method of allocation which does not take account of the equitable considerations referred to in the immediately preceding paragraph. No Person guilty of fraudulent misrepresentation (within the meaning of Section 11(f) of the Securities Act) shall be entitled to contribution from any Person.

7.6. Rule 144 Reporting. In order to make the benefits of the rules and regulations of the SEC that may permit the sale of the Registrable Securities to the public without registration available to GSK, Amicus agrees to use commercially reasonable efforts to:

(a) make and keep public information available, as those terms are understood and defined in Rule 144(c)(1) or any similar or analogous rule promulgated under the Securities Act, at all times after the SPA Effective Date;

(b) file with the SEC, in a timely manner, all reports and other documents required of Amicus under the Exchange Act; and

(c) so long as GSK owns any Registrable Securities, furnish GSK forthwith upon request: (i) a written statement by Amicus as to its compliance with the reporting requirements of Rule 144 under the Securities Act, and of the Exchange Act; (ii) a copy of the most recent annual or quarterly report of Amicus; and (iii) such other reports and documents as GSK may reasonably request in availing itself of any rule of regulation of the SEC allowing it to sell any such securities without registration.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

7.7. Assignment of Registration Rights. The rights and obligations under this Section 7 may only be assigned by GSK to a transferee or assignee of Registrable Securities that is (a) an Affiliate or (b) a successor (by operation of law or otherwise) to substantially all the business or assets of GSK; provided, however, that such attempted assignment shall be void unless (i) GSK, within thirty (30) days after such transfer, furnishes to Amicus written notice of the name and address of such transferee or assignee and the securities with respect to which such registration rights are being assigned, and (ii) such transferee agrees to be subject to all obligations and restrictions with respect to the Shares set forth in this Agreement.

## 8. Stock Ownership Governance

8.1. Standstill Agreement. Until eighteen months after the Closing Date (the “Standstill Term”), except (i) with the prior written consent of Amicus or (ii) by way of stock dividends or other distributions made to Amicus’ stockholders generally, GSK will not, and will not encourage, direct, facilitate or cause any of its Affiliates, employees, representatives or agents to, directly or indirectly, subject to Section 8.2:

(a) acquire or agree, offer, seek or propose to acquire ownership (including, but not limited to, beneficial ownership as defined in Rule 13d-3 under the Securities Exchange Act) of any Voting Stock of Amicus or securities convertible or exchangeable into or exercisable for any Voting Stock of Amicus if, as a result of such acquisition, GSK in the aggregate would own more than 19.9% of the Voting Stock of Amicus at the time of such acquisition;

(b) cause to be acquired ownership (including, but not limited to, beneficial ownership as defined in Rule 13d-3 under the Securities Exchange Act) of any Voting Stock of Amicus or securities convertible or exchangeable into or exercisable for any Voting Stock of Amicus if, as a result of such acquisition, the Person acquiring ownership together with GSK and its Affiliates, employees, representatives or agents, in the aggregate, would own more than 19.9% of the Voting Stock of Amicus at the time of such acquisition;

(c) make, or in any way participate in, any “solicitation” of “proxies” (as such terms are defined under Regulation 14A of the Securities Exchange Act) to vote or seek to advise or influence in any manner whatsoever any Person with respect to Voting Stock of Amicus;

(d) form, join or in any way participate in a “group” (within the meaning of Section 13(d)(3) of the Securities Exchange Act) with respect to any Voting Stock of Amicus;

(e) arrange, or in any way participate in, any financing for the purchase of any Voting Stock of Amicus or securities convertible or exchangeable into or exercisable for any Voting Stock of Amicus;

(f) otherwise act, whether alone or in concert with others, to seek to propose under Rule 14a-8 of the Exchange Act to Amicus or any of its stockholders any merger, business combination, restructuring, recapitalization or similar transaction to or with Amicus or induce or attempt to induce any other person to initiate any stockholder proposal;

(g) call or seek to have called any meeting of the stockholders of Amicus or execute any written consent in lieu of a meeting of holders of Voting Stock of Amicus;

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(h) seek election or seek to place a representative on the Board of Directors of Amicus (the “ Board of Directors ”) or seek the removal of any member of the Board of Directors; or

(i) enter into any discussions, negotiations, arrangements or understandings with or advise, assist or encourage any third party with respect to, any of the foregoing. Neither the ownership nor purchase by a venture capital arm of GSK or GSK’s Affiliates, directly or indirectly, of securities of Amicus shall be deemed to be a breach of the obligations under this Section 8.1; provided, such venture capital arm does not later transfer beneficial ownership of such securities (or agrees to vote such securities on behalf of) GSK or its Affiliates.

Amicus and GSK acknowledge and agree that the acquisition by any employee benefit plan of GSK or its Affiliates in any diversified index, mutual or pension fund managed by an independent investment advisor, which fund in turn holds, directly or indirectly, Voting Stock of Amicus shall not be deemed to be a breach of this Section 8.1. For clarity, the provisions of Section 8.1 shall not be construed or interpreted to prohibit GSK or an Affiliate in any manner from making any bid or offer to license or acquire rights to any asset(s) of Amicus (other than substantially all of the assets of Amicus) as opposed to acquiring securities of Amicus if such bid or offer is solicited from GSK or an Affiliate by Amicus. The obligations in Section 8.1 will not prohibit GSK or an Affiliate from confidentially communicating to Amicus’ Chief Executive Officer or Chairman of the Board of Directors a non-public indication of GSK’s interest in pursuing a potential transaction involving Amicus in such a manner that would not require Amicus to make a public disclosure. In addition, if GSK or an Affiliate acquires securities of, or other ownership interest in, a third party that directly or indirectly owns any Voting Stock of Amicus, such acquisition shall not be deemed to be a breach by GSK of the obligations under Section 8.1.

8.2. Exceptions to Section 8.1. Standstill Provisions. Notwithstanding the foregoing but subject to the proviso appearing beneath clause (d) below, the obligations under Section 8.1 shall terminate as to GSK and its Affiliates in the event that:

(a) any third party commences an unsolicited tender or exchange offer which, if successful, would result in such third party beneficially owning not less than 50% of all outstanding Voting Stock (on a Common Stock equivalent basis), and such offer is not withdrawn or terminated within ten (10) business days after its commencement;

(b) it is publicly disclosed that at least 50% of all outstanding Voting Stock (on a Common Stock equivalent basis) have been acquired by any person or group that is unaffiliated with GSK and its Affiliates;

(c) Amicus publicly announces a decision of Amicus’ Board of Directors to conduct a formal process to sell all or substantially all of the assets of Amicus; provided that the restrictions in Section 8.1 will automatically be reinstated and be in full force and effect if and at such time as Amicus publicly announces a termination of such process;

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(d) a third party commences a tender offer for more than fifty percent (50%) of the Voting Stock of Amicus, and Amicus has publicly recommended acceptance of such tender offer; provided, the obligations in Section 8.1 will automatically be reinstated in the event such tender offer is terminated;

(e) Amicus enters into any binding written agreement (i) to sell or dispose of securities representing at least 50% of all outstanding Voting Stock (on a Common Stock equivalent basis) to any person or group that is unaffiliated with GSK and all of its Affiliates or (ii) providing for a transaction that, if consummated, would result in (A) the holders of the outstanding Voting Stock immediately prior to such transaction ceasing to hold more than fifty percent (50%) of the combined voting power of the surviving, purchasing or continuing entity immediately after such transaction or (B) the sale of all or substantially all of the assets of Amicus to a third party that does not control, is not controlled by and is not under common control with Amicus;

(f) upon the filing of a preliminary or final proxy statement by any third party with respect to the commencement of a proxy or consent solicitation subject to Section 14 of the Exchange Act to elect or remove a majority of Amicus' Board of Directors; or

(g) upon the adoption of a plan of liquidation or dissolution with respect to Amicus.

8.3. Vote Along Agreement . \*\*\*\*\*.

8.4 Market Stand-Off Agreement . During the Standstill Term, GSK agrees that in connection with any registration of Amicus's securities that, upon the request of Amicus or the underwriters managing any underwritten offering of Amicus's securities, not to sell, make any short sale of, loan, grant any option for the purchase of, or otherwise dispose of any Registrable Securities without the prior written consent of Amicus or such underwriters, as the case may be, for such period of time from the effective date of such registration as Amicus or the underwriters may specify, provided that each executive officer and director of Amicus agrees to a similar lockup.

8.5 Lock-Up Period . Excluding any transfers or intra-company disposal of Shares between GSK and any of its Affiliates, during the eighteen (18) month period beginning on the Closing Date and ending on the eighteen (18) month anniversary thereof (the "Lock-Up Period"), GSK shall not, and shall cause any other Holder not to, without the prior written consent of Amicus, sell, contract to sell, pledge or otherwise dispose of, directly or indirectly, any Shares or enter into a transaction which would have the same effect.

8.6 Remedies . Without prejudice to the rights and remedies otherwise available to the parties, Amicus shall be entitled to equitable relief by way of injunction if GSK or any other Holder breaches or threatens to breach any of the provisions of this Section 8.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

## 9. Covenants.

### 9.1. Covenant of Amicus.

(a) Amicus hereby covenants and agrees that it shall take all necessary and appropriate actions to ensure that it shall have available under its Restated Certificate of Incorporation as in effect on the Closing Date sufficient authorized but unissued shares of its Common Stock to issue and sell to GSK all of the Shares.

(b) Amicus will file with Nasdaq all documentation required by Nasdaq, if any, in connection with the issuance of the Shares.

9.2. Notice of Market Sale. GSK will notify Amicus prior to selling the Shares, and will use commercially reasonable efforts to ensure that the Shares are disposed of in an orderly fashion in compliance with the volume and manner of sale restrictions imposed pursuant to Rule 144. Notwithstanding the foregoing, GSK shall have no obligations to Amicus under this Section 9.2 in respect of a private sale by GSK of the Shares.

## 10. Termination.

10.1. Termination. This Agreement may be terminated at any time prior to the Closing:

(a) by mutual written consent of GSK and Amicus;

(b) by GSK or Amicus:

(i) if there shall be any statute, law, regulation or rule that makes consummating the transactions contemplated hereby illegal or if any court or other Governmental Entity of competent jurisdiction shall have issued judgment, order, decree or ruling, or shall have taken such other action restraining, enjoining or otherwise prohibiting the consummation of the transactions contemplated hereby and such judgment, order, decree or ruling shall have become final and non-appealable;

(ii) if the License and Collaboration Agreement shall have terminated; or

(iii) the United States Federal Trade Commission (“FTC”) and/or the United States Department of Justice shall seek a preliminary injunction under the HSR Act against Amicus and GSK to enjoin the transactions contemplated by this Agreement or the License and Collaboration Agreement; or

(c) by GSK:

(i) if Amicus shall have (A) failed to perform any of its material obligations contained herein, or (B) breached any of its material representations or warranties contained herein, provided that GSK gives Amicus written notice of such failure to perform or breach and Amicus does not cure such failure to perform or breach within thirty (30) days after its receipt of such written notice;

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

(ii) if any of the conditions set forth in Sections 6 shall become impossible to fulfill (other than as a result of any breach by GSK of the terms of this Agreement) and shall not have been waived in accordance with the terms of this Agreement; or

(iii) if the Common Stock shall no longer be listed for trading on the Nasdaq National Market or other national securities exchange or automated quotation system.

(d) by Amicus:

(i) if GSK shall have (A) failed to perform any of its material obligations contained herein, or (B) breached any of its material representations or warranties contained herein, provided that Amicus gives GSK written notice of such failure to perform or breach and GSK does not cure such failure to perform or breach within thirty (30) days after its receipt of such written notice; or

(ii) if any of the conditions set forth in Section 6 shall become impossible to fulfill (other than as a result of any breach by Amicus of the terms of this Agreement) and shall not have been waived in accordance with the terms of this Agreement.

10.2. Effect of Termination. In the event of termination of this Agreement by either GSK or Amicus as provided in Section 10.1, this Agreement shall forthwith become void and have no effect, without any liability or obligation on the part of GSK or Amicus, other than the provisions of this Section 10.2, and except to the extent that such termination results from a material breach by a party of its representations, warranties, covenants or agreements set forth in this Agreement.

## 11. Miscellaneous.

11.1. Governing Law. This Agreement shall be governed by and construed in accordance with the laws of the Commonwealth of Delaware, without regard to the choice of law provisions thereof, and the federal laws of the United States.

11.2. Public Statements. Any statement to the public regarding this Agreement shall be approved in advance by Amicus and GSK, except as otherwise required by law, regulation or legal process. Notwithstanding the foregoing, GSK acknowledges that Amicus shall file this Agreement with the SEC.

11.3. Successors and Assigns. Except as otherwise expressly provided herein, the respective rights and obligations of either Party under this Agreement shall not be assignable in whole or in part by a Party without the prior written consent of the other Party, which consent shall not be unreasonably withheld or delayed. Notwithstanding the preceding sentence, in connection with the merger, acquisition, transfer of all or substantially all of a Party's assets or other change in control of either Party, such Party may assign its rights and obligations under this Agreement in whole or in part to such Party's transferee or successor in interest without the prior written consent of the other Party. This Agreement shall bind and inure to the benefit of Parties and their permitted successors and assigns.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

11.4. Entire Agreement . This Agreement, the License and Collaboration Agreement and the exhibits thereto, and that certain Confidential Disclosure Agreement dated as of March 29, 2006 and amended as of December 28, 2006 between the Amicus and GSK and the other documents delivered pursuant hereto, constitute the full and entire understanding and agreement among the Parties with regard to the subjects hereof and no Party shall be liable or bound to any other Party in any manner by any representations, warranties, covenants, or agreements except as specifically set forth herein or therein. Nothing in this Agreement, express or implied, is intended to confer upon any Party, other than the parties hereto and their respective successors and assigns, any rights, remedies, obligations, or liabilities under or by reason of this Agreement, except as expressly provided herein.

11.5. Separability . In the event any provision of this Agreement shall be invalid, illegal, or unenforceable, it shall to the extent practicable, be modified so as to make it valid, legal and enforceable and to retain as nearly as practicable the intent of the Parties, and the validity, legality, and enforceability of the remaining provisions shall not in any way be affected or impaired thereby.

11.6. Amendment and Waiver . Except as otherwise provided herein, any term of this Agreement may be amended and the observance of any term of this Agreement may be waived (either generally or in a particular instance, either retroactively or prospectively, and either for a specified period of time or indefinitely), with the written consent of Amicus and GSK. Any amendment or waiver effected in accordance with this Section shall be binding upon any holder of any securities purchased under this Agreement (including securities into which such securities have been converted), each future holder of all such securities, and Amicus.

11.7. Notices . All notices, requests, or other communications given hereunder shall be in writing and shall be deemed to have been duly given if (a) delivered by hand; (b) mailed by registered or certified mail; (c) sent by air courier; or (d) sent by cable, telex or facsimile, followed within twenty-four (24) hours by notification pursuant to (a), (b) or (c) above, in each case to the address set forth below or to such other address as a Party may specify for itself by written notice given as aforesaid.

If to GSK:                      Glaxo Group Limited  
   Great West Road  
   Brentford, Middlesex  
   United Kingdom  
   TW8 9GS  
   Facsimile: +44 (020) 804 76904  
   Attention: Company Secretary

With a copy to:                GlaxoSmithKline  
   980 Great West Road  
   Brentford, Middlesex, TW8 9GS  
   UK Facsimile: +44 (020) 804 70641  
   Attention : Marc Dunoyer  
   President, GSK Rare Diseases

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

And

Glaxo Smith Kline  
2301 Renaissance Boulevard  
Mail Code RN0220  
King of Prussia, PA 19406  
Facsimile: (610) 787-7084  
Attention: Vice President and Associate  
General Counsel, Legal Operations —  
Business Development Transactions

If to Amicus:

Amicus Therapeutics, Inc.  
6 Cedar Brook Drive  
Cranbury, NJ 08512  
Facsimile: 609-662-2001  
Attention: Senior VP, Business Operations

with a copy to:

Amicus Therapeutics, Inc.  
6 Cedar Brook Drive  
Cranbury, NJ 08512  
Facsimile: 609-662-2001  
Attention: Senior VP & General Counsel

11.8. Fees And Expenses. Amicus and GSK shall each bear their own expenses and legal fees incurred on their behalf with respect to this Agreement and the transactions contemplated hereby.

11.9. Titles and Subtitles. The titles of the Sections and subsections of this Agreement are for convenience of reference only and are not to be considered in construing this Agreement.

11.10. Counterparts; Effectiveness. This Agreement may be executed in any number of counterparts, each of which shall be deemed an original, but all of which together shall constitute one instrument. This Agreement shall become effective when each party hereto shall have received counterparts thereof signed and delivered (by telecopy or other electronic means) by the other party hereto.

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

IN WITNESS WHEREOF, this Stock Purchase Agreement is hereby executed as of the date first above written.

**AMICUS THERAPEUTICS, INC.**

By: /s/ John F. Crowley  
Name: John F. Crowley  
Title: Chairman and CEO

**GLAXO GROUP LIMITED**

By: /s/ Paul Williamson  
Name: Paul Williamson  
Title: Corporate Director

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.

**Schedule A**

**Expected Amicus Capitalization Table After Closing Date**

Common shares issued & outstanding	34,503,739
Common shares to be issued on exercise of warrants	<u>1,854,946</u>
Total common shares & equivalents outstanding	36,358,685
Total stock options issued & outstanding	5,142,790(a)
Total stock options reserved for future issuance	1,858,942(b)
Preferred shares issued & outstanding	<u>—</u> (c)
<b>Grand total</b>	<b><u>43,360,417</u></b>

(a) weighted average exercise price = \$2.06 & standard vesting term = 4 years

(b) 4,946,524 warrants outstanding at exercise rate = 0.375

(c) 10,000,000 shares authorized

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\*\*\*\*\* - Material has been omitted and filed separately with the Commission.



**LETTER AGREEMENT**

Ken Valenzano  
337 New Brunswick Avenue  
East Brunswick, NJ 08816

**Re: Severance and Change in Control Agreements**

Dear Ken:

On behalf of Amicus Therapeutics, Inc., (the "Company"), this Letter Agreement, dated as of May 10, 2010, shall serve to confirm our agreement in the event Amicus terminates your employment without cause or in the event of a Change in Control, Sale or Merger of the Company. By accepting the terms of this Letter Agreement, you agree that the rights identified in this Letter Agreement contain the complete understanding between you and the Company related to Severance and Change in Control payments and supersedes and replaces all previous agreements related to such payments. The May 11, 2005 Offer of Employment Letter countersigned by you and attached hereto shall otherwise remain in full force and effect and is hereby confirmed and ratified.

**Severance Pay**

In the event that your employment is terminated by the Company, except for "Cause" as defined below, you will be eligible to receive the following:

1. six (6) months salary continuation to be paid in accordance with the Company's payroll practices;
2. an additional six (6) months of option vesting;
3. in the event that your termination occurs after June 30<sup>th</sup> of the calendar year, you will be entitled to a payment of a bonus equal to the bonus earned in the preceding year pro-rated for the number of months actually worked in the year of termination, payable on the date of termination; and
4. you will be entitled to a continuation of your health benefit coverage under COBRA, premiums to be paid by the Company, for a period of twelve (12) months, which shall commence on the date of termination and run concurrently with the period of salary continuation.

6 Cedar Brook Drive Cranbury, NJ 08512 T: 609-662-2000 F: 609-662-2001 [www.amicustherapeutics.com](http://www.amicustherapeutics.com)

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For purposes of this Agreement, “Cause” means termination for any of the following reasons: (1) willful or deliberate misconduct by you that materially damages the Company; (2) misappropriation of Company assets; (3) conviction of, or a plea of guilty or “no contest” to, a felony; or (4) any willful disobedience of the lawful and unambiguous instructions of the CEO of the Company; provided that the CEO has given you written notice of such disobedience or neglect and you have failed to cure such disobedience or neglect within a period reasonable under the circumstances.

### **Change in Control**

If there is a Change in Control Event and either (i) you are terminated without Cause within twelve months of such Change in Control Event or (ii) a condition occurs which constitutes Good Reason within twelve months of such Change in Control Event and after you have complied with the applicable notice period and the Company has failed to remedy such condition, you actually resign (all as described in detail in the definition of “Good Reason” in the following paragraph), then (i) you will be entitled to receive twelve (12) months of salary continuation, to be paid in accordance with the Company’s payroll practices, plus, in the event that the resignation for Good Reason or termination without Cause following a change in control event occurs after June 30<sup>th</sup> of the calendar year, you will be entitled to a payment of a bonus equal to the bonus earned in the preceding year pro-rated for the number of months actually worked in the year of your resignation or termination, payable on the date of termination or resignation for Good Reason. In addition, you will be entitled to continuation of your health benefit coverage under COBRA, premiums to be paid by the Company, for a period of twelve (12) months, which shall commence on the date of resignation or termination and run concurrently with the period of salary continuation, and (ii) all unvested stock options will have their remaining vesting schedule accelerated so that all stock options are fully vested.

“Change in Control Event” means any of the following: (i) any person or entity (except for a current stockholder) becomes the beneficial owner of greater than 50% of the then outstanding voting power of the Company; (ii) a merger or consolidation with another entity where the voting securities of the Company outstanding immediately before the transaction constitute less than a majority of the voting power of the voting securities of the Company or the surviving entity outstanding immediately after the transaction, or (iii) the sales or disposition of all or substantially all of the Company’s assets. “Good Reason” means (i) a material diminution in your authorities, duties, or responsibilities, or (ii) a material change in the geographic location at which you must perform services; provided, however, that you must provide the Company with notice of the existence of the Good Reason condition within ninety (90) days of its initial existence after which the Company will have a period of thirty (30) day within which it may remedy the condition and not be required to pay the severance payment; and provided, further, that any Good Reason termination must occur within two (2) years of the initial existence of the Good Reason condition.

Your right to receive accelerated vesting and severance payments pursuant to this letter agreement shall be subject to the condition that you execute a full release and waiver of all claims against the Company and related parties, in a form acceptable to the Company; provided that such payments and benefits will be paid, if ever, only on the date specified as the deadline for signing and delivering the release, (the “Release Deadline”), even if your release becomes irrevocable (i.e., you sign and deliver the release to the Company) before that date. In the event the Release Deadline is more than thirty (30) days and you sign and deliver the release before the Release Deadline, the Company may elect to make such severance payments no earlier than thirty (30) days prior to the Release Deadline.

It is the intention of the parties that compensation paid or delivered to you by the Company either is paid in compliance with, or is exempt from, Section 409A of the Internal Revenue Code of 1986, as amended and the rules and regulations promulgated thereunder (collectively, “Section 409A”). However, the Company does not warrant to you that all compensation paid or delivered to you for your services will be exempt from, or paid in compliance with, Section 409A. Notwithstanding any other provisions of this Agreement, in the event that any payment or benefit under this Agreement received or to be received by you (the “Payment”) is determined to be subject (in whole or part) to the penalties imposed by Section 409A of the Code (the “Additional Taxes”), then you shall be entitled to receive an additional payment (a “Gross-Up Payment”) in an amount such that after payment by you of the Additional Taxes, you retain an amount equal to the Payment net of any applicable taxes and withholdings other than Additional Taxes. All determinations required to be made under this provision, including whether and when a Gross-Up Payment is required, the amount of such Gross-Up Payment and the assumptions to be utilized in arriving at such determination, shall be made by the Company’s accountants or such other certified public accounting firm designated by you and reasonably acceptable to the Company. Any certified public accounting firm chosen by you shall provide detailed supporting calculations both to the Company and you. Any Gross-Up Payment due under this paragraph shall be paid to you no later than December 31 of the calendar year following the calendar year in which you remit the Additional Taxes to the applicable authorities.

For the purposes of determining when amounts otherwise payable on account of your termination of employment will be paid, which amounts become due because of your termination of employment, “termination of employment” or words of similar import shall be construed as the date that you first incur a “separation from service” for purposes of Section 409A on or following termination of employment. Furthermore, if you are a “specified employee” of a public company as determined pursuant to Section 409A as of your termination of employment, any amounts payable on account of your termination of employment which constitute deferred compensation within the meaning of Section 409A and which are otherwise payable during the first six months following your termination (or prior to your death after termination) shall be paid to you in a cash lump-sum on the earlier of (1) the date of your death and (2) the first business day of the seventh calendar month immediately following the month in which your termination occurs.

In applying Section 409A to amounts paid pursuant to this letter, any right to a series of installment payments shall be treated as a right to a series of separate payments.

**Employment “At-Will”**

It is important that you understand that the Company does not guarantee employment for any specific period of time. You will continue to be employed on at “at-will” basis. This means that both the Company and you will have the right to terminate your employment at any time, for any reason, with or without prior notice or cause. Neither you nor the Company will have an express or implied contract limiting your right to resign or the Company’s right to terminate your employment at any time, for any reason, with or without prior notice or cause. The “at-will” relationship will apply to you throughout your employment and cannot be changed except by an express individual written employment agreement signed by you and the Chief Executive Officer of the Company.

It is understood and agreed that this Letter Agreement constitutes the full agreement between you and the Company on the subjects of Severance and Change in Control payments. By signing below, you agree that no other promises, express or implied, have been made to you either verbally or in writing and that no further modifications to these terms and conditions will be effective except by a written agreement signed by the Chief Executive Officer of the Company and you and as authorized by the Company’s Board of Directors or an authorized Committee thereof. This Letter Agreement may be executed in counterparts, each of which shall be deemed an original but all of which shall together constitute on and the same agreement.

[Signature Page Follows]

Amicus Therapeutics, Inc.

By: /s/ John F. Crowley  
John F. Crowley  
Chairman and Chief Executive Officer

**Accepted and Agreed:**

/s/ Ken Valenzano  
Ken Valenzano



**LETTER AGREEMENT**

Ken Peist  
7 Dustin Drive  
Lawrenceville, NJ 08648

**Re: Severance and Change in Control Agreements**

Dear Ken:

On behalf of Amicus Therapeutics, Inc., (the "Company"), this Letter Agreement, dated as of January 3, 2011, shall serve to confirm our agreement in the event Amicus terminates your employment without cause or in the event of a Change in Control, Sale or Merger of the Company. By accepting the terms of this Letter Agreement, you agree that the rights identified in this Letter Agreement contain the complete understanding between you and the Company related to Severance and Change in Control payments and supersedes and replaces all previous agreements related to such payments. The November 16, 2007 Offer of Employment Letter countersigned by you and attached hereto shall otherwise remain in full force and effect and is hereby confirmed and ratified.

**Severance Pay**

In the event that your employment is terminated by the Company, except for "Cause" as defined below, you will be eligible to receive the following:

1. six (6) months salary continuation to be paid in accordance with the Company's payroll practices;
2. an additional six (6) months of option vesting;
3. in the event that your termination occurs after June 30<sup>th</sup> of the calendar year, you will be entitled to a payment of a bonus equal to the bonus earned in the preceding year pro-rated for the number of months actually worked in the year of termination, payable on the date of termination; and
4. you will be entitled to a continuation of your health benefit coverage under COBRA, premiums to be paid by the Company, for a period of twelve (12) months, which shall commence on the date of termination and run concurrently with the period of salary continuation.

6 Cedar Brook Drive Cranbury, NJ 08512 T: 609-662-2000 F: 609-662-2001 [www.amicustherapeutics.com](http://www.amicustherapeutics.com)

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For purposes of this Agreement, “Cause” means termination for any of the following reasons: (1) willful or deliberate misconduct by you that materially damages the Company; (2) misappropriation of Company assets; (3) conviction of, or a plea of guilty or “no contest” to, a felony; or (4) any willful disobedience of the lawful and unambiguous instructions of the CEO of the Company; provided that the CEO has given you written notice of such disobedience or neglect and you have failed to cure such disobedience or neglect within a period reasonable under the circumstances.

### **Change in Control**

If there is a Change in Control Event and either (i) you are terminated without Cause within twelve months of such Change in Control Event or (ii) a condition occurs which constitutes Good Reason within twelve months of such Change in Control Event and after you have complied with the applicable notice period and the Company has failed to remedy such condition, you actually resign (all as described in detail in the definition of “Good Reason” in the following paragraph), then (i) you will be entitled to receive twelve (12) months of salary continuation, to be paid in accordance with the Company’s payroll practices, plus, in the event that the resignation for Good Reason or termination without Cause following a change in control event occurs after June 30<sup>th</sup> of the calendar year, you will be entitled to a payment of a bonus equal to the bonus earned in the preceding year pro-rated for the number of months actually worked in the year of your resignation or termination, payable on the date of termination or resignation for Good Reason. In addition, you will be entitled to continuation of your health benefit coverage under COBRA, premiums to be paid by the Company, for a period of twelve (12) months, which shall commence on the date of resignation or termination and run concurrently with the period of salary continuation, and (ii) all unvested stock options will have their remaining vesting schedule accelerated so that all stock options are fully vested.

“Change in Control Event” means any of the following: (i) any person or entity (except for a current stockholder) becomes the beneficial owner of greater than 50% of the then outstanding voting power of the Company; (ii) a merger or consolidation with another entity where the voting securities of the Company outstanding immediately before the transaction constitute less than a majority of the voting power of the voting securities of the Company or the surviving entity outstanding immediately after the transaction, or (iii) the sales or disposition of all or substantially all of the Company’s assets. “Good Reason” means (i) a material diminution in your authorities, duties, or responsibilities, or (ii) a material change in the geographic location at which you must perform services; provided, however, that you must provide the Company with notice of the existence of the Good Reason condition within ninety (90) days of its initial existence after which the Company will have a period of thirty (30) day within which it may remedy the condition and not be required to pay the severance payment; and provided, further, that any Good Reason termination must occur within two (2) years of the initial existence of the Good Reason condition.

Your right to receive accelerated vesting and severance payments pursuant to this letter agreement shall be subject to the condition that you execute a full release and waiver of all claims against the Company and related parties, in a form acceptable to the Company; provided that such payments and benefits will be paid, if ever, only on the date specified as the deadline for signing and delivering the release, (the “Release Deadline”), even if your release becomes irrevocable (i.e., you sign and deliver the release to the Company) before that date. In the event the Release Deadline is more than thirty (30) days and you sign and deliver the release before the Release Deadline, the Company may elect to make such severance payments no earlier than thirty (30) days prior to the Release Deadline.

It is the intention of the parties that compensation paid or delivered to you by the Company either is paid in compliance with, or is exempt from, Section 409A of the Internal Revenue Code of 1986, as amended and the rules and regulations promulgated thereunder (collectively, “Section 409A”). However, the Company does not warrant to you that all compensation paid or delivered to you for your services will be exempt from, or paid in compliance with, Section 409A. Notwithstanding any other provisions of this Agreement, in the event that any payment or benefit under this Agreement received or to be received by you (the “Payment”) is determined to be subject (in whole or part) to the penalties imposed by Section 409A of the Code (the “Additional Taxes”), then you shall be entitled to receive an additional payment (a “Gross-Up Payment”) in an amount such that after payment by you of the Additional Taxes, you retain an amount equal to the Payment net of any applicable taxes and withholdings other than Additional Taxes. All determinations required to be made under this provision, including whether and when a Gross-Up Payment is required, the amount of such Gross-Up Payment and the assumptions to be utilized in arriving at such determination, shall be made by the Company’s accountants or such other certified public accounting firm designated by you and reasonably acceptable to the Company. Any certified public accounting firm chosen by you shall provide detailed supporting calculations both to the Company and you. Any Gross-Up Payment due under this paragraph shall be paid to you no later than December 31 of the calendar year following the calendar year in which you remit the Additional Taxes to the applicable authorities.

For the purposes of determining when amounts otherwise payable on account of your termination of employment will be paid, which amounts become due because of your termination of employment, “termination of employment” or words of similar import shall be construed as the date that you first incur a “separation from service” for purposes of Section 409A on or following termination of employment. Furthermore, if you are a “specified employee” of a public company as determined pursuant to Section 409A as of your termination of employment, any amounts payable on account of your termination of employment which constitute deferred compensation within the meaning of Section 409A and which are otherwise payable during the first six months following your termination (or prior to your death after termination) shall be paid to you in a cash lump-sum on the earlier of (1) the date of your death and (2) the first business day of the seventh calendar month immediately following the month in which your termination occurs.

In applying Section 409A to amounts paid pursuant to this letter, any right to a series of installment payments shall be treated as a right to a series of separate payments.

**Employment “At-Will”**

It is important that you understand that the Company does not guarantee employment for any specific period of time. You will continue to be employed on at “at-will” basis. This means that both the Company and you will have the right to terminate your employment at any time, for any reason, with or without prior notice or cause. Neither you nor the Company will have an express or implied contract limiting your right to resign or the Company’s right to terminate your employment at any time, for any reason, with or without prior notice or cause. The “at-will” relationship will apply to you throughout your employment and cannot be changed except by an express individual written employment agreement signed by you and the Chief Executive Officer of the Company.

It is understood and agreed that this Letter Agreement constitutes the full agreement between you and the Company on the subjects of Severance and Change in Control payments. By signing below, you agree that no other promises, express or implied, have been made to you either verbally or in writing and that no further modifications to these terms and conditions will be effective except by a written agreement signed by the Chief Executive Officer of the Company and you and as authorized by the Company’s Board of Directors or an authorized Committee thereof. This Letter Agreement may be executed in counterparts, each of which shall be deemed an original but all of which shall together constitute on and the same agreement.

[Signature Page Follows]

Amicus Therapeutics, Inc.

By: /s/ John F. Crowley  
John F. Crowley  
Chairman and Chief Executive Officer

**Accepted and Agreed:**

/s/ Kenneth Peist  
Kenneth Peist



**LETTER AGREEMENT**

Enrique Dilone  
52 Liberty Ridge Rd  
Basking Ridge, NJ 07920

**Re: Severance and Change in Control Agreements**

Dear Enrique:

On behalf of Amicus Therapeutics, Inc., (the "Company"), this Letter Agreement, dated as of January 3, 2011, shall serve to confirm our agreement in the event Amicus terminates your employment without cause or in the event of a Change in Control, Sale or Merger of the Company. By accepting the terms of this Letter Agreement, you agree that the rights identified in this Letter Agreement contain the complete understanding between you and the Company related to Severance and Change in Control payments and supersedes and replaces all previous agreements related to such payments. The July 27, 2009 Offer of Employment Letter countersigned by you and attached hereto shall otherwise remain in full force and effect and is hereby confirmed and ratified.

**Severance Pay**

In the event that your employment is terminated by the Company, except for "Cause" as defined below, you will be eligible to receive the following:

1. six (6) months salary continuation to be paid in accordance with the Company's payroll practices;
2. an additional six (6) months of option vesting;
3. in the event that your termination occurs after June 30<sup>th</sup> of the calendar year, you will be entitled to a payment of a bonus equal to the bonus earned in the preceding year pro-rated for the number of months actually worked in the year of termination, payable on the date of termination; and
4. you will be entitled to a continuation of your health benefit coverage under COBRA, premiums to be paid by the Company, for a period of twelve (12) months, which shall commence on the date of termination and run concurrently with the period of salary continuation.

6 Cedar Brook Drive Cranbury, NJ 08512 T: 609-662-2000 F: 609-662-2001 [www.amicustherapeutics.com](http://www.amicustherapeutics.com)

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For purposes of this Agreement, “Cause” means termination for any of the following reasons: (1) willful or deliberate misconduct by you that materially damages the Company; (2) misappropriation of Company assets; (3) conviction of, or a plea of guilty or “no contest” to, a felony; or (4) any willful disobedience of the lawful and unambiguous instructions of the CEO of the Company; provided that the CEO has given you written notice of such disobedience or neglect and you have failed to cure such disobedience or neglect within a period reasonable under the circumstances.

### **Change in Control**

If there is a Change in Control Event and either (i) you are terminated without Cause within twelve months of such Change in Control Event or (ii) a condition occurs which constitutes Good Reason within twelve months of such Change in Control Event and after you have complied with the applicable notice period and the Company has failed to remedy such condition, you actually resign (all as described in detail in the definition of “Good Reason” in the following paragraph), then (i) you will be entitled to receive twelve (12) months of salary continuation, to be paid in accordance with the Company’s payroll practices, plus, in the event that the resignation for Good Reason or termination without Cause following a change in control event occurs after June 30<sup>th</sup> of the calendar year, you will be entitled to a payment of a bonus equal to the bonus earned in the preceding year pro-rated for the number of months actually worked in the year of your resignation or termination, payable on the date of termination or resignation for Good Reason. In addition, you will be entitled to continuation of your health benefit coverage under COBRA, premiums to be paid by the Company, for a period of twelve (12) months, which shall commence on the date of resignation or termination and run concurrently with the period of salary continuation, and (ii) all unvested stock options will have their remaining vesting schedule accelerated so that all stock options are fully vested.

“Change in Control Event” means any of the following: (i) any person or entity (except for a current stockholder) becomes the beneficial owner of greater than 50% of the then outstanding voting power of the Company; (ii) a merger or consolidation with another entity where the voting securities of the Company outstanding immediately before the transaction constitute less than a majority of the voting power of the voting securities of the Company or the surviving entity outstanding immediately after the transaction, or (iii) the sales or disposition of all or substantially all of the Company’s assets. “Good Reason” means (i) a material diminution in your authorities, duties, or responsibilities, or (ii) a material change in the geographic location at which you must perform services; provided, however, that you must provide the Company with notice of the existence of the Good Reason condition within ninety (90) days of its initial existence after which the Company will have a period of thirty (30) day within which it may remedy the condition and not be required to pay the severance payment; and provided, further, that any Good Reason termination must occur within two (2) years of the initial existence of the Good Reason condition.

Your right to receive accelerated vesting and severance payments pursuant to this letter agreement shall be subject to the condition that you execute a full release and waiver of all claims against the Company and related parties, in a form acceptable to the Company; provided that such payments and benefits will be paid, if ever, only on the date specified as the deadline for signing and delivering the release, (the “Release Deadline”), even if your release becomes irrevocable (i.e., you sign and deliver the release to the Company) before that date. In the event the Release Deadline is more than thirty (30) days and you sign and deliver the release before the Release Deadline, the Company may elect to make such severance payments no earlier than thirty (30) days prior to the Release Deadline.

It is the intention of the parties that compensation paid or delivered to you by the Company either is paid in compliance with, or is exempt from, Section 409A of the Internal Revenue Code of 1986, as amended and the rules and regulations promulgated thereunder (collectively, “Section 409A”). However, the Company does not warrant to you that all compensation paid or delivered to you for your services will be exempt from, or paid in compliance with, Section 409A. Notwithstanding any other provisions of this Agreement, in the event that any payment or benefit under this Agreement received or to be received by you (the “Payment”) is determined to be subject (in whole or part) to the penalties imposed by Section 409A of the Code (the “Additional Taxes”), then you shall be entitled to receive an additional payment (a “Gross-Up Payment”) in an amount such that after payment by you of the Additional Taxes, you retain an amount equal to the Payment net of any applicable taxes and withholdings other than Additional Taxes. All determinations required to be made under this provision, including whether and when a Gross-Up Payment is required, the amount of such Gross-Up Payment and the assumptions to be utilized in arriving at such determination, shall be made by the Company’s accountants or such other certified public accounting firm designated by you and reasonably acceptable to the Company. Any certified public accounting firm chosen by you shall provide detailed supporting calculations both to the Company and you. Any Gross-Up Payment due under this paragraph shall be paid to you no later than December 31 of the calendar year following the calendar year in which you remit the Additional Taxes to the applicable authorities.

For the purposes of determining when amounts otherwise payable on account of your termination of employment will be paid, which amounts become due because of your termination of employment, “termination of employment” or words of similar import shall be construed as the date that you first incur a “separation from service” for purposes of Section 409A on or following termination of employment. Furthermore, if you are a “specified employee” of a public company as determined pursuant to Section 409A as of your termination of employment, any amounts payable on account of your termination of employment which constitute deferred compensation within the meaning of Section 409A and which are otherwise payable during the first six months following your termination (or prior to your death after termination) shall be paid to you in a cash lump-sum on the earlier of (1) the date of your death and (2) the first business day of the seventh calendar month immediately following the month in which your termination occurs.

In applying Section 409A to amounts paid pursuant to this letter, any right to a series of installment payments shall be treated as a right to a series of separate payments.

**Employment “At-Will”**

It is important that you understand that the Company does not guarantee employment for any specific period of time. You will continue to be employed on at “at-will” basis. This means that both the Company and you will have the right to terminate your employment at any time, for any reason, with or without prior notice or cause. Neither you nor the Company will have an express or implied contract limiting your right to resign or the Company’s right to terminate your employment at any time, for any reason, with or without prior notice or cause. The “at-will” relationship will apply to you throughout your employment and cannot be changed except by an express individual written employment agreement signed by you and the Chief Executive Officer of the Company.

It is understood and agreed that this Letter Agreement constitutes the full agreement between you and the Company on the subjects of Severance and Change in Control payments. By signing below, you agree that no other promises, express or implied, have been made to you either verbally or in writing and that no further modifications to these terms and conditions will be effective except by a written agreement signed by the Chief Executive Officer of the Company and you and as authorized by the Company’s Board of Directors or an authorized Committee thereof. This Letter Agreement may be executed in counterparts, each of which shall be deemed an original but all of which shall together constitute on and the same agreement.

[Signature Page Follows]

Amicus Therapeutics, Inc.

By: /s/ John F. Crowley  
John F. Crowley  
Chairman and Chief Executive Officer

**Accepted and Agreed:**

/s/ Enrique Dilone  
Enrique Dilone

**Consent of Independent Registered Public Accounting Firm**

We consent to the incorporation by reference in the Registration Statement (Form S-8 No. 333-145305) pertaining to the: 1) Amicus Therapeutics, Inc. 2002 Equity Incentive Plan, as Amended, 2) Amicus Therapeutics, Inc. 2007 Equity Incentive Plan, 3) Amicus Therapeutics, Inc. 2007 Director Option Plan, 4) Amicus Therapeutics, Inc. 2007 Employee Stock Purchase Plan, and in the Registration Statement (Form S-8 No. 333-157219) pertaining to the: 1) Amicus Therapeutics, Inc. Amended and Restated 2007 Equity Incentive Plan and 2) Amicus Therapeutics, Inc. 2007 Director Option Plan, and in the Registration Statement (Form S-3 No. 333-158405) of our reports dated March 4, 2011, with respect to the consolidated financial statements of Amicus Therapeutics Inc. included in the Annual Report (Form 10-K) for the year ended December 31, 2010.

/s/ ERNST & YOUNG LLP

MetroPark, New Jersey  
March 4, 2011

**CERTIFICATIONS PURSUANT TO SECTION 302 OF  
THE SARBANES-OXLEY ACT OF 2002  
CERTIFICATION BY PRINCIPAL EXECUTIVE OFFICER**

I, John F. Crowley, certify that:

1. I have reviewed this annual report on Form 10-K of Amicus Therapeutics, Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
  - a. designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this annual report is being prepared;
  - b. designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
  - c. evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
  - d. disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting;
5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
  - a. all significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
  - b. any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: March 4, 2011

/s/ John F. Crowley

John F. Crowley  
Chairman and Chief Executive Officer

**CERTIFICATIONS PURSUANT TO SECTION 302 OF  
THE SARBANES-OXLEY ACT OF 2002  
CERTIFICATION BY PRINCIPAL FINANCIAL OFFICER**

I, Daphne Quimi, certify that:

1. I have reviewed this annual report on Form 10-K of Amicus Therapeutics, Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
  - a. designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this annual report is being prepared;
  - b. designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
  - c. evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
  - d. disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting;
5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
  - a. all significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
  - b. any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: March 4, 2011

/s/ Daphne Quimi

Daphne Quimi  
Corporate Controller

**Certification by the Principal Executive Officer Pursuant to 18 U. S. C. Section 1350, as  
Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002**

Pursuant to 18 U. S. C. Section 1350, I, John F. Crowley, hereby certify that, to the best of my knowledge, Amicus Therapeutics Inc., (the "Company") Annual Report on Form 10-K for the year ended December 31, 2010 (the "Report"), as filed with the Securities and Exchange Commission on March 4, 2011, fully complies with the requirements of Section 13(a) or Section 15(d) of the Securities Exchange Act of 1934, as amended, and that the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

/s/ John F. Crowley

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John F. Crowley  
Chairman and Chief Executive Officer  
March 4, 2011

**Certification by the Principal Financial Officer Pursuant to 18 U. S. C. Section 1350, as  
Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002**

Pursuant to 18 U. S. C. Section 1350, I, Daphne Quimi, hereby certify that, to the best of my knowledge, the Amicus Therapeutics Inc. (the "Company") Annual Report on Form 10-K for the year ended December 31, 2010 (the "Report"), as filed with the Securities and Exchange Commission on March 4, 2011, fully complies with the requirements of Section 13(a) or Section 15(d) of the Securities Exchange Act of 1934, as amended, and that the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

/s/ Daphne Quimi

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Daphne Quimi  
Corporate Controller  
March 4, 2011