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

FORM 10-K

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

For the fiscal year ended December 31, 2007

Commission File Number 001-33497

Amicus Therapeutics, Inc.

(Exact name of registrant as specified in its charter)

Delaware	**20-0422823**
(State or other jurisdiction of incorporation or organization)	**(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	Name of each exchange on which registered
Common Stock, par value $0.01 per share	**The NASDAQ Stock Market LLC**

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

Indicate by check mark if the registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act. Yes o **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 o **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** o

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. o

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

Large accelerated filer o **Accelerated filer** o **Non-accelerated filer** ☑

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

The aggregate market value of the 8,713,683 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 29, 2007) was approximately $100,207,355. Shares of voting and non-voting stock held by executive officers, directors and holders of more than 5% 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 January 31, 2008, there were 22,431,817 shares of common stock outstanding.

DOCUMENTS INCORPORATED BY REFERENCE: Portions of the Proxy Statement for the registrant's 2008 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 prospectus include, among other things, statements about:

- our plans to develop and commercialize Amigal, Plicera and AT2220;
- our ongoing and planned discovery programs, preclinical studies and clinical trials;
- our ability to enter into selective collaboration arrangements;
- the timing of and our ability to obtain and maintain regulatory approvals for our product candidates;
- the rate and degree of market acceptance and clinical utility of our products;
- our ability to quickly and efficiently identify and develop product candidates;
- the extent to which our scientific approach may potentially address a broad range of diseases across multiple therapeutic areas;
- our commercialization, marketing and manufacturing capabilities and strategy;
- our intellectual property position; and
- our estimates regarding expenses, future revenues, capital requirements and needs for additional financing.

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 clinical-stage biopharmaceutical company focused on the discovery, development and commercialization of a new class of orally-administered, small molecule drugs, known as pharmacological chaperones, for the treatment of a range of human genetic diseases. Our lead products in development are Amigal (migalastat hydrochloride) for Fabry disease, Plicera (isofagomine tartrate) for Gaucher disease and AT2220 for Pompe disease. We completed our Phase 2 clinical trials of Amigal, are currently conducting Phase 2 clinical trials of Plicera and completed Phase 1 clinical trials of AT2220. Fabry, Gaucher and Pompe are relatively rare disorders but represent substantial commercial markets due to the severity of the symptoms and the chronic nature of the diseases. The worldwide net product sales for the five currently approved therapeutics to treat Fabry, Gaucher and Pompe disease were approximately $1.9 billion in 2007, as publicly reported by the companies that market these therapeutics.

Certain human diseases result from 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 proteins. Misfolded 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, thereby increasing protein activity, improving cellular function and potentially reducing cell stress.

The current standard of treatment for Fabry, Gaucher and Pompe is enzyme replacement therapy. This therapy compensates for the reduced level of activity of specialized proteins called enzymes through regular infusions of recombinant enzyme. Instead of adding enzyme from an external source by intravenous infusion, our approach uses small molecule, orally-administered pharmacological chaperones to restore the function of the enzyme that is already made by the patient’s own body. We believe our product candidates may have advantages relative to enzyme replacement therapy relating to bio-distribution and ease of use, potentially improving treatment of these diseases.

Our goal is to become a leading biopharmaceutical company focused on the discovery, development and commercialization of pharmacological chaperone therapies for the treatment of a wide range of human diseases. Our initial clinical efforts are currently focused on developing pharmacological chaperones for the treatment of lysosomal storage disorders, which are chronic genetic diseases, such as Fabry, Gaucher and Pompe that frequently result in severe symptoms. We believe our technology also is broadly applicable to other diseases for which protein stabilization and improved folding may be beneficial, including certain types of neurological disease, metabolic disease, cardiovascular disease and cancer.

In November 2007, we entered into a strategic collaboration with Shire Pharmaceuticals Ireland Ltd. (Shire), a subsidiary of Shire plc, to jointly develop our three lead pharmacological chaperone compounds for lysosomal storage disorders. Shire will receive rights to commercialize these products outside of the United States (U.S.). We retain all rights to commercialize these products in the U.S.

Our Lead Programs

Our three most advanced product development programs target lysosomal storage disorders. Each of these disorders results from the deficiency of a single enzyme.

- *Amigal for Fabry disease.* We are developing Amigal for the treatment of Fabry disease and have completed four Phase 2 clinical trials. Based on the results of these Phase 2 clinical trials, we plan to meet with U.S. and European regulatory authorities to discuss the design of a Phase 3 clinical trial.
- *Plicera for Gaucher disease.* We are developing Plicera for the treatment of Gaucher disease and are currently conducting two Phase 2 clinical trials of Plicera in Type I Gaucher patients. The first of these trials has completed enrollment and we expect to receive data to be presented in the first half of 2008. The second of these trials is currently underway.

- *AT2220 for Pompe disease.* We are developing AT2220 for the treatment of Pompe disease, and have completed three Phase 1 clinical trials. We plan to initiate a Phase 2 clinical trial in 2008.

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 which reduce its stability and may prevent it 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 error, missense mutations often result in proteins that have a reduced level of biological activity. In addition to missense mutations, there are also other types of mutations that can result in proteins with reduced 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 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.

At Amicus, we have developed a novel approach to address human genetic diseases. We use small molecule drugs, which are called pharmacological chaperones, to selectively bind to a target protein and increase its stability. The binding of the chaperone molecule helps the protein fold into its correct three-dimensional shape. This allows the protein to be trafficked from the ER to the appropriate location in the cell, thereby increasing protein activity, improving cellular function and potentially reducing cell stress.

Pharmacological chaperones represent a new way of increasing the levels of specific proteins to improve cellular function and treat disease. Our proprietary approach to the discovery of pharmacological chaperone drug candidates involves the use of rapid molecular and cell-based screening methods combined with our understanding of the intended biological function of proteins implicated in disease. We use this knowledge to select and develop compounds with desirable properties. In many cases, we are able to start with specific molecules and classes of compounds already known to interact with the target protein but not used previously as therapies. This can greatly reduce the time and cost of the early stages of drug discovery and development.

We believe our technology is broadly applicable to other diseases for which protein stabilization and improved folding may be beneficial, including certain types of neurological disease, metabolic disease, cardiovascular disease and cancer. We are also exploring other applications in which the ability of pharmacological chaperones to increase the activity of normal proteins may provide a therapeutic benefit.

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

To date, we have 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.

The current therapeutic standard of care for the most common lysosomal storage disorders is enzyme replacement therapy. Enzyme replacement therapy involves regular infusions of recombinant human enzyme to compensate for the deficient lysosomal enzyme. We believe that pharmacological chaperone therapy may have advantages relative to enzyme replacement therapy for the treatment of lysosomal storage disorders.

The following table compares some features of enzyme replacement therapy to pharmacological chaperone therapy.

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

An additional therapeutic approach to the treatment of certain lysosomal storage disorders is called substrate reduction therapy. We believe our pharmacological chaperone therapies may have advantages relative to substrate reduction therapy. Substrate reduction therapy uses orally-administered small molecules; however, the underlying mechanism of action is very different than for pharmacological chaperones. 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. This 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, 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.

Our Lead Product Candidates

The following table summarizes key information about our product candidates. All of our current product candidates are orally-administered, small molecules based on our pharmacological chaperone technology.

Product Candidate	Indication	Stage of Development	Worldwide Commercial Rights
Amigal (migalastat hydrochloride)	Fabry Disease	Phase 2 Complete	Amicus U.S., Shire ex-U.S.
Plicera **(**isofagomine tartrate**)**	Gaucher Disease	Phase 2 Ongoing	Amicus U.S., Shire ex-U.S.
AT2220	Pompe Disease	Phase 1 Complete	Amicus U.S., Shire ex-U.S.

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. As of November 2007, we are developing Amigal in partnership with Shire.

We completed four Phase 2 clinical trials of Amigal in 2007. The primary objective of the Phase 2 trials was to evaluate the safety and tolerability of treatment with Amigal. The secondary objective was to evaluate certain pharmacodynamic measures of treatment, including effects on α -GAL (the target enzyme deficient in Fabry patients) and levels of GL-3 (the substrate that builds up in the cells of patients) in cells and tissues affected by the disease. An additional objective was the preliminary assessment of cardiac and renal function. The four open-label, multi-national Phase 2 trials of Amigal enrolled 18 men and 9 women with Fabry disease between the ages of 17 and 65. The four studies examined various dose levels and frequencies of Amigal administration and had 12 or 24 week primary treatment arms with an optional treatment extension.

Twenty-six patients completed the primary treatment arms and all entered the optional treatment extension. The 26 patients had 21 different missense genetic mutations that cause Fabry disease. The mutations represented the full spectrum of Fabry patients, including those with both early-onset and late-onset forms of the disease. Twenty-three patients are currently being treated with Amigal under the treatment extension, including 8 who have been treated for more than a year and 4 who have been treated for almost 2 years.

The key findings in the Phase 2 studies were:

- Amigal was generally safe and well-tolerated at all doses evaluated and no drug-related serious adverse events were reported.
- Amigal increased the level of the enzyme deficient in Fabry patients in 24 of 26 study subjects.
- Amigal was shown to reduce the accumulated substrate in a majority of study subjects.
- Renal and cardiac function results were encouraging, including those seen in patients treated for nearly two years.
- 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.
- Twenty-three patients have elected to continue Amigal treatment in an extension protocol.

Based on the results of these Phase 2 clinical trials, Amicus and Shire plan to meet with U.S. and European regulatory authorities to discuss the design of a Phase 3 clinical trial for Amigal. In February 2004, the U.S. Food and Drug Administration (FDA) granted orphan drug designation to Amigal for the treatment of Fabry disease and in March 2006, the European Medicines Agency (EMEA), recommended 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 α-GAL. Symptoms can be severe and debilitating, including kidney failure and increased risk of heart attack and stroke. The deficiency of α-GAL in Fabry patients is caused by inherited genetic mutations. Certain of these mutations cause changes in the amino acid sequence of α-GAL that may result in the production of α-GAL with reduced stability that does not fold into its correct three-dimensional shape. Although α-GAL produced in patient cells often retains the potential for some level of biological activity, the cell's quality control mechanisms recognize and retain misfolded α-GAL in the ER, until it is ultimately moved to another part of the cell for degradation and elimination. Consequently, little or no α-GAL 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 α-GAL 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 α-GAL by selectively binding to the enzyme, which increases its stability and helps the enzyme fold into its correct three-dimensional shape. This stabilization of α-GAL 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 α-GAL 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 α-GAL, those Fabry disease patients with a missense mutation or other genetic mutations that result in production of α-GAL that is less stable but with some residual enzyme activity are the ones most likely to respond to treatment with Amigal. We estimate that the majority of patients with Fabry disease may respond to pharmacological chaperone therapy. Patients with genetic mutations leading to a partially made α-GAL enzyme or α-GAL 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 α-GAL 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 recent 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 α-GAL 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 α-GAL 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 JAMA (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 α-GAL 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 α-GAL activity and mutations. The incidence of Fabry mutations in this study was 1:3100, over ten times higher than previous estimates. This high incidence was attributed to a large number of newborn males with α-GAL 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. In order to facilitate the proper diagnosis of Fabry disease patients seen by specialist physicians, we intend to provide support for testing for the disease, which is performed using a simple blood test for the level of α-GAL activity.

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 α-GAL 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 α-GAL and therefore may respond to treatment with Amigal. Based on this, we believe that a majority 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. There are currently two products approved for the treatment of Fabry disease. One of the products is Fabrazyme, a product approved globally and commercialized by Genzyme Corporation. Fabrazyme was approved in the U.S. in 2003 and has orphan drug exclusivity in the U.S. until 2010. It was approved in the EU in 2001 and has orphan drug exclusivity in the EU until 2011. The other product approved for treatment of Fabry disease is Replagal, a product approved in the EU and other countries but not in the U.S.,commercialized by Shire. Replagal was approved in the EU in August 2001 and has orphan drug exclusivity in the EU until 2011. The net product sales of Fabrazyme for 2007 were approximately $424 million as publicly reported by Genzyme Corporation. The net product sales for Replagal for the nine months ended September 30, 2007 were approximately $105 million as publicly reported by Shire.

Prior to the availability of enzyme replacement therapy, 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 and Replagal. Published data for patients treated with Fabrazyme and Replagal 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 and Replagal are believed to have difficulty penetrating some tissues and cell types. In particular, it is widely believed that Fabrazyme and Replagal 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 and Replagal 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 in the U.S. and of Fabrazyme and Replagal in the EU will not prevent us from obtaining marketing approval of Amigal in either geography. See “Government Regulation.”

Plicera for Gaucher Disease

Overview

Our second most advanced clinical product candidate, Plicera, is an orally-administered, small molecule, pharmacological chaperone for the treatment of Gaucher disease. As of November 2007, Plicera is being developed in partnership with Shire.

We completed Phase 1 clinical trials which demonstrated that Plicera was safe and well tolerated in healthy subjects at all doses tested. We are currently conducting two Phase 2 clinical trials of Plicera in Type I Gaucher patients.

- The first study, GAU-CL-201, involves subjects with Type I Gaucher disease who were currently receiving enzyme replacement therapy and agreed to discontinue their enzyme replacement therapy for a total of 7 weeks. The study was designed to assess the safety and pharmacodynamic effects of Plicera, particularly its effect on GCase levels.

We also monitored the effect of Plicera on parameters that are commonly abnormal in Gaucher disease including levels of red blood cells and platelets, although we do not expect to observe a change in these parameters in this 4-week trial because of its short duration. Patients were assigned to one of four treatment arms which include: 25 mg once per day, 150 mg once per day, 150 mg every four days, or 150 mg every seven days. Thirty individuals were enrolled in this study and enrollment is complete. Data are expected to be available in the first half of 2008.

- The second study, GAU-CL-202, involves subjects with Type I Gaucher disease who are naïve to enzyme replacement therapy and substrate reduction therapy. The study is designed to evaluate the safety of Plicera and its effect on parameters that are commonly abnormal in Gaucher disease including levels of red blood cells, platelets, liver and spleen volumes and other biomarkers related to Gaucher disease. Patients will be assigned to one of two treatment arms and will receive treatment with Plicera for approximately 6 months. This trial is currently underway.

Causes of Gaucher Disease and Rationale for Use of Plicera

Gaucher disease is a lysosomal storage disorder resulting from a deficiency in the enzyme, β-glucocerebrosidase (GCase). Signs and symptoms can be severe and debilitating, including an enlarged liver and spleen, abnormally low levels of red blood cells and platelets, and skeletal complications. In some forms of the disease there is also significant impairment of the central nervous system. The deficiency of GCase in Gaucher patients is caused by inherited genetic mutations. Certain of these mutations cause changes in the amino acid sequence of GCase that may result in the production of GCase with reduced stability that does not fold into its correct three-dimensional shape. Although GCase produced in patient cells often retains the potential for some level of biological activity, the cell's quality control mechanisms recognize and retain misfolded GCase in the ER until it is ultimately moved to another part of the cell for degradation and elimination. Consequently, little or no GCase moves to the lysosome, where it normally breaks down its substrate, a complex lipid called glucocerebroside. This leads to accumulation of glucocerebroside in cells, which is believed to result in the clinical manifestations of Gaucher disease. In addition, the accumulation of the misfolded GCase enzyme in the ER may lead to cellular stress and inflammatory-like responses, which may contribute to cellular dysfunction and disease.

Plicera is designed to act as a pharmacological chaperone for GCase by selectively binding to the enzyme, which increases the stability of the enzyme and helps it fold into its correct three-dimensional shape. This stabilization of GCase 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 glucocerebroside. As a result of restoring proper trafficking of GCase from the ER to lysosomes, Plicera reduces the accumulation of misfolded GCase in the ER, which may alleviate cellular stress and inflammatory-like responses that may be contributing factors in Gaucher disease.

Because Plicera increases the cellular levels of a patient's naturally produced GCase, those Gaucher disease patients with a missense mutation or other genetic mutation that results in production of GCase that is less stable but with some residual enzyme activity are the ones most likely to respond to treatment with Plicera. We estimate that the substantial majority of patients with Gaucher disease may respond to pharmacological chaperone therapy. Patients with genetic mutations leading to a partially made GCase enzyme or GCase enzyme with an irreversible loss of activity are less likely to respond to treatment with Plicera.

Gaucher Disease Background

Gaucher disease is often described in terms of the following three clinical subtypes:

- *Type I — Chronic Nonneuronopathic Gaucher Disease.* Type I Gaucher disease is the most common subtype affecting more than 90% of patients and symptoms usually first appear in adulthood. Type I Gaucher disease is characterized by the occurrence of an enlarged spleen and liver, anemia, low platelet counts and fractures and bone pain. Patients with Type I Gaucher disease do not experience the neurological features associated with Types II and III Gaucher disease. The clinical severity of Type I Gaucher disease is extremely variable with some patients experiencing the full range of symptoms, while others are asymptomatic throughout most of their lives.

- *Type II — Acute Neuronopathic Gaucher Disease.* Type II Gaucher disease symptoms typically appear in infancy with an average age of onset of about three months. Type II Gaucher disease involves rapid neurodegeneration with extensive visceral involvement that usually results in death before two years of age, typically due to respiratory complications. The clinical presentation in Type II Gaucher disease is typically more uniform than Type I Gaucher disease.

- *Type III — Subacute Neuronopathic Gaucher Disease.* Type III Gaucher disease symptoms typically first appear in infancy or early childhood and involve some neurological symptoms, along with visceral and bone complications. Age

of onset and disease severity can vary widely. Disease progression in Type III Gaucher disease is typically slower than in Type II Gaucher disease.

Gaucher Disease Market Opportunity

Gaucher disease is a relatively rare disorder. According to estimates reported by the American Society of Health-System Pharmacists (August 2003) and the National Institute of Neurological Disorders and Stroke (updated as of January 2006) there are approximately 10,000 patients worldwide. Type I Gaucher disease is, by far, the most common of the subtypes.

Published data, including data from the Human Gene Mutation Database, suggest that the substantial majority of patients with Gaucher disease have a missense mutation in at least one copy of the gene. The majority of the Type I Gaucher patients in the U.S., Europe and Israel have at least one copy of either the N370S or the L444P mutation, both of which are missense mutations. Based on our experience in the field and studies we have completed, including a Gaucher Ex Vivo Response Study, we believe that the substantial majority of individuals with Gaucher disease may benefit from treatment with Plicera. In addition, we believe that Plicera may also benefit some patients with the neuronopathic forms of Gaucher disease (Type II and Type III) because of the ability of the small molecule to cross the blood-brain barrier.

Existing Products for the Treatment of Gaucher Disease and Potential Advantages of Plicera

The current standard of treatment for Gaucher patients is enzyme replacement therapy. There are currently two products approved for the treatment of Gaucher disease, one of which is an enzyme replacement therapy. One of the products is Cerezyme, an enzyme replacement therapy approved globally and commercialized by Genzyme Corporation. Cerezyme was approved in the U.S. in 1994 and in the EU in 1997 and no longer has orphan drug exclusivity in the U.S. In the U.S., Cerezyme is indicated for long-term enzyme replacement therapy for pediatric and adult patients with a confirmed diagnosis of Type I Gaucher disease. In the EU, it is indicated for long-term enzyme replacement therapy for pediatric and adult patients with a confirmed diagnosis of Type I Gaucher disease and for Type III Gaucher disease patients who exhibit clinically significant non-neurological manifestations. The other product approved for treatment of Gaucher disease is Zavesca, a substrate reduction therapy product approved in the U.S., the EU and other countries and commercialized by Actelion, Ltd. Zavesca was approved in the U.S. in 2003 and has orphan drug exclusivity in the U.S. until 2010. It was approved in the EU in 2002 and has orphan drug exclusivity in the EU until 2012. It is indicated for adults with mild to moderate Type I Gaucher disease for whom enzyme replacement therapy is not an option. The net product sales of Cerezyme for the year 2007 were approximately $1.1 billion as publicly reported by Genzyme Corporation. The net product sales for Zavesca for the year ended 2007 were approximately $29 million as publicly reported by Actelion Ltd.

For Gaucher disease patients who respond to Plicera, we believe that the use of Plicera may have advantages relative to the use of Cerezyme. Published data demonstrate that treatment with Cerezyme can lead to the reduction of glucocerebroside in multiple tissue types, especially the liver and spleen, and to increased levels of red blood cells and platelets. However, because it is a large protein molecule, Cerezyme is believed to have difficulty penetrating some tissues and cell types. In particular, it is widely believed that Cerezyme is unable to cross the blood-brain barrier and thus unlikely to address the neurological symptoms of Type II and Type III Gaucher disease. Studies in animals show that Plicera distributes throughout the body. In particular, studies show that Plicera crosses the blood-brain barrier, suggesting that it may provide a clinical benefit to patients with Type II and Type III Gaucher disease. Additionally, treatment with Cerezyme requires intravenous infusions every other week, presenting an inconvenience to Gaucher disease patients. Oral treatment with Plicera may be more convenient for patients and may not have the safety risks associated with intravenous infusions.

We also believe that Plicera may have advantages over the use of Zavesca, a substrate reduction therapy. Zavesca is an orally-administered small molecule; however, the underlying mechanism of action is very different than for pharmacological chaperones. 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. This is not the same enzyme that is deficient in Gaucher disease. Importantly, the enzyme that is inhibited is needed to make molecules that are used for many types of biological processes. As a result, inhibiting this enzyme may have adverse effects that are difficult to predict. By contrast, Plicera is designed to bind directly to GCase, increasing its stability and helping it fold into its correct three-dimensional shape. This in turn enables proper trafficking to the lysosome where it can directly decrease substrate accumulation. Several side effects were reported by Actelion, Ltd. in clinical trials of Zavesca, including diarrhea, which was observed in more than 85% of patients who received the drug. Other side effects included hand tremors and numbness and tingling in the hands, arms, legs or feet. Plicera's mechanism of action is very different from Zavesca's, and we do not expect it to have the same side-effect profile.

In February 2006, the FDA granted orphan drug designation for the active ingredient in Plicera for the treatment of Gaucher disease in the U.S. We believe that the orphan drug designation of Zavesca in the U.S. and the EU will not prevent us from obtaining marketing approval of Plicera in either geography. See "Government Regulation."

AT2220 for Pompe Disease

Overview

Our third most advanced product candidate, AT2220, is an orally-administered small molecule pharmacological chaperone for the treatment of Pompe disease. As of November 2007, AT2220 is being developed in partnership with Shire.

We completed three Phase 1 clinical trials of AT2220 for Pompe disease in 2007. Three double-blind, placebo-controlled, dose escalation Phase 1 studies in healthy volunteers were completed. These studies were designed to evaluate the safety, tolerability and pharmacokinetics of AT2220. In a single ascending dose study, 32 individuals received oral doses of 50, 150, 300, or 600 mg AT2220 or placebo. In a multiple ascending dose study, 24 individuals received oral doses of 50, 150, or 450 mg/day AT2220 or placebo for 7 days. In a second multiple ascending dose study, 16 individuals received oral doses of 1000 or 2000 mg/day or placebo for 14 days. In all three studies, AT2220 was generally safe and well-tolerated at all doses and was orally bioavailable with a plasma half-life of 4 to 5 hours. There were no drug-related serious adverse events and no adverse events were considered to be definitely or probably related to study treatment. In the multiple ascending dose studies, all possibly-related adverse events were mild or moderate in severity and resolved spontaneously.

We expect to initiate a Phase 2 clinical trial of AT2220 for Pompe disease in the first half of 2008. The FDA's Office of Orphan Products Development has granted orphan drug designation for the active ingredient in AT2220 in the U.S.

Causes of Pompe Disease and Rationale for Use of AT2220

Pompe disease is a neuromuscular and lysosomal storage disorder caused by a deficiency in the enzyme α-glucosidase (Gaa). Symptoms can be severe and debilitating, including progressive muscle weakness throughout the body, particularly the heart and skeletal muscles. The deficiency of Gaa in Pompe patients is caused by inherited genetic mutations. Certain of these mutations cause changes in the amino acid sequence of Gaa that may result in the production of Gaa with reduced stability that does not fold into its correct three-dimensional shape. Although Gaa produced in patient cells often retains the potential for biological activity, the cell's quality control mechanisms recognize and retain misfolded Gaa in the ER, until it is ultimately moved to another part of the cell for degradation and elimination. Certain other mutations cause changes in RNA processing that lead to the production of normal Gaa, but at levels that are much lower than in an unaffected individual. In either case, little or no Gaa moves to the lysosome, where it normally breaks down its substrate, glycogen. This leads to accumulation of glycogen in cells, which is believed to result in the majority of clinical manifestations of Pompe disease. In addition, the accumulation and mistrafficking of Gaa may lead to stress on cells and inflammatory-like responses, which may contribute to cellular dysfunction and disease.

AT2220 is designed to act as a pharmacological chaperone for Gaa by selectively binding to Gaa and increasing its stability which helps the enzyme fold into its correct three-dimensional shape. We believe this stabilization of Gaa allows the cell's quality control mechanisms to recognize the protein 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 glycogen. We believe AT2220 may increase proper trafficking of Gaa in patients that produce unstable misfolded Gaa, and in patients that produce low levels of normal Gaa because some fraction of normal Gaa can also fail to pass the cell's quality control system. AT2220 has been shown to increase Gaa levels in cell lines derived from Pompe patients, in transferred cells expressing mutant forms of Gaa and in healthy mice and monkeys. In addition, as a result of increasing the proper trafficking of unstable misfolded Gaa to the lysosome, AT2220 may reduce the accumulation of misfolded Gaa in the ER, which may alleviate cellular stress and inflammatory-like responses that may be contributing factors in Pompe disease.

Because AT2220 is believed to increase the activity of a patient's naturally produced Gaa, those Pompe disease patients with a mutation that results in production of Gaa with some residual enzyme activity are the ones most likely to respond to treatment with AT2220. We estimate that the majority of patients with Pompe disease may respond to pharmacological chaperone therapy. Patients with genetic mutations leading to a partially made Gaa enzyme or Gaa enzyme with an irreversible loss of activity are less likely to respond to treatment with AT2220.

Pompe Disease Background

Pompe disease, also known as glycogen storage disease type II or acid maltase deficiency, is a relatively rare disorder caused by mutations in Gaa. The mutations in Gaa result in the accumulation of lysosomal glycogen, especially in skeletal, cardiac and smooth muscle tissues. According to reported estimates of the Acid Maltase Deficiency Association, the United Pompe Foundation and the Lysosomal Disease Program at Massachusetts General Hospital, there are 5,000-10,000 patients with Pompe disease worldwide.

Pompe disease ranges from a rapidly fatal infantile form with severe cardiac involvement to a more slowly progressive, later-onset form primarily affecting skeletal muscle. All forms are characterized by severe muscle weakness that worsens over time. In the rapid onset form, patients are usually diagnosed shortly after birth and often experience enlargement of the heart and severe muscle weakness. In later-onset Pompe disease, symptoms may not appear until late childhood or adulthood and patients often experience progressive muscle weakness.

Pompe Disease Market Opportunity

Pompe disease is a relatively rare disorder. Most reported estimates project that there are 5,000 to 10,000 patients worldwide, the majority of whom have later-onset Pompe disease.

Based on published data from the Human Gene Mutation Database and our experience in the field, we believe that many of the known genetic mutations that cause Pompe disease are mutations that result in measurable residual enzyme activity. The majority of Pompe patients have either juvenile or adult-onset disease, and both types of patients generally have measurable levels of residual enzyme activity. Because pharmacological chaperone therapy is most likely to benefit patients with some residual enzyme activity, we believe that a majority of the Pompe patient population may benefit from treatment with AT2220. There are a few mutations reported in Pompe disease that are more common in specific ethnic populations, including a splice-site mutation common in Caucasians with adult-onset disease. Studies published in the Journal of Medical Genetics, Human Mutation, and the Journal of Neurology suggest that over 70% of all Caucasians with adult-onset Pompe disease have at least one copy of this splice-site mutation. Because this splice-site mutation results in the production of normal Gaa protein, albeit at a level lower than in a non-affected individual, we believe patients with this mutation may be addressable with pharmacological chaperone therapy.

Existing Products for the Treatment of Pompe Disease and Potential Advantages of AT2220

The current standard of treatment for Pompe patients is enzyme replacement therapy. There is currently one product approved for the treatment of Pompe disease, Myozyme, approved in the U.S. and the EU and commercialized by Genzyme Corporation. Myozyme was approved in the U.S. in April 2006 and has orphan drug exclusivity in the U.S. until 2013. It was approved in the EU in March 2006 and has orphan drug exclusivity in the EU until 2016. Although Myozyme is approved for use in all Pompe patients, studies currently reported on the label included only patients with infantile-onset disease. Data from a study looking at Myozome in patients with later-onset disease was reported in late 2007 and is currently under review by various regulatory authorities. This study reportedly achieved the primary endpoint of demonstrating an improvement in motor and pulmonary function. The net product sales of Myozyme for the first full year of sales in 2007 were approximately $201 million as publicly reported by Genzyme Corporation.

For Pompe disease patients who respond to AT2220, we believe that the use of AT2220 may have advantages relative to the use of Myozyme. Available data demonstrate that treatment with Myozyme can improve survival in patients with the infantile form of the disease and may slow the decline in motor and pulmonary function in patients with the later onset form of the disease. Because it is a large protein molecule, Myozyme is believed to have difficulty penetrating many tissues and cell types. Because AT2220 is a small molecule that has demonstrated high oral bioavailability and good biodistribution properties in preclinical testing, it has the potential to reach all cells of the target tissues of Pompe disease patients. Furthermore, treatment with Myozyme requires intravenous infusions every other week, frequently on site at health care facilities, presenting an inconvenience to Pompe disease patients. The label for Myozyme also indicates that the infusion has safety concerns, with infusion reactions observed in 51% of patients, and severe infusion-related reactions observed in 14% of patients. Oral treatment with AT2220 may be more convenient for patients and may not have the safety risks associated with intravenous infusions.

We believe that the orphan drug designation of Myozyme in the U.S. and in the EU will not prevent us from obtaining marketing approval of AT2220 in either geography. See “Government Regulation.”

Other Programs

We believe that our pharmacological chaperone technology is applicable to the development of drugs for the treatment of a wide range of human genetic and other diseases. We are currently researching the use of pharmacological chaperones for the treatment of diseases other than lysosomal storage disorders, including neurological diseases such as Parkinson’s disease. An epidemiological and biochemical link between Gaucher and Parkinson’s disease has been established. Furthermore, in 2007, we generated data showing that treatment with Plicera led to reduction in a-synuclein in the brains of animal model for Parkinson’s disease.

Strategic Alliances and Arrangements

We intend to form strategic alliances to gain access to the financial, technical, clinical and commercial resources necessary to develop and market pharmacological chaperone therapeutics. We expect these alliances to provide us with financial support in the form of equity investments, research and development funding, license fees, milestone payments and royalties or profit-sharing based on sales of pharamacological chaperone therapeutics. We currently have one strategic alliance with Shire.

On November 7, 2007, we entered into a License and Collaboration Agreement with Shire. Under the agreement, Amicus and Shire will jointly develop Amicus' three lead pharmacological chaperone compounds for lysosomal storage disorders: Amigal, Plicera and AT2220. We granted Shire the rights to commercialize these products outside the U.S. We will retain all rights to our other programs and to develop and commercialize Amigal, Plicera and AT2220 in the U.S.

We received an initial, non-refundable license fee payment of $50 million from Shire. Joint development costs toward global approval of the three compounds will be shared 50/50 going forward. In addition, we are eligible to receive, for all three drug product candidates, aggregate potential milestone payments of up to $150 million if certain clinical and regulatory milestones are achieved for all three of the programs, and $240 million in sales-based milestones for all three of the programs. We will also be eligible to receive tiered double-digit royalties on net sales of the products which are marketed outside of the U.S. Not including royalties and cost-sharing, the deal is valued at up to U.S. $440 million.

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 our three leading product candidates are described below and include both patents and patent applications we own or exclusively license:

- We have an exclusive license to five issued U.S. patents and several pending U.S. applications that cover use of Amigal, as well as corresponding foreign applications. U.S. patents relating to Amigal expire in 2018 (not including the Hatch-Waxman statutory extension, which is described below), while the foreign counterpart patents, if granted, would 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 α-GAL, and methods for the treatment of Fabry disease using Amigal and other specific competitive inhibitors of α-GAL. 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 several pending U.S. applications directed to synthetic steps related to the commercial process for preparing Amigal, which may result in patents that expire in 2026. Lastly, we jointly own one pending U.S. 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. We have filed, or plan to file, foreign counterparts of these applications, where appropriate, by the applicable deadlines.

- We have an exclusive license to seven U.S. patents and two pending U.S. applications, and five foreign patents and several pending foreign applications that cover Plicera or its use. Two of the U.S. patents relating to Plicera compositions of matter expire in 2015 and 2016 (not including the Hatch-Waxman statutory extension, which is described below); the five composition of matter foreign patents and one pending foreign application, if granted, expire in 2015 (not including the SPC extensions, which are described below). The other five U.S. patents and two pending applications, which claim methods of increasing the activity of and preventing the degradation of GCase, and methods for the treatment of Gaucher disease using Plicera and other specific competitive inhibitors of GCase, expire in 2018. We own a pending U.S. application directed to the particular form of the active agent in Plicera, which, if granted, will expire in 2027. We own one pending U.S. application directed to dosing regimens for Plicera, which if granted, will expire in 2028. We own one pending U.S. application directed to specific treatment and monitoring regimens with Plicera. If granted, this also will expire in 2028. Lastly, we own one pending U.S. application directed to methods of synthesis of 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 three U.S. patents that cover use of AT2220, two pending U.S. applications, as well as corresponding foreign applications. The U.S. patents relating to AT2220 expire in 2018 (not including the Hatch-Waxman statutory extension, which is described below), while the foreign counterpart patents, if granted, would expire in 2019 (not including the 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 Gaa, and methods for the treatment of Pompe disease using AT2220 and other specific competitive inhibitors of Gaa.

Our patent estate also includes patent applications we license or own relating to combination compositions or uses for our product candidates or new potential product candidates. Some of these applications are pending in the U.S. and foreign patent offices, and include one family of patents licensed from Mt. Sinai School of Medicine and one U.S. patent application and international application jointly owned with the Université of Montréal. Others have to date only been filed as provisional applications in the U.S. We have filed or expect to file some of these as non-provisional applications in U.S. and in other countries at the appropriate time. These patent applications, assuming they issue as patents, would expire in the U.S. between 2023 and 2028.

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 of New York University. 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. In connection with this agreement, we issued 232,266 shares of our common stock to Mt. Sinai School of Medicine in April 2002. In October 2006 we issued Mt. Sinai School of Medicine 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. This agreement expires upon expiration of the last of the licensed patent rights, which will be in 2019 if a foreign patent is granted and 2018 otherwise, or later 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 $29,500. 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 2 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 Mt. Sinai School of Medicine and will owe no milestone payments. We 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 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.

Manufacturing

We continue to rely on contract manufacturers to supply the active pharmaceutical ingredients and gelatin capsules for Amigal, Plicera and AT2220. The active pharmaceutical ingredients for all three 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. While we believe that our technologies, knowledge, experience and scientific resources provide us with competitive advantages, we face potential competition from many different sources, including commercial pharmaceutical and biotechnology 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.

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):

Competitor	Indication	Product	Class of Product	Status	2007 Sales (in millions)
Genzyme Corporation	Fabry disease	Fabrazyme	Enzyme Replacement Therapy	Marketed	$ 424
	Gaucher disease	Cerezyme	Enzyme Replacement Therapy	Marketed	$1,100
	Pompe disease	Myozyme	Enzyme Replacement Therapy	Marketed	$ 201
	Gaucher disease	Genz-112638	Substrate Reduction Therapy	Phase 2	N/A
Shire	Fabry disease	Replagal	Enzyme Replacement Therapy	Marketed	$ 105*
	Gaucher disease	GA-GCB	Enzyme Replacement Therapy	Phase 3	N/A
Actelion, Ltd.	Gaucher disease	Zavesca	Substrate Reduction Therapy	Marketed	$ 29
Protalix Biotherapeutics	Gaucher disease	prGCD	Enzyme Replacement Therapy	Phase 3	N/A

* Nine Months Sales through September 30, 2007

We are aware of other companies that are conducting preclinical development activities for enzyme replacement therapies to treat Gaucher disease and Pompe disease.

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 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 preclinical laboratory and animal tests, the submission to the FDA of a notice of claimed investigational exemption or 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 each IND is required prior to the commencement of clinical testing in humans. If the FDA has not commented on or questioned the IND within this 30-day period, the clinical trial proposed in the IND may begin.

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 federal 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 NDAs 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 2 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 manufacturer and/or sponsor under an approved new drug application are 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 consider certain information or clarification regarding information already provided in the 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, an approvable letter or a not-approvable letter. Both approvable and not-approvable letters generally 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 a resubmission of 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 pre-clinical 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.

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 minimization action plans, 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 periodic unannounced 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. 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 2003 (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 a new drug candidate may request FDA to designate the drug candidate as a fast track drug concurrent with or after the filing of the IND for the drug candidate. 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.

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 filing of an 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. 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 and Replagal 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 and Replagal. Similarly, we believe the orphan drug designation of Zavesca in the EU will not prevent us from obtaining marketing approval of Plicera in the EU for the treatment of Gaucher disease because Plicera will provide significant benefits over Zavesca.

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, 2007, we had 91 full-time employees, 60 of whom were primarily engaged in research and development activities and 31 of whom provide administrative services. A total of 32 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. 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 furnishes 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 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™ and Zavesca® are the property of their respective owners.

Item 1A. RISK FACTORS.

Investing in our common stock involves a high degree of risk. You should carefully consider the following risk factors, as well as other information in this report, before deciding to invest in shares of our common stock. 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.

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 net loss attributable to common stockholders was $41.5 million, $65.9 million and $20.1 million for the years ended 2007, 2006 and 2005 respectively. As of December 31, 2007, we had an accumulated deficit of $124.8 million. To date, we have financed our operations primarily through private placements of our redeemable convertible preferred stock and through proceeds from our initial public offering. 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. We anticipate that our expenses will increase substantially as we:

- continue our ongoing Phase 2 extension study of Amigal (migalastat hydrochloride) for the treatment of Fabry disease and initiate a Phase 3 clinical trial of Amigal;
- continue our ongoing Phase 2 clinical trials of Plicera (isofagomine tartrate) for the treatment of Gaucher disease and potentially conduct additional Phase 2 and later-stage clinical trials of Plicera;
- initiate a Phase 2 clinical trial of AT2220 for the treatment of Pompe disease and potentially conduct later-stage clinical trials of AT2220;
- continue the research and development of additional product candidates;
- seek regulatory approvals for our product candidates that successfully complete clinical trials;
- establish a sales and marketing infrastructure to commercialize products for which we may obtain regulatory approval; and
- add operational, financial and management information systems and personnel, including personnel to support our product development efforts and our obligations as a public company.

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 our research and development expenses to increase in connection with our ongoing activities, particularly as we initiate a Phase 3 clinical trial of Amigal, continue our Phase 2 clinical trials of Plicera and initiate a Phase 2 clinical trial of AT2220, and for any later-stage clinical trials of our product candidates. In addition, subject to obtaining regulatory approval of any of our product candidates, we expect to incur significant commercialization expenses for product sales and marketing, securing commercial quantities of product from our manufacturers and product distribution. We currently have no additional commitments or arrangements for any additional financing to fund the research and development and commercial launch of our product candidates.

We believe that the net proceeds from our initial public offering, together with our existing cash and cash equivalents and marketable securities, will be sufficient to enable us to fund our operating expenses and capital expenditure requirements until at least early 2010. Capital may not be available when needed on terms that are acceptable to us, or at all. 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, Plicera and AT2220;
- the scope, progress, results and costs of preclinical development, laboratory testing and clinical trials for our other product candidates;
- 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.

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 raise capital by issuing equity securities, our stockholders will experience dilution. 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 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 limited 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 candidates, Amigal, Plicera and AT2220. 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, Plicera or AT2220, 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, Amigal, Plicera and AT2220. Our ability to generate product revenue, which we do not expect will occur for at least the next several years, if ever, 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:

- obtaining supplies of Amigal, Plicera and AT2220 for completion of our clinical trials on a timely basis;

- successful completion of preclinical studies and clinical trials;
- 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 product candidates are being developed to address is relatively 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, Gaucher 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.

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, Gaucher disease or Pompe 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.

Our efforts to develop all of our product candidates are at an early stage. 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.

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. We note that a patient who started dosing in March 2006 in the ongoing Phase 2 clinical trials for Amigal for the treatment of Fabry disease elected to withdraw from the study in March 2006, shortly after initial dosing. This patient had a history of hypertension and discontinued study treatment due to increased blood pressure, which was reported by the investigator as possibly related to the study drug.

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, we cannot be sure what endpoints the FDA will require us to measure in later-stage clinical trials of our product candidates. We are aware that the currently available enzyme replacement therapy for the treatment of Fabry disease was approved by the FDA based on an endpoint measuring GL-3 levels in a specific type of kidney cell. We cannot be certain that the FDA will permit the use of this endpoint in our Phase 2 trials of Amigal. 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. To date, we have only three lead product candidates: Amigal, Plicera and AT2220. We have not obtained regulatory approval nor commercialized any of these or any other product candidates. We have completed Phase 2 clinical trials for Amigal and are currently conducting Phase 2 clinical trials for Plicera and have completed Phase 1 clinical trials for AT2220 but have not yet initiated a Phase 3 clinical trial for any of our product candidates. 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. Given that each of our product candidates is in the early stages of required testing, 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. Additionally, many patients with Fabry disease, Gaucher disease and Pompe disease may already be receiving existing therapies, such as enzyme replacement therapy, which would render them ineligible for our current clinical trials if they are not willing to stop receiving such therapies. 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;
- 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 or 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 may be insufficient or inadequate or we may not be able to reach agreements on acceptable terms with prospective clinical research organizations; 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;
- 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.

The commercial success of any product candidates that we may develop, including Amigal, Plicera and AT2220, 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, Plicera and AT2220, if they receive marketing approval, 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 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. 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. 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. We may not be able to establish sales and distribution partnerships 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.

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 Fabry disease. These products include Genzyme Corporation's Fabrazyme and Shire plc's Replagal. In addition, Genzyme Corporation and Actelion, Ltd. market and sell Cerezyme and Zavesca, respectively, for the treatment of Gaucher disease, and Genzyme Corporation markets and sells Myozyme for the treatment of Pompe disease. We are also aware of other enzyme replacement and substrate reduction therapies in development by third parties.

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.

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 will be 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 manufacturers are subject to unannounced inspections by the FDA, state regulators and similar regulators outside the U.S. Our failure, or the failure of our third party manufacturers, to comply with applicable regulations could result in sanctions being imposed on us, including fines, injunctions, civil penalties, failure of regulatory authorities to grant marketing approval of our product candidates, delays, suspension or withdrawal of approvals, license revocation, seizures or recalls of product candidates or products, operating restrictions and criminal prosecutions, any of which 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 certain preclinical development activities of our product candidates, such as long-term safety studies in animals, or clinical trials for our product candidates. We rely on, or work in conjunction with, third parties, such as contract research organizations, medical institutions and clinical investigators, to perform this function. 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. We also may seek to establish collaborations for the sales, marketing and distribution of our products outside the U.S. 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 collaborations 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 in the event of a material breach or lack of scientific progress by us;
- 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 Shire Pharmaceuticals Ireland Ltd. (Shire) is important to our business. If this collaboration is unsuccessful or if Shire terminates this collaboration or its participation in individual programs, our business could be adversely affected.

In November 2007, we entered into a license and collaboration agreement with Shire to jointly develop our three lead pharmacological chaperone compounds for lysosomal storage disorders: Amigal, Plicera and AT2220. Under this agreement, Shire will contribute 50% of joint development costs toward global approval, we are eligible to receive an additional $150 million if certain clinical and regulatory milestones are met; and we are also eligible to receive up to $240 million in sales-based milestones, as well as tiered double-digit royalties.

Shire may elect to terminate this collaboration or its participation in the development of individual programs under certain circumstances or in the event of a material uncured breach by us. We expect that a substantial amount of the funding for our operations will come from this collaboration. If this collaboration is unsuccessful, or if it is terminated in whole or in part, our business could be adversely affected and we could require additional financing earlier than we currently expect.

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 own or have licensed relating to use of Amigal expire in 2018 in the U.S. and 2019 outside of the U.S., and the foreign counterparts, if issued, would expire in 2019. Patents that we own or have licensed relating to Plicera expire between 2015 and 2016 in the U.S. and in 2015 outside of the U.S. for composition of matter, and in 2018 in the U.S. for methods of use. We currently have no issued patents or pending applications 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 outside of the U.S. 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, two of our three lead product candidates. 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. We have received written notice from one of these third parties indicating that it believes we may need a license to certain of these patents in order to avoid infringing such patents. If any of these third party 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. 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 future 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, Plicera and AT2220, 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.

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.

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. For example, in a clinical trial of Amigal for Fabry disease, one patient with a history of hypertension experienced increased blood pressure during the course of the trial which was reported by the investigator as possibly related to the drug. Further, Amigal has been shown to cause reversible infertility effects in mice.

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 Amigal or Plicera for these indications, 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.

Risks Related to Employee Matters and Managing Growth

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 President and Chief Executive Officer, Matthew R. Patterson, our Chief Operating Officer, James E. Dentzer, our Chief Financial Officer, and David J. Lockhart, Ph.D., our Chief Scientific 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). The U.S. recently called Mr. Crowley to service, which he fulfilled, from September 11, 2006 to March 5, 2007, and he may be called to active duty service again 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 Messrs. Patterson and Dentzer and Dr. Lockhart. 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 personnel, clinical personnel and sales and marketing personnel will also be critical to our success. 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.

We also experience competition for the hiring of scientific and clinical personnel from universities and research institutions. 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.

We expect to expand our development, regulatory and sales and marketing capabilities, and as a result, we may encounter difficulties in managing our growth, which could disrupt our operations.

We are a development stage company with 91 full-time employees as of December 31, 2007. Of these employees, 60 work primarily in research and development and 31 provide administrative services. We expect to experience significant growth in the number of our employees and the scope of our operations, particularly in the areas of drug development, regulatory affairs and sales and marketing. Assuming our plans and business conditions progress consistent with our current projections, we plan to grow to a total of approximately 115 to 140 employees by the end of 2008. To manage our anticipated future growth, we must continue to implement and improve our managerial, operational and financial systems, expand our facilities and continue to recruit and train additional qualified personnel. Due to our limited resources, we may not be able to effectively manage the expansion of our operations or recruit and train additional qualified personnel. The physical expansion of our operations may lead to significant costs and may divert our management and business development resources. Any inability on the part of our management to manage growth could delay the execution of our business plans or disrupt our operations.

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 74% of our common stock. 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 that became effective upon our initial public offering 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 will depend 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 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.

We will incur increased costs as a result of being a public company.

As a public company, we will incur significant legal, accounting, reporting and other expenses that we did not incur as a private company, including costs related to compliance with the regulations of the Sarbanes-Oxley Act of 2002. We expect these rules and regulations to increase our legal and financial compliance costs and to make some activities more time-consuming and costly. We also expect these new rules and regulations may make it more difficult and more expensive for us to obtain director and officer liability insurance and we may be required to accept reduced policy limits and coverage or incur substantially higher costs to obtain the same or similar coverage. As a result, we may experience more difficulty attracting and retaining qualified individuals to serve on our board of directors or as executive officers. We cannot predict or estimate the amount of additional costs we may incur as a result of these requirements or the timing of such costs.

Item 1B. UNRESOLVED STAFF COMMENTS.

None.

Item 2. PROPERTIES.

We currently lease approximately 49,000 square feet of subleased office and laboratory space in Cranbury, New Jersey under lease agreements that terminate in 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. SUBMISSION OF MATTERS TO A VOTE OF SECURITY HOLDERS.

No matters were submitted to a vote of security holders during the fourth quarter of the year ended December 31, 2007.

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, for the periods indicated, the range of high and low closing sales prices of our common stock as quoted on the NASDAQ Global Market for the period since our initial public offering on May 31, 2007.

	2007	
	High	Low
May 31, 2007 — June 30, 2007	$16.80	$10.30
Third Quarter	16.75	10.00
Fourth Quarter	18.22	9.20

The closing price for our common stock as reported by the NASDAQ Global Market on January 31, 2008 was $9.72 per share. As of January 31, 2008, there were 70 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

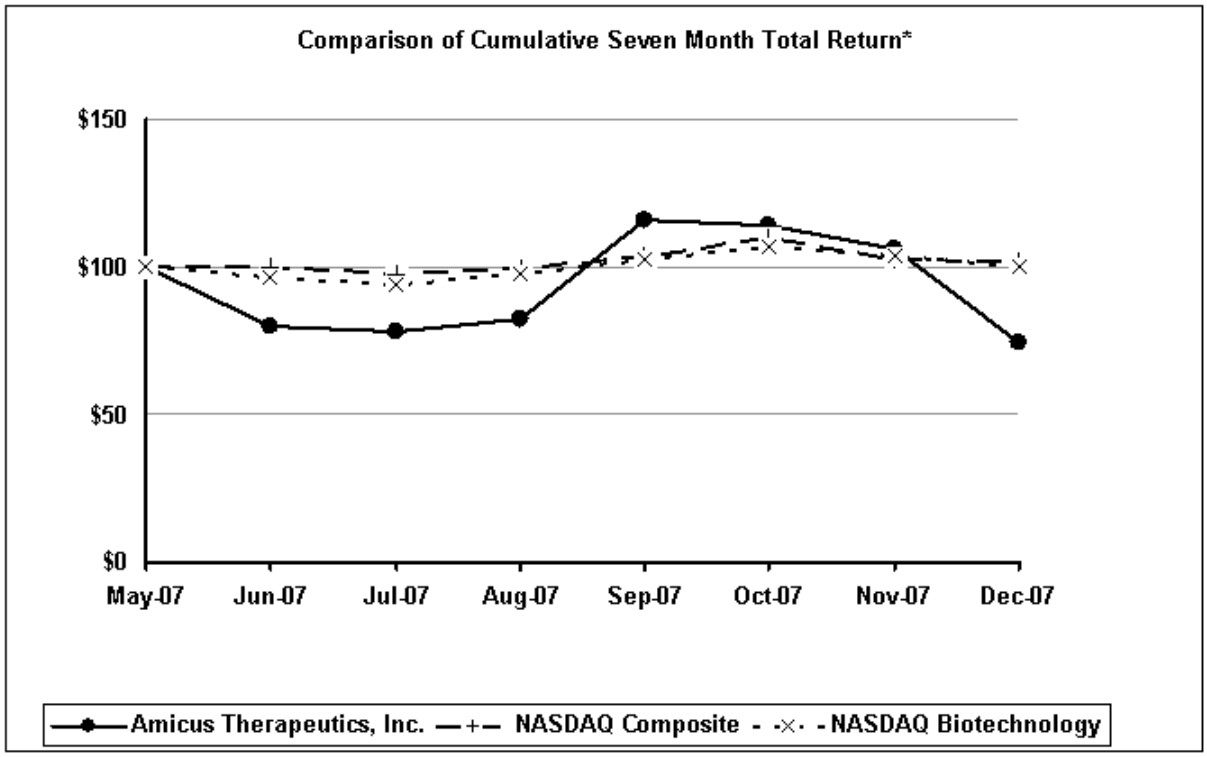
Our initial public offering of common stock was effected through a Registration Statement on Form S-1 (File No. 333-141700) that was declared effective by the Securities and Exchange Commission (SEC) on May 30, 2007, which registered an aggregate of 5,750,000 shares of our common stock. On June 5, 2007, 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.

We paid to the underwriters underwriting discounts totaling approximately $5.3 million in connection with the offering. In addition, we incurred additional costs of approximately $1.6 million in connection with the offering, which when added to the underwriting discounts paid by us, amounts to total expenses of approximately $6.9 million. Thus, the net offering proceeds to us, after deducting underwriting discounts and offering expenses, were approximately $68.1 million. 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, 2007, we had invested the $68.1 million in net proceeds from the offering in money market funds and in investment-grade, interest bearing instruments, pending their use. Through December 31, 2007, we used these proceeds for clinical development of our drug candidates, for research and development activities relating to additional preclinical programs and to fund working capital and other general corporate purposes, which may include the acquisition or licensing of complementary technologies, products or businesses.

Performance Graph

The following performance graph shows the total shareholder return of an investment of $100 cash on May 30, 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, 2007. 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.

	May-07	Jun-07	Jul-07	Aug-07	Sep-07	Oct-07	Nov-07	Dec-07
Amicus Therapeutics, Inc.	100	80	78	82	116	114	106	74
NASDAQ Composite	100	100	98	100	104	110	102	102
NASDAQ Biotechnology	100	96	94	97	102	107	104	100

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, 2007:

Period	(a) Total number of shares purchased	(b) Average Price Paid per Share	(c) Total number of shares purchased as part of publicly announced plans or programs	(d) Maximum number of shares that may yet be purchased under the plans or programs
October 1, 2007 - October 31, 2007	2,645	$17.10	—	10,580
November 1, 2007 - November 31, 2007	220	$16.17	—	10,580
December 1, 2007 - December 31, 2007	220	$15.30	—	10,580
Total	3,085		—	

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

Item 6. SELECTED FINANCIAL DATA.
(in thousands except share and per share data)

	Year Ended December 31					Period from February 4, 2002 (inception) to December 31, 2007
	2003	2004	2005	2006	2007	
Statement of Operations Data:						
Revenue:						
Research revenue	$ —	$ —	$ —	$ —	$ 1,375	$ 1,375
Collaboration revenue	—	—	—	—	409	409
Total revenue	—	—	—	—	1,784	1,784
Operating expenses:						
Research and development	4,433	6,301	13,652	33,630	31,074	89,878
General and administrative	1,005	2,081	6,877	12,277	15,278	38,070
Impairment of leasehold improvements	1,030	—	—	—	—	1,030
Depreciation and amortization	132	146	303	952	1,237	2,794
In-process research and development	—	—	—	—	—	418
Total operating expenses	6,600	8,528	20,832	46,859	47,589	132,190
Loss from operations	(6,600)	(8,528)	(20,832)	(46,859)	(45,805)	(130,406)
Other income (expenses):						
Interest income	5	190	610	1,990	5,135	7,941
Interest expense	(172)	(550)	(82)	(273)	(348)	(1,430)
Change in fair value of warrant liability	—	(2)	(280)	(23)	(149)	(454)
Other expense	—	—	—	(1,180)	—	(1,180)
Loss before tax benefit	(6,768)	(8,890)	(20,584)	(46,345)	(41,167)	(125,529)
Income tax benefit	—	83	612	—	—	695
Net loss	(6,768)	(8,807)	(19,972)	(46,345)	(41,167)	(124,834)
Deemed dividend	—	—	—	(19,424)	—	(19,424)
Preferred stock accretion	(17)	(126)	(139)	(159)	(351)	(802)
Net loss attributable to common stockholders	$ (6,785)	$ (8,933)	$ (20,111)	$ (65,928)	$ (41,518)	(145,060)
Net loss attributable to common stockholders per common share — basic and diluted	$ (22.06)	$ (29.05)	$ (49.02)	$ (89.58)	$ (3.14)	
Weighted-average common shares outstanding — basic and diluted	307,539	307,539	410,220	735,967	13,235,755	

	As of December 31,				
	2003	2004	2005	2006	2007
Balance Sheet Data:					
Cash and cash equivalents and marketable securities	$ 15	$ 4,336	$ 24,418	$ 54,699	$ 161,527
Working capital	(5,588)	3,569	22,267	44,814	147,247
Total assets	501	5,073	28,670	59,645	167,097
Total liabilities	5,776	1,346	4,031	13,071	63,800
Redeemable convertible preferred stock	2,432	20,013	60,469	124,089	—
Deficit accumulated during the development stage	(8,503)	(17,351)	(37,322)	(83,667)	(124,834)
Total stockholders' (deficiency) equity	$ (7,708)	$(16,287)	$(35,830)	$ (77,515)	$ 103,297

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

Overview

We are a clinical-stage biopharmaceutical company focused on the discovery, development and commercialization of novel small molecule, orally-administered drugs, known as pharmacological chaperones, for the treatment of a range of human genetic diseases. Certain human diseases result from 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 proteins. Misfolded 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, thereby increasing protein activity, improving cellular function and potentially reducing cell stress. We completed our Phase 2 clinical trials of Amigal (migalastat hydrochloride), are currently conducting Phase 2 clinical trials of Plicera (isofagomine tartrate) and completed Phase 1 clinical trials of AT2220.

We have generated significant losses to date and expect to continue to generate losses as we continue the clinical development of Amigal, Plicera and AT2220 and conduct research on other programs. From our inception in February 2002 through December 31, 2007, we have accumulated a deficit of $124.8 million. As we have not yet generated commercial sales revenue from any of our product candidates, our losses will continue as we conduct our research and development activities. These activities are budgeted to expand over time and will require further resources if we are to be successful. As a result, our operating losses are likely to be substantial over the next several years. We may need to obtain additional funds to further develop our research and development programs and product candidates.

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 after deducting underwriting discounts, commissions and offering expenses payable by us. In connection with the closing of the initial public offering, all of the Company's shares of redeemable convertible preferred stock outstanding at the time of the offering were automatically converted into 16,112,721 shares of common stock.

Collaboration with Shire Pharmaceuticals Ireland Ltd. (Shire)

On November 7, 2007, we entered into a license and collaboration agreement with Shire. Under the agreement, Amicus and Shire will jointly develop Amicus' three lead pharmacological chaperone compounds for lysosomal storage disorders: Amigal, Plicera and AT2220. We granted Shire the rights to commercialize these products outside the United States (U.S.). We will retain all rights to our other programs and to develop and commercialize Amigal, Plicera and AT2220 in the U.S.

We received an initial, non-refundable license fee payment of $50 million from Shire. Joint development costs toward global approval of the three compounds will be shared 50/50 going forward. In addition, we are eligible to receive, for all three drug product candidates, aggregate potential milestone payments of up to $150 million if certain clinical and regulatory milestones are achieved for all three of the programs, and $240 million in sales-based milestones for all three of the programs. We will also be eligible to receive tiered double-digit royalties on net sales of the products which are marketed outside of the U.S.

Financial Operations Overview

Revenue

The collaboration agreement with Shire became effective in November 2007. Shire paid us an initial, non-refundable license fee of $50 million in November 2007. At December 31, 2007, we recognized approximately $0.4 million of the license fee in Collaboration Revenue and $1.4 million of Research Revenue for reimbursed research and development fees. The license fee will be recognized as Collaboration Revenue over the 18 year performance obligation period. We have not generated any commercial sales revenue since our inception.

Research and Development Expenses

We expect our research and development expense to increase as we continue to develop our product candidates. Research and development expense consists of:

- internal costs associated with our research activities;
- payments we make to third party contract research organizations, contract manufacturers, investigative sites, and consultants;
- technology and intellectual property 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 do not believe that allocating internal costs on the basis of estimates of time spent by our employees would accurately represent the actual costs of a project. We do, however, 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, 2007, we have incurred research and development expense in the aggregate of $89.9 million.

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

	Years Ended December 31,			Period from February 4, 2002 (Inception) to December 31, 2007
	2005	2006	2007	
Product Candidate				
Third party direct project expenses Amigal (Fabry Disease — Phase 2)	$ 5,579	$ 3,361	$ 4,648	$ 21,030
Plicera (Gaucher Disease — Phase 2)	2,109	9,905	4,378	16,108
AT2220 (Pompe Disease — Phase 1)	374	4,427	3,426	8,188
Total third party direct project expenses	8,062	17,693	12,452	45,326
Other project costs (1)				
Personnel costs	3,581	8,187	9,720	24,431
Other costs (2)	2,009	7,750	8,902	20,121
Total other project costs	5,590	15,937	18,622	44,552
Total research and development costs	$ 13,652	$ 33,630	$ 31,074	$ 89,878

(1) Other project costs are leveraged across multiple clinical and pre-clinical projects.

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

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, or the period, if any, in which material net cash inflows may commence from Amigal, Plicera, AT2220 or any of our other preclinical

product candidates. This uncertainty is due to the numerous risks and uncertainties associated with the duration and cost of clinical trials, which vary significantly over the life of a project as a result of differences arising 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; and
- the results of our clinical trials.

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. 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 of that product candidate. For example, if the U.S. Food and Drug Administration (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, 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. We expect that our general and administrative expenses will increase as we add personnel and are subject to the reporting obligations applicable to public companies. From our inception in February 2002 through December 31, 2007, we spent $38.1 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.

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 in accordance with the Securities and Exchange Commission (SEC) Staff Accounting Bulletin (SAB) No. 101, *Revenue Recognition in Financial* Statements (SAB 101), as amended by Staff Accounting Bulletin No. 104, *Revision of Topic 13* (SAB 104). Currently, we derive all of our revenues from our collaboration agreement with Shire and expect that we will continue to derive all, or at least a substantial portion of our revenues from collaboration agreements over the next several years.

Collaboration arrangements may contain multiple elements, including up front licensing fees, reimbursement payments for ongoing research and development, payments for achieving research and development and product approval milestones, sales-based milestones and royalties based on percentages of net product sales. When evaluating multiple element arrangements, we follow the provisions of Emerging Issues Task Force (EITF) Issue 00-21, *Revenue Arrangements with Multiple Deliverables* (EITF 00-21). EITF 00-21 provides guidance on determining if an arrangement involves a single unit of accounting or separate units of accounting and if the arrangement is determined to have separate units, how to allocate amounts received in the

arrangement for revenue recognition purposes. If a collaboration agreement involves a single unit of accounting, the revenue recognition policy and the performance obligation period is determined for the entire arrangement while if separate units of accounting are involved, we determine a revenue recognition policy for each unit.

In November 2007, we entered into a collaboration agreement with Shire. We evaluated the multiple elements of this agreement in accordance with the guidance in EITF 00-21 and determined that the multiple deliverables did not have value on a stand alone basis and were unable to obtain verifiable objective evidence to determine the fair value of undelivered elements. As a result, we concluded that there was one single unit of accounting for this collaboration and determined that the period of our performance obligations under the agreement is 18 years. Upon execution of the agreement in November 2007, we received an initial upfront payment of $50 million from Shire which will be amortized to Collaboration Revenue over the period of the performance obligations which is contractually defined at 18 years.

Additionally, under the agreement we are entitled to receive reimbursement of up to 50% of research and development costs incurred in the development of the products covered by the agreement. We recognize revenue for reimbursed research and development costs in accordance with EITF Issue 99-19, *Reporting Revenue Gross as a Principal Versus Net as an Agent* (EITF 99-19). We will record the revenue associated with these reimbursable amounts to Research Revenue while the costs associated with these reimbursable amounts will be recorded in research and development expenses.

Accrued Expenses

As part of the process of preparing our financial statements, we are required to estimate accrued expenses. This process involves identifying services that have been performed on our behalf and estimating the level of service performed and the associated cost incurred for the service when we have not yet been invoiced or otherwise notified of actual cost. 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

Effective January 1, 2006, we adopted the fair value recognition provisions of Financial Accounting Standards Board (FASB) Statement of Financial Accounting Standards (SFAS) No. 123(R), *Share-Based Payment*, (SFAS No. 123(R)) using the prospective transition method. Under the prospective transition method, compensation expense is recognized in the financial statements on a prospective basis for all share-based payments granted subsequent to December 31, 2005, based upon the grant-date fair value estimated in accordance with the provisions of SFAS No. 123(R). Options granted prior to January 1, 2006, as a non-public company and accounted for using the intrinsic value method, will continue to be expensed over the vesting period. The fair value of awards expected to vest, as measured at grant date, is expensed on a straight-line basis over the vesting period of the related awards. Under the prospective transition method, results for prior periods are not restated.

We recognized employee stock-based compensation expense of $0.5 million, $3.3 million, and $4.0 million for the years ended 2005, 2006 and 2007, respectively.

Upon adoption of SFAS No. 123(R), we selected the Black-Scholes option pricing model as the most appropriate model for determining the estimated fair value for stock-based awards. The fair value of stock option awards subsequent to December 31, 2005 is amortized on a straight-line basis over the requisite service periods of the awards, which is generally the vesting period. 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 according to the SEC shortcut approach as described in SAB 107, *Disclosure about Fair Value of Financial Instruments* (SAB 107), which is 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:

	Years Ended December 31,	
	2006	2007
Expected stock price volatility	74.8%	78.3%
Risk free interest rate	4.7	4.5
Expected life of options (years)	6.25	6.25
Expected annual dividend per share	$ 0.00	$ 0.00

The weighted-average fair value (as of the date of grant) of the options granted during the years ended December 31, 2006 and 2007 was $10.20 and $9.45, respectively.

Prior to becoming a public company, the exercise prices for options granted were set by our board of directors, the members of which have extensive experience in the life sciences industry and all but one of whom are non-employee directors, with input from our management, based on our board's determination of the fair market value of our common stock at the time of the grants. In connection with the IPO, we performed a retrospective determination of fair value for financial reporting purposes of our common stock underlying stock option grants in 2005, 2006 and through April 2007, utilizing a combination of valuation methods described in the American Institute of Certified Public Accountants *Technical Practice Aid, Valuation of Privately-Held-Company Equity Securities Issued as Compensation*, (the Practice Aid). We utilized the same combination of valuation methods to perform contemporaneous valuations of our common stock for each quarter subsequent to March 31, 2006.

These assumptions and methods are more fully described in our Form S-1/A (333-141700) that was declared effective by the SEC in May 2007.

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

We calculated net loss per share in accordance with SFAS No. 128, *Earnings Per Share*. We have determined that the Series A, B, C, and D redeemable convertible preferred stock represented participating securities in accordance with EITF Issue No. 03-6 *Participating Securities and the Two — Class Method under FASB Statement No. 128*. However, because we operate at a loss, and losses are not allocated to the redeemable convertible preferred stock, the two class method does not affect our calculation of earnings per share. 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. As a result of the IPO in May 2007, all outstanding redeemable convertible preferred stock was converted to common stock and were no longer outstanding as of December 31, 2007.

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):

	Years Ended December 31,		
	2005	2006	2007
Historical			
Numerator:			
Net loss	$ (19,972)	$ (46,345)	$ (41,167)
Deemed dividend	—	(19,424)	—
Accretion of redeemable convertible preferred stock	(139)	(159)	(351)
Net loss attributable to common stockholders	$ (20,111)	$ (65,928)	$ (41,518)
Denominator:			
Weighted average common shares outstanding — basic and diluted	410,220	735,967	13,235,755

Dilutive common stock equivalents would include the dilutive effect of convertible securities, common stock options and warrants for common stock equivalents. Potentially dilutive common stock equivalents totaled approximately 9.5 million, 17.5 million and 24.9 million for the years ended December 31, 2005, 2006 and 2007, 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, 2007 Compared to Year Ended December 31, 2006

Research and Development Expense. Research and development expense was $31.1 million in 2007 representing a decrease of $2.5 million or 7% from $33.6 million in 2006. The variance was primarily attributable to a reduction in contract research and manufacturing costs due to the timing of studies of $4.5 million, partially offset by higher personnel costs of $1.6 million associated with headcount growth.

General and Administrative Expense. General and administrative expense was $15.3 million in 2007, an increase of $3.0 million or 24% from $12.3 million in 2006. The variance was primarily attributable to higher personnel costs of $2.0 million associated with headcount growth and higher professional fees of $0.3 million related primarily to finalizing the collaboration agreement with Shire.

Depreciation and Amortization. Depreciation and amortization expense was $1.2 million in 2007, and increase of $0.2 million or 20%, from $1.0 million in 2006. The increase is primarily due to assets acquired in the first nine months of 2007.

Interest Income and Interest Expense. Interest income was $5.1 million in 2007, compared to $2.0 million in 2006. The increase of $3.1 million or 155% was due to higher average cash and cash equivalents balances as a result of the issuance of the Series D redeemable convertible preferred stock, the $68.1 million of proceeds from the IPO and the receipt of the $50 million upfront licensing payment from Shire. Interest expense was $0.3 million in 2007, compared to $0.3 million in 2006.

Year Ended December 31, 2006 Compared to Year Ended December 31, 2005

Research and Development Expense. Research and development expense was $33.6 million in 2006, an increase of $19.9 million, or 145%, from $13.7 million in 2005. The increase was primarily attributable to third party direct project expenses, including increased contract research and manufacturing costs for Plicera and AT2220 of $9.6 million, an increase in personnel costs of $4.6 million associated with headcount and salary increases in our research, clinical, and regulatory functions and the impact of adopting SFAS 123(R), and other costs associated with licenses totaling $2.5 million as well as higher facility, supply, overhead, and non-program specific research.

General and Administrative Expense. General and administrative expense was $12.3 million in 2006, an increase of $5.4 million, or 78%, from $6.9 million in 2005. The increase resulted principally from an increase in personnel costs of $3.7 million attributable to increased headcount, a rise in salaries, and the impact of adopting SFAS 123(R).

Depreciation and Amortization. Depreciation and amortization expense was $1.0 million in 2006, and increase of $0.7 million or 233%, from $0.3 million in 2005. The increase is primarily due to leasehold improvements completed in late 2005 and early 2006 as well as purchases of equipment during 2006.

Interest Income and Interest Expense. Interest income was $2.0 million in 2006, compared to $0.6 million in 2005. The increase in interest income resulted from higher average cash and cash equivalents balances and higher average interest rates in 2006. Interest expense was $0.3 million in 2006, compared to $0.1 million in 2005. The increase in interest expense resulted from additional capital lease borrowings during 2006.

Other Expense. During 2006, we capitalized $1.2 million of costs directly attributable to the planned offering of our anticipated IPO. These costs were expensed when we withdrew our offering in the third quarter of 2006.

Tax Benefit. In 2005, we recognized tax benefits related to our sale of net operating losses in the New Jersey Tax Transfer Program. Our tax benefit was $0.6 million in 2005. We sold $6.7 million of net operating losses in 2005. We did not sell net operating losses in the New Jersey Tax Transfer Program in 2006 and therefore we did not recognize any tax benefits in 2006.

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 and $50.0 million from the non-refundable license fee from the Shire collaboration agreement in November 2007. The following table summarizes our significant funding sources as of December 31, 2007:

Funding	Year	No. Shares	Approximate Amount (1) (in thousands)
Series A Redeemable Convertible Preferred Stock	2002	444,443	$ 2,500
Series B Redeemable Convertible Preferred Stock	2004, 2005, 2006, 2007	4,917,853	31,189
Series C Redeemable Convertible Preferred Stock	2005, 2006	5,820,020	54,999
Series D Redeemable Convertible Preferred Stock	2006, 2007	4,930,405	60,000
Common Stock	2007	5,000,000	75,000
Upfront License Fee from Shire	2007	—	50,000
		21,112,721	$ 273,688

(1) Represents gross proceeds

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 2007 of $2.4 million.

As of December 31, 2007, we had cash and cash equivalents and marketable securities of $161.5 million. We hold our cash and investment balances in a variety of interest-bearing instruments, including obligations of U.S. government agencies and money market accounts. We invest cash in excess of our immediate requirements with regard to liquidity and capital preservation. Wherever possible, we seek to minimize the potential effects of concentration and degrees of risk.

Also, we maintain cash balances with financial institutions in excess of insured limits. We do not anticipate any losses with respect to such cash balances.

Net Cash Provided by and Used in Operating Activities

Net cash provided by operations for the year ended December 31, 2007 was $15.2 million due to the change in operating assets and liabilities of $51.0 million, non-cash charges for depreciation and amortization of $1.2 million, stock-based compensation of $4.0 million and the change in fair value of warrant liability of $0.2 million, offset by the net loss for the year ended December 31, 2007 of $41.2 million. The change in operating assets and liabilities of $51.0 million was due primarily to deferred revenue related to the $50 million upfront licensing payment from Shire.

Net cash used in operations for the year ended December 31, 2006 was $33.9 million. The net loss for the year ended December 31, 2006 of $46.3 million was offset primarily by non-cash charges for depreciation and amortization of $1.0 million, stock-based compensation of $3.3 million and stock-based license payment of $1.2 million and changes in operating assets and liabilities of $7.0 million.

Net Cash Used in Investing Activities

Net cash used in investing activities for the year ended December 31, 2007 was $75.0 million. Net cash used in investing activities reflects $200.7 million for the purchase of marketable securities and $0.7 million for the acquisition of property and equipment, offset by $126.4 million for the sale and redemption of marketable securities.

Net cash used in investing activities was $26.6 million for the year ended December 31, 2006. Net cash used in investing activities reflects $62.0 million cash used for the purchase of marketable securities and $2.0 million for the acquisition of property and equipment, offset by $37.4 million of cash provided by the sale and redemption of marketable securities.

Net Cash Provided by Financing Activities

Net cash provided by financing activities for the year ended December 31, 2007 was $91.9 million, consisting primarily of $24.1 million from the issuance of Series D redeemable convertible preferred stock, $68.1 million from the issuance of common stock, $0.5 million from asset financing arrangements, and $0.6 million proceeds from exercise of stock options and warrants offset by payments of equipment debt financing obligations of $1.4 million.

Net cash provided by financing activities for the year ended December 31, 2006 was $66.2 million. Net cash provided by financing activities mainly reflects $27.5 million of proceeds from the issuance of our Series C redeemable convertible preferred stock, $35.9 million of proceeds from the issuance of our Series D redeemable convertible preferred stock, $3.4 million of proceeds from our capital asset financing arrangement and $0.3 million proceeds from the exercise of stock options and warrants, offset by $0.9 million of payments of capital lease obligations.

Funding Requirements

We expect to incur losses from operations for the foreseeable future. We expect to incur increasing research and development expenses, including expenses related to the hiring of personnel and additional clinical trials. We expect that our general and administrative expenses will also increase as we expand our finance and administrative staff, add infrastructure, and incur additional costs related to being a public company, including directors' and officers' insurance, investor relations programs, and increased professional fees. Our future capital requirements will depend on a number of factors, including the continued progress of our research and development of products, the progress and results of our clinical trials, the duration and cost of discovery and preclinical development and laboratory testing and clinical trials for our product candidates, the timing and outcome of regulatory review of our product candidates, the number and development requirements of other product candidates that we pursue, the costs involved in preparing, filing, prosecuting, maintaining, defending, and enforcing patent claims and other intellectual property rights, the acquisition of licenses to new products or compounds, the status of competitive products, the availability of financing, our success in developing markets for our product candidates and the costs of commercialization activities, including product marketing, sales and distribution.

We believe that our existing cash and cash equivalents and short-term investments, together with the expected reimbursement of research and development expenses and research milestones from our collaboration with Shire, will be sufficient to enable us to fund our operating expenses and capital expenditure requirements at least until 2011.

We do not anticipate that we will generate revenue from commercial sales for at least the next several years. 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.

Financial Uncertainties Related to Potential Future Milestone Payments

We have acquired rights to develop and commercialize our product candidates through licenses granted by various parties. Two of these agreements contain milestone payments that are due with respect to Plicera only if certain specified pre-commercialization events occur. Amigal and AT2220 do not trigger such milestone payments. 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. In general, potential milestone payments for

Plicera may or may not be triggered under these licenses, and may vary in size, depending on a number of variables, almost all of which are currently uncertain.

The events that trigger these payments include:

- completion of Phase 2 clinical trials;
- commencement of Phase 3 clinical trials;
- submission of an NDA to the FDA or foreign equivalents; and
- receipt of marketing approval from the FDA or foreign equivalents.

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 Mt. Sinai School of Medicine (MSSM). We expect to pay royalties to all three licensors with respect to Plicera. To date, we have not made any royalty payments on sales of our products and believe we are several years away from selling any products that would require us to make any such royalty payments. 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. In conjunction with the $50 million upfront payment from Shire in November 2007, we recorded an accrual for its best estimate of royalties due to MSSM on the upfront payment.

Contractual Obligations

The following table summarizes our significant contractual obligations and commercial commitments at December 31, 2007 and the effects such obligations are expected to have on our liquidity and cash flows in future periods (in thousands).

	Total	Less than 1 Year	1-3 Years	3-5 Years	Over 5 Years
Operating lease obligations	$ 6,003	$ 1,655	$ 4,129	$ 219	—
Capital lease obligations	3,039	1,745	1,294	—	—
Employment agreement	1,851	1,388	463	—	—
Total fixed contractual obligations (1)	$ 10,893	$ 4,788	$ 5,886	$ 219	—

(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.

In May 2005, we entered into a seven-year, non-cancelable operating sublease agreement for office and laboratory space in Cranbury, New Jersey. The operating sublease will expire by its terms in February 2012. In August 2006, we entered into a sublease agreement for office space in an adjacent building. This sublease will expire by its terms in August 2009.

In August 2002, we entered into capital lease agreements that provide for up to $1 million of equipment financing through August 2004. The facility was increased to $3 million in May 2005 and to $5 million in November 2005. These financing arrangements include interest of approximately 9-12%, and lease terms of 36 or 48 months. Eligible assets under the lease lines include laboratory and scientific equipment, computer hardware and software, general office equipment, furniture and leasehold improvements. Upon termination of the lease agreements, we may renew the lease or purchase the leased equipment for $1.00. We also have the option to purchase the equipment at set prices before termination of the lease. In addition, at lease inception, we issued a warrant to the equipment financing lender to purchase 5,333 shares of common stock. These warrants were outstanding at December 31, 2005 and 2006. The warrants have an exercise price of $5.63 per share (adjusted for stock splits, stock dividends, etc.). The value of the warrants was calculated using the Black-Scholes option pricing model and was capitalized as debt issuance cost and amortized to interest expense over the term of the obligation. The value of the warrants and total charge to interest expense was not material for each of the years presented. The funding period of the facility expired by its own term in 2007.

On April 28, 2006, we entered into an employment agreement with our president and chief executive officer that provides for an annual base salary of $400,000, a cash bonus of up to 50% of base salary, an executive medical reimbursement contract, annual reimbursement up to $220,000 for medical expenses not covered by the executive medical reimbursement contract or our medical or health insurance policies, and gross up for federal and state income taxes of income tax incurred in connection with medical reimbursement. 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. The table above includes costs associated with the remainder of the second one-year term and third one-year term ending April 28, 2009. The cost of the executive medical reimbursement contract is estimated based on current premiums.

We have entered into agreements with clinical research organizations and other outside contractors who will be partially responsible for conducting and monitoring our clinical trials for Amigal, Plicera and AT2220. These contractual obligations are not reflected in the table above because we may terminate them without penalty.

Except for the Shire collaboration agreement described above, 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 have no off-balance sheet arrangements as of December 31, 2006 and 2007.

Recent Accounting Pronouncements

In December 2007, the EITF of the FASB reached a consensus on Issue No. 07-1, *Accounting for Collaborative Arrangements* (EITF 07-1). The EITF concluded on the definition of a collaborative arrangement and that revenues and costs incurred with third parties in connection with collaborative arrangements would be presented gross or net based on the criteria in EITF 99-19 and other accounting literature. Based on the nature of the arrangement, payments to or from collaborators would be evaluated and its terms, the nature of the entity's business, and whether those payments are within the scope of other accounting literature would be presented. Companies are also required to disclose the nature and purpose of collaborative arrangements along with the accounting policies and the classification and amounts of significant financial-statement amounts related to the arrangements. Activities in the arrangement conducted in a separate legal entity should be accounted for under other accounting literature; however required disclosure under EITF 07-1 applies to the entire collaborative agreement. This Issue is effective for financial statements issued for fiscal years beginning after December 15, 2008, and interim periods within those fiscal years, and is to be applied retrospectively to all periods presented for all collaborative arrangements existing as of the effective date. We do not expect this will have a significant impact on our financial statements.

In December 2007, the FASB issued SFAS No. 141(R), *Business Combinations* (SFAS No. 141(R)), which replaces SFAS No. 141, *Business Combinations*, requires an acquirer to recognize the assets acquired, the liabilities assumed, and any non-controlling interest in the acquiree at the acquisition date, measured at their fair values as of that date, with limited exceptions. This Statement also requires the acquirer in a business combination achieved in stages to recognize the identifiable assets and liabilities, as well as the non-controlling interest in the acquiree, at the full amounts of their fair values. SFAS No. 141(R) makes various other amendments to authoritative literature intended to provide additional guidance or to confirm the guidance in that literature to that provided in this Statement. This Statement applies prospectively to business combinations for which the acquisition date is on or after the beginning of the first annual reporting period beginning on or after December 15, 2008. We do not expect this will have a significant impact on our financial statements.

In December 2007, FASB issued SFAS No. 160, *Noncontrolling Interests in Consolidated Financial Statements* (SFAS No. 160), which amends Accounting Research Bulletin No. 51, *Consolidated Financial Statements*, to improve the relevance, comparability, and transparency of the financial information that a reporting entity provides in its consolidated financial statements. SFAS No. 160 establishes accounting and reporting standards that require the ownership interests in subsidiaries not held by the parent to be clearly identified, labeled and presented in the consolidated statement of financial position within equity, but separate from the parent's equity. This statement also requires the amount of consolidated net income attributable to the parent and to the non-controlling interest to be clearly identified and presented on the face of the consolidated statement of income. Changes in a parent's ownership interest while the parent retains its controlling financial interest must be accounted for consistently, and when a subsidiary is deconsolidated, any retained non-controlling equity investment in the former subsidiary must be initially measured at fair value. The gain or loss on the deconsolidation of the subsidiary is measured using the fair value of any non-controlling equity investment. The Statement also requires entities to provide sufficient disclosures that clearly identify and distinguish between the interests of the parent and the interests of the non-controlling owners. This Statement applies prospectively to all entities that prepare consolidated financial statements and applies prospectively for fiscal years, and interim periods within those fiscal years, beginning on or after December 15, 2008. We do not expect this will have a significant impact on our financial statements.

In June 2007, the EITF of the FASB reached a consensus on Issue No. 07-3, *Accounting for Nonrefundable Advance Payments for Goods or Services Received for Use in Future Research and Development Activities* (EITF 07-3). EITF 07-3 requires that non-refundable advance payments for goods or services that will be used or rendered for future research and

development activities should be deferred and capitalized. As the related goods are delivered or the services are performed, or when the goods or services are no longer expected to be provided, the deferred amounts would be recognized as an expense. This Issue is effective for financial statements issued for fiscal years beginning after December 15, 2007 and earlier application is not permitted. This consensus is to be applied prospectively for new contracts entered into on or after the effective date. The pronouncement is not expected to have a material effect on our financial statements.

In February 2007, the FASB issued SFAS No. 159, *The Fair Value Option for Financial Assets and Financial Liabilities—Including an amendment of FASB Statement No. 115* (SFAS No. 159), which is effective for fiscal years beginning after November 15, 2007. SFAS No. 159 permits the Company to choose to measure many financial instruments and certain other items at fair value. The objective is to improve financial reporting by providing entities with the opportunity to mitigate volatility in reported earnings caused by measuring related assets and liabilities differently without having to apply complex hedge accounting provisions. SFAS No. 159 is expected to expand the use of fair value measurement, which is consistent with the FASB's long-term measurement objectives for accounting for financial instruments. SFAS No. 159 is effective for fiscal year 2008 but early adoption is permitted. We are currently evaluating the impact, if any, that the adoption of SFAS No. 159 will have on our financial statements.

In September 2006, the FASB issued SFAS No. 157, *Fair Value Measures* (SFAS No. 157). SFAS No. 157 defines fair value, establishes a framework for measuring fair value and enhances disclosures about fair value measures required under other accounting pronouncements, but does not change existing guidance as to whether or not an instrument is carried at fair value. SFAS No. 157 is effective as of the beginning of the Company's 2008 fiscal year. We do not expect this will have a significant impact on our financial statements.

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

The primary objective of our investment activities is to preserve our capital to fund operations. We also seek to maximize income from our investments without assuming significant risk. To achieve our objectives, we maintain a portfolio of cash equivalents and investments in a variety of securities of high credit quality. As of December 31, 2007, we had cash and cash equivalents and investments in marketable securities of $161.5 million. A portion of our investments may be subject to interest rate risk and could fall in value if market interest rates increase. However, because our investments are short-term in duration, we believe that our exposure to interest rate risk is not significant and a 1% movement in market interest rates would not have a significant impact on the total value of our portfolio. We actively monitor changes in interest rates.

Item 8. FINANCIAL STATEMENTS AND SUPPLEMENTARY DATA.

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

	December 31, 2006	2007
Assets:		
Current assets:		
Cash and cash equivalents	$ 12,127	$ 44,188
Investments in marketable securities	42,572	117,339
Prepaid expenses and other current assets	321	1,513
Total current assets	55,020	163,040
Property and equipment, less accumulated depreciation and amortization of $1,557 and $2,793 at December 31, 2006 and 2007, respectively	4,358	3,790
Other non-current assets	267	267
Total Assets	$ 59,645	$ 167,097
Liabilities and Stockholders' (Deficiency) Equity		
Current liabilities:		
Accounts payable	$ 1,195	$ 530
Accrued expenses	7,704	9,935
Current portion of capital lease obligations	1,307	1,527
Current portion of deferred revenue	—	3,801
Total current liabilities	10,206	15,793
Warrant liability	609	—
Deferred revenue, less current portion	—	46,813
Capital lease obligations, less current portion	2,256	1,194
Commitments and contingencies		
Series A redeemable convertible preferred stock, $.01 par value, 444,443 shares authorized, issued and outstanding at December 31, 2006 (aggregate liquidation preference $2,500 at December 31, 2006), no shares authorized, issued, or outstanding at December 31, 2007	2,476	—
Series B redeemable convertible preferred stock, $.01 par value, 4,936,730 shares authorized, 4,877,056 shares issued and outstanding at December 31, 2006 (aggregate liquidation preference $31,000 at December 31, 2006), no shares authorized, issued, or outstanding at December 31, 2007	30,868	—
Series C redeemable convertible preferred stock, $.01 par value, 5,820,020 shares authorized, issued and outstanding at December 31, 2006 (aggregate liquidation preferences $54,999 at December 31, 2006), no shares authorized, issued, or outstanding at December 31, 2007	54,869	—
Series D redeemable convertible preferred stock, $.01 par value, 4,930,405 shares authorized, 2,953,878 issued and outstanding at December 31, 2006 (aggregate liquidation preference $36,000), no shares authorized, issued, or outstanding at December 31, 2007	35,876	—
Stockholders' (deficiency) equity:		
Common stock, $.01 par value, 21,333,333 shares authorized, 990,492 shares issued and outstanding at December 31, 2006, 50,000,000 shares authorized, 22,408,731 shares issued and outstanding at December 31, 2007	70	285
Additional paid-in capital	6,067	227,438
Accumulated other comprehensive income	15	408
Deficit accumulated during the development stage	(83,667)	(124,834)
Total stockholders' (deficiency) equity	(77,515)	103,297
Total Liabilities and Stockholders' (Deficiency) Equity	$ 59,645	$ 167,097

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 February 4, 2002 (Inception) to December 31, 2007
	2005	2006	2007	
Revenue:				
Research revenue	$ —	$ —	$ 1,375	$ 1,375
Collaboration revenue	—	—	409	409
Total revenue	—	—	1,784	1,784
Operating Expenses:				
Research and development	13,652	33,630	31,074	89,878
General and administrative	6,877	12,277	15,278	38,070
Impairment of leasehold improvements	—	—	—	1,030
Depreciation and amortization	303	952	1,237	2,794
In-process research and development	—	—	—	418
Total operating expenses	20,832	46,859	47,589	132,190
Loss from operations	(20,832)	(46,859)	(45,805)	(130,406)
Other income (expenses):				
Interest income	610	1,990	5,135	7,941
Interest expense	(82)	(273)	(348)	(1,430)
Change in fair value of warrant liability	(280)	(23)	(149)	(454)
Other expense	—	(1,180)	—	(1,180)
Loss before income tax benefit	(20,584)	(46,345)	(41,167)	(125,529)
Income tax benefit	612	—	—	695
Net loss	(19,972)	(46,345)	(41,167)	(124,834)
Deemed dividend	—	(19,424)	—	(19,424)
Preferred stock accretion	(139)	(159)	(351)	(802)
Net loss attributable to common stockholders	$ (20,111)	$ (65,928)	$ (41,518)	$ (145,060)
Net loss attributable to common stockholders per common share — basic and diluted	$ (49.02)	$ (89.58)	$ (3.14)	
Weighted-average common shares outstanding — basic and diluted	410,220	735,967	13,235,755	

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 five year period ended December 31, 2007
(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	—	—	—	—	365	—	365

compensation							
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 five year period ended December 31, 2007
(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

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

	Years Ended December 31,			Period from February 4, 2002 (Inception) to December 31, 2007
	2005	2006	2007	
Operating activities				
Net loss	$ (19,972)	$ (46,345)	$ (41,167)	$ (124,834)
Adjustments to reconcile net loss to net cash used in operating activities:				
Non-cash interest expense	—	—	—	525
Depreciation and amortization	303	952	1,237	2,792
Amortization of non-cash compensation	365	—	—	522
Stock-based compensation	—	2,816	3,823	6,638
Non-cash charge for stock based compensation issued to consultants	111	475	162	853
Change in fair value of warrant liability	281	22	149	454
Stock-based license payment	—	1,220	—	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	(286)	120	(1,192)	(1,513)
Other non-current assets	(491)	265	—	(288)
Account payable and accrued expenses	1,565	6,586	1,566	10,465
Deferred revenue	—	—	50,614	50,614
Net cash (used in)/provided by operating activities	(18,124)	(33,889)	15,192	(50,969)
Investing activities				
Sale and redemption of marketable securities	3,093	37,441	126,370	169,066
Purchases of marketable securities	(16,990)	(62,013)	(200,743)	(286,114)
Purchases of property and equipment	(3,041)	(2,031)	(669)	(7,611)
Net cash used in investing activities	(16,938)	(26,603)	(75,042)	(124,659)
Financing activities				
Proceeds from the issuance of preferred stock, net of issuance costs	40,316	63,371	24,053	143,022
Proceeds from issuance of common stock, net of issuance costs	—	—	68,093	68,093
Proceeds from the issuance of convertible notes	—	—	—	5,000
Payments of capital lease obligations	(273)	(881)	(1,388)	(2,866)
Payments from exercise of stock options	24	158	510	692
Proceeds from exercise of warrants (common and preferred)	75	91	97	264
Proceeds from capital asset financing arrangement	1,112	3,431	546	5,611
Net cash provided by financing activities	41,254	66,170	91,911	219,816
Net increase in cash and cash equivalents	6,192	5,678	32,061	44,188
Cash and cash equivalents at beginning of year/ period	257	6,449	12,127	—
Cash and cash equivalents at end of year/period	$ 6,449	$ 12,127	$ 44,188	$ 44,188
Supplemental disclosures of cash flow information				
Cash paid during the period for interest	$ 482	$ 273	$ 348	$ 1,136
Non-cash activities				
Conversion of preferred stock to common stock	$ —	$ —	$ 148,591	148,951
Conversion of notes payable to Series B redeemable convertible preferred stock	$ —	$ —	$ —	$ 5,000
Accretion of redeemable convertible preferred stock	$ 139	$ 159	$ 351	$ 802
Beneficial conversion feature related to issuance of the additional issuance of Series C redeemable convertible preferred stock	$ —	$ 19,424	$ —	$ 19,424

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 for the purpose of creating a premier drug development company at the forefront of therapy for human genetic diseases initially based on intellectual property in-licensed from Mount Sinai School of Medicine. 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 November 2007, the Company entered into a License and Collaboration Agreement with Shire Pharmaceuticals Ireland Ltd. (Shire). Under the agreement, the Company and Shire will jointly develop the Company's three lead pharmacological chaperone compounds for lysosomal storage disorders: Amigal (migalastat hydrochloride), Plicera (isofagomine tartrate) and AT2220. For further information, see "— Note 12. Development and Commercialization Agreement with Shire."

The Company has an accumulated deficit of approximately $124.8 million at December 31, 2007 and anticipates incurring losses through the year 2008 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), the upfront licensing payment from Shire and other financing arrangements. The Company believes that its existing cash and cash equivalents and short-term investments will be sufficient to covers its cash flow requirements for 2008.

2. Summary of Significant Accounting Policies

Reverse Stock Split

As a result of the 1:7.5 reverse stock split that became effective on May 24, 2007, every 7.5 shares of the Company's redeemable convertible preferred stock and common stock were combined into one share of the Company's redeemable convertible preferred stock and one share of common stock, respectively. All references to redeemable convertible preferred stock, redeemable convertible preferred stock outstanding, common stock, common shares outstanding, average number of common shares outstanding and per share amounts in these consolidated financial statements and notes to consolidated financial statements prior to the effective date of the reverse stock split have been restated to reflect the 1:7.5 reverse stock split on a retroactive basis.

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.

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.

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

Cash and Cash Equivalents

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.

Investment in Marketable Securities

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. In accordance with Financial Accounting Standards Board (FASB) Statement of Financial Accounting Standards (SFAS) No. 115, *Accounting for Certain Investments in Debt and Equity Securities*, 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. 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. No other than temporary impairment charges have been recorded in any of the years presented herein.

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.

Fair Value of Financial Instruments

SFAS No. 107, *Disclosures about Fair Value of Financial Instruments* (SFAS No. 107), requires disclosures of fair value information about financial instruments, whether or not recognized in the balance sheet, for which it is practicable to estimate that value. Due to the short-term nature, the carrying amounts reported in the financial statements approximate the fair value for cash and cash equivalents, accounts payable and accrued expenses. The estimated fair values of the Company's redeemable convertible preferred stock at December 31, 2006 was approximately $171.3 million, based on the September 2006 Series D redeemable convertible preferred stock price of $12.15 per share. The redeemable convertible preferred stock was converted into common stock of the Company upon completion of the Company's IPO. The warrants to purchase shares of Series B redeemable convertible preferred stock were recorded at fair value based on the Black-Scholes-Merton methodology and were valued at $0.6 million at December 31, 2006.

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.

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

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. The Company reported an impairment charge of $1.0 million during 2003 related to impaired capitalized leasehold improvements. There were no other impairment charges recognized during the years ended December 31, 2005, 2006 and 2007.

In-Process Research and Development

During 2002, the Company acquired certain development rights to intellectual property in the form of patent rights owned by Mount Sinai School of Medicine of New York University in exchange for 232,266 shares of common stock. The patent rights cover compounds that improve protein folding and protein stability.

The patent rights were reviewed to determine the stage of their development, the achievement of technological feasibility, and the technical milestones needed before commercialization is possible. It was determined, as of the acquisition date that each patent had significant technical risk associated with achieving the technological feasibility needed for U. S. Food and Drug Administration (FDA) approval and each patent has significant milestones to reach before commercialization is reasonably certain. It was also determined that all of the patents had no alternative future uses if they were not successful. Accordingly, the license was classified as in-process research and development and expensed immediately as of the acquisition date and included in research and development expense. The Company valued the acquired patents using fair value techniques, as a quoted market price was not available. The estimated fair value of the transfer at the date of the transaction was approximately $0.4 million.

Revenue Recognition

The Company recognizes revenue in accordance with the Securities and Exchange Commission (SEC) Staff Accounting Bulletin (SAB) No. 101, *Revenue Recognition in Financial* Statements (SAB 101), as amended by Staff Accounting Bulletin No. 104, *Revision of Topic 13* (SAB 104).

In determining the accounting for collaboration agreements, the Company follows the provisions of Emerging Issues Task Force (EITF) Issue 00-21, *Revenue Arrangements with Multiple Deliverables* (EITF 00-21). EITF 00-21 provides guidance on whether an arrangement involves multiple revenue-generating deliverables that should be accounted for as a single unit of accounting or divided into separate units of accounting for revenue recognition purposes and, if this division is required, how the arrangement consideration should be allocated among the separate units of accounting. If the arrangement represents a single unit of accounting, the revenue recognition policy and the performance obligation period must be determined (if not already contractually defined) for the entire arrangement. If the arrangement represents separate units of accounting according to the EITF's separation criteria, a revenue recognition policy must be determined for each unit. Revenues for non-refundable upfront license fee payments will be recognized on a straight line basis as Collaboration Revenue over the period of the performance obligations.

Revenues for research and development costs that are reimbursable under collaboration agreements in accordance with EITF Issue 99-19, *Reporting Revenue Gross as a Principal Versus Net as an Agent* (EITF 99-19) are recognized. The revenue associated with these reimbursable amounts 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 the risks and rewards as the principal in the research and development activities.

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.

Other Income and Expenses

During the second and third quarter of 2006, the Company deferred and capitalized $1.2 million of costs directly attributable to the planned offering of its securities as other non-current assets. These costs were recorded as other expenses when the planned offering was withdrawn during the third quarter of 2006.

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 carryforwards, 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.

In June 2006, the FASB issued FASB Interpretation No. 48, "*Accounting for Uncertainty in Income Taxes — an Interpretation of FASB Statement No. 109*" (FIN 48). FIN 48 addresses the accounting and disclosure of uncertain tax positions. FIN 48 prescribes a recognition threshold and measurement attribute for the financial statement recognition and measurement of a tax position taken or expected to be taken. The Company adopted FIN 48 on January 1, 2007 as required and determined that the adoption of FIN 48 did not have a material impact on the Company's financial position and results of operations.

Other Comprehensive Income/ (Loss)

SFAS No. 130, *Reporting Comprehensive Income*, requires components of other comprehensive income/(loss), including unrealized gains and losses on available-for-sale securities, to be included as part of total comprehensive income/(loss). The components of comprehensive gain/loss 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.

Redeemable Convertible Preferred Stock

The carrying value of redeemable convertible preferred stock is increased by periodic accretions so that the carrying amount will equal the redemption amount at the earliest redemption date. These increases are reflected through charges to additional paid-in capital since the Company does not have retained earnings. Redeemable convertible preferred stock was converted into common stock upon completion of the Company's IPO on a one-for-one basis.

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

Warrants to Purchase Redeemable Convertible Preferred Stock

The Company accounted for its warrants to purchase shares of its Series B redeemable convertible preferred stock (Series B Warrants) in accordance with FASB Staff Position 150-5: *Issuer's Accounting under FASB Statement No. 150 for Freestanding Warrants and Other Similar Instruments on Shares That Are Redeemable (FSP 150-5)*. As the Series B Preferred shares underling the warrants had redemption rights, the warrants to purchase Series B shares were classified as a liability. The Company measured the fair value of its warrant liability using the Black-Scholes option pricing model with changes in fair value recognized as non-operating income or expense. The value of the warrant liability at issuance was $0.4 million. In connection with the Company's IPO, the Series B warrants were exercised for 40,797 shares of Series B redeemable convertible preferred stock and automatically converted into common stock on a one-for-one basis.

Stock-Based Compensation

At December 31, 2007, the Company had two stock-based employee compensation plans, which are described more fully in "— Note 7. Stock Option Plans." Until May 2007, the Company had one stock-based employee compensation plan. Prior to January 1, 2006, the Company accounted for this plan under the recognition and measurement provisions of Accounting Principles Board Opinion No. 25, *Accounting for Stock Issued to Employees* (APB No. 25) and related interpretations, as permitted by SFAS No. 123, *Accounting for Stock-Based Compensation*. 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. Under the prospective transition method, compensation cost recognized for all stock-based payments granted subsequent to January 1, 2006 is based on the grant-date fair value estimated in accordance with the provisions of SFAS No. 123(R). Results for prior periods have not been restated. As a result of adopting SFAS No. 123(R) on January 1, 2006, net income for the year ended December 31, 2006 was less than it would have been had the Company continued to account for stock-based compensation under APB No. 25.

Effective January 1, 2006, the Company adopted the fair value recognition provisions of SFAS No. 123(R), *Share-Based Payment* (SFAS No. 123(R)), using the prospective transition method. Under the prospective transition method, compensation expense is recognized in the financial statements on a prospective basis for all share-based payments granted subsequent to January 1, 2006, based upon the grant-date fair value estimated in accordance with the provisions of SFAS No. 123(R). For options granted prior to January 1, 2006, as a non-public company and accounted for using the intrinsic value method, the Company will continue to expense any intrinsic value recognized over the vesting period. The grant-date fair value of awards expected to vest is expensed on a straight-line basis over the vesting period of the related awards. Under the prospective transition method, results for prior periods are not restated and pro forma disclosures for outstanding awards accounted for under the intrinsic value method of APB No. 25 are not presented since the Company used the minimum value method for pro forma disclosure purposes prior to January 1, 2006.

Prior to the adoption of SFAS No. 123(R), the Company presented its unamortized portion of deferred compensation cost for non-vested stock options in the statement of changes in stockholders' (deficiency) equity with a corresponding credit to additional paid in capital. Upon the adoption of SFAS No. 123(R), these amounts were offset against each other. Under SFAS No. 123(R), an equity instrument is not considered to be issued until the instrument vests. As a result, compensation cost is recognized over the requisite service period with an offsetting credit to additional paid in capital, and the deferred compensation balance of $2.5 million at January 1, 2006 was net against additional paid in capital during the first quarter of 2006.

During the years ended December 31, 2005, 2006 and 2007, the Company recorded total employee stock-based compensation expense of approximately $0.5 million, $3.3 million and $4.0 million, respectively. During the year ended December 31, 2006, the Company recorded incremental stock-based compensation expense of approximately $2.2 million ($2.99 per basic and diluted share) related to expensing of stock options under SFAS No. 123(R). Stock-based compensation expense had no impact on the Company's cash flows from operations and financing activities.

SFAS No. 123(R) does not change the accounting guidance for how the Company accounts for options issued to non-employees. The Company accounts for options issued to non-employees in accordance with SFAS No. 123 and EITF Issue No. 96-18, *Accounting for Equity Instruments That Are Issued to Other Than Employees for Acquiring, or in Conjunction with Selling, Goods or Services* (EITF 96-18). As such, the value of such options is periodically re-measured and income or expense is recognized during the vesting terms.

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Upon adoption of SFAS No. 123(R), the Company selected the Black-Scholes option pricing model as the most appropriate model for determining the estimated fair value for stock-based awards. The fair value is then amortized on a straight-line basis over the requisite service periods of the awards, which is generally the vesting period. 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 weighted average approach using its own historical volatility and other similar public entity volatility information until historical volatility of the Company is relevant to measure expected volatility for future option grants. The average expected life was determined according to the SEC shortcut approach as described in SAB No. 107, *Share-Based Payment* (SAB No. 107), which is 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:

	Years Ended December 31, 2006	2007
Expected stock price volatility	74.8%	78.3%
Risk free interest rate	4.7	4.5
Expected life of options (years)	6.25	6.25
Expected annual dividend per share	$0.00	$0.00

Beneficial Conversion Charges

When the Company issues debt or equity securities which are convertible into common stock at a discount from the common stock fair value at the date the debt or equity financing is committed, a beneficial conversion charge is measured as the difference between the closing price and the conversion price at the commitment date. The beneficial conversion charge is presented as a discount or reduction to the related security, with an offsetting amount increasing additional paid-in capital. The Company also recorded a beneficial conversion charge (also referred to as a deemed dividend) during April of 2006 of approximately $19.4 million related to the issuance of certain shares of Series C redeemable convertible preferred stock. The beneficial conversion charge for equity instruments is recorded with offsetting charges and credits to additional paid in capital with no effect on total shareholder equity. The Series C investors committed to finance the additional issuance of the Series C redeemable convertible preferred stock on March 31, 2006. The estimated fair value of the common stock was approximately $16.13 per share at the commitment date of the additional issuance and the beneficial conversion charge was recognized upon issuance of the Series C redeemable convertible preferred stock as such stock could be converted upon issuance. The Company did not record a beneficial conversion charge for any other redeemable convertible preferred stock issuances as the common stock fair value was less than the conversion price of each offering on the respective commitment dates of those offerings.

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

The Company calculates net loss per share in accordance with SFAS No. 128, *Earnings Per Share*. The Company has determined that its Series A, B, C, and D redeemable convertible preferred stock represent participating securities in accordance with EITF Issue No. 03-6, *Participating Securities and the Two — Class Method under FASB Statement No. 128* . However, since the Company operates at a loss, and losses are not allocated to the redeemable convertible preferred stock, the two class method does not affect the Company's calculation of earnings per share. The Company has 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.

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Notes To Consolidated Financial Statements — (Continued)

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 and pro forma net loss attributable to common stockholders per common share (in thousands except share amounts):

	Years Ended December 31,		
	2005	2006	2007
Historical			
Numerator:			
Net loss	$ (19,972)	$ (46,345)	$ (41,167)
Deemed dividend	—	(19,424)	—
Accretion of redeemable convertible preferred stock	(139)	(159)	(351)
Net loss attributable to common stockholders	$ (20,111)	$ (65,928)	$ (41,518)
Denominator:			
Weighted average common shares outstanding — basic and diluted	410,220	735,967	13,235,755

Dilutive common stock equivalents would include the dilutive effect of convertible securities, common stock options and warrants for common stock equivalents. Potentially dilutive common stock equivalents totaled approximately 9.5 million, 17.5 million and 24.9 million for the years ended December 31, 2005, 2006 and 2007, 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 do not foresee payment of a dividend in any upcoming fiscal period.

Recent Accounting Pronouncements

In December 2007, the EITF of the FASB reached a consensus on issue No. 07-1, *Accounting for Collaborative Arrangements* (EITF 07-1). The EITF concluded on the definition of a collaborative arrangement and that revenues and costs incurred with third parties in connection with collaborative arrangements would be presented gross or net based on the criteria in EITF 99-19 and other accounting literature. Based on the nature of the arrangement, payments to or from collaborators would be evaluated and its terms, the nature of the entity's business, and whether those payments are within the scope of other accounting literature would be presented. Companies are also required to disclose the nature and purpose of collaborative arrangements along with the accounting policies and the classification and amounts of significant financial-statement balances related to the arrangements. Activities in the arrangement conducted in a separate legal entity should be accounted for under other accounting literature; however required disclosure under EITF 07-1 applies to the entire collaborative agreement. This Issue is effective for financial statements issued for fiscal years beginning after December 15, 2008, and interim periods within those fiscal years, and is to be applied retrospectively to all periods presented for all collaborative arrangements existing as of the effective date. The Company does not expect this will have a significant impact on the financial statements of the Company.

In December 2007, the FASB issued SFAS No. 141(R), *Business Combinations* (SFAS No. 141(R)), which replaces SFAS No. 141, *Business Combinations*, requires an acquirer to recognize the assets acquired, the liabilities assumed, and any non-controlling interest in the acquiree at the acquisition date, measured at their fair values as of that date, with limited exceptions. This Statement also requires the acquirer in a business combination achieved in stages to recognize the identifiable assets and liabilities, as well as the non-controlling interest in the acquiree, at the full amounts of their fair values. SFAS No. 141(R) makes various other amendments to authoritative literature intended to provide additional guidance or to confirm the guidance in that literature to that provided in this Statement. This Statement applies prospectively to business combinations for which the acquisition date is on or after the beginning of the first annual reporting period beginning on or after December 15, 2008. The Company does not expect this will have a significant impact on the financial statements of the Company.

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In December 2007, FASB issued SFAS No. 160, *Noncontrolling Interests in Consolidated Financial Statements* (SFAS No. 160), which amends Accounting Research Bulletin No. 51, *Consolidated Financial Statements*, to improve the relevance, comparability, and transparency of the financial information that a reporting entity provides in its consolidated financial statements. SFAS No. 160 establishes accounting and reporting standards that require the ownership interests in subsidiaries not held by the parent to be clearly identified, labeled and presented in the consolidated statement of financial position within equity, but separate from the parent's equity. This statement also requires the amount of consolidated net income attributable to the parent and to the non-controlling interest to be clearly identified and presented on the face of the consolidated statement of income. Changes in a parent's ownership interest while the parent retains its controlling financial interest must be accounted for consistently, and when a subsidiary is deconsolidated, any retained non-controlling equity investment in the former subsidiary must be initially measured at fair value. The gain or loss on the deconsolidation of the subsidiary is measured using the fair value of any non-controlling equity investment. The Statement also requires entities to provide sufficient disclosures that clearly identify and distinguish between the interests of the parent and the interests of the non-controlling owners. This Statement applies prospectively to all entities that prepare consolidated financial statements and applies prospectively for fiscal years, and interim periods within those fiscal years, beginning on or after December 15, 2008. The Company does not expect this will have a significant impact on the financial statements of the Company.

In June 2007, the EITF of the FASB reached a consensus on Issue No. 07-3, *Accounting for Nonrefundable Advance Payments for Goods or Services Received for Use in Future Research and Development Activities* (EITF 07-3). EITF 07-3 requires that non-refundable advance payments for goods or services that will be used or rendered for future research and development activities should be deferred and capitalized. As the related goods are delivered or the services are performed, or when the goods or services are no longer expected to be provided, the deferred amounts would be recognized as an expense. This Issue is effective for financial statements issued for fiscal years beginning after December 15, 2007 and earlier application is not permitted. This consensus is to be applied prospectively for new contracts entered into on or after the effective date. The pronouncement is not expected to have a material effect on the financial statements of the Company.

In February 2007, the FASB issued SFAS No. 159, *The Fair Value Option for Financial Assets and Financial Liabilities—Including an amendment of FASB Statement No. 115* (SFAS No. 159), which is effective for fiscal years beginning after November 15, 2007. SFAS No. 159 permits the Company to choose to measure many financial instruments and certain other items at fair value. The objective is to improve financial reporting by providing entities with the opportunity to mitigate volatility in reported earnings caused by measuring related assets and liabilities differently without having to apply complex hedge accounting provisions. SFAS No. 159 is expected to expand the use of fair value measurement, which is consistent with the FASB's long-term measurement objectives for accounting for financial instruments. SFAS No. 159 is effective for fiscal year 2008 but early adoption is permitted. The Company is currently evaluating the impact, if any, that the adoption of SFAS No. 159 will have on the Company's financial statements.

In September 2006, the FASB issued SFAS No. 157, *Fair Value Measures* (SFAS No. 157). SFAS No. 157 defines fair value, establishes a framework for measuring fair value and enhances disclosures about fair value measures required under other accounting pronouncements, but does not change existing guidance as to whether or not an instrument is carried at fair value. SFAS No. 157 is effective as of the beginning of the Company's 2008 fiscal year. The Company does not expect this will have a significant impact on the financial statements of the Company.

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 as defined by SFAS No. 131, *Disclosure About Segments of an Enterprise and Related Information.*

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Notes To Consolidated Financial Statements — (Continued)

3. Investments in Marketable Securities

The following is a summary of available for sale securities held by the Company (in thousands):

	Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
December 31, 2006				
Corporate Debt Securities	$ 42,557	$ 16	$ (1)	$ 42,572
December 31, 2007				
Corporate Debt Securities	$ 116,931	$ 423	$ (15)	$ 117,339

All of the Company's available for sale investments as of December 31, 2006 and 2007 are due in one year or less.

Unrealized gains and losses are reported as a component of accumulated other comprehensive gain/loss in stockholders' (deficiency) equity. For the years ended December 31, 2006 and 2007, unrealized holding gains included in accumulated other comprehensive income/(loss) were $0.03 million and $0.4 million, respectively.

For the years ended December 31, 2006 and 2007, there were no realized gains or losses. The cost of securities sold is based on specific identification method.

Unrealized loss positions in the available for sale securities as of December 31, 2006 and 2007 reflect temporary impairments that have not been recognized and have been in a loss position for less than twelve months. The fair value of these available for sale securities in unrealized loss positions was $4.8 million and $14.7 million as of December 31, 2006 and 2007, respectively.

Unrealized gains and losses in the Company's portfolio relate to fixed income debt securities. For these securities, the unrealized losses are due to increases in interest rates. There are no changes in credit risk of the debt securities. The Company has concluded that the unrealized losses in its marketable securities are not other-than-temporary as the Company has the ability to hold the securities to maturity or a planned forecasted recovery.

4. Property and Equipment

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

	December 31, 2006	December 31, 2007
Property and equipment consist of the following:		
Computer equipment	$ 564	$ 694
Computer software	105	167
Research equipment	2,685	3,089
Furniture and fixtures	525	579
Leasehold improvements	2,036	2,054
	5,915	6,583
Less accumulated depreciation and amortization	(1,557)	(2,793)
	$ 4,358	$ 3,790

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Notes To Consolidated Financial Statements — (Continued)

Included in property and equipment are costs capitalized pursuant to capital lease obligations of $4.8 million and $5.5 million at December 31, 2006 and 2007. Depreciation and amortization expense relating to the capital lease obligations was $0.1 million, $0.8 million, $1.1 million and $2.0 million for the years ended December 31, 2005, 2006, and 2007, and for the Period February 4, 2002 (inception) to December 31, 2007, respectively.

5. Accrued Expenses

Accrued expenses consist of the following (in thousands):

	December 31, 2006	December 31, 2007
Accrued professional fees	$ 253	$ 979
Accrued contract manufacturing & contract research costs	5,682	6,035
Accrued compensation and benefits	1,236	2,279
Accrued facility costs	482	364
Accrued other	51	278
	$ 7,704	$ 9,935

6. Capital Structure

Common Stock

As of December 31, 2007, 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 June 2007, the Company closed its IPO of 5,000,000 shares of its common stock at a public offering price of $15.00 per share. Net proceeds to the Company were approximately $68.1 million, after deducting underwriting discounts, commissions and offering expenses totaling approximately $6.9 million. In connection with the initial public offering, the outstanding shares of Series A redeemable convertible preferred stock were converted into 444,443 shares of common stock, the outstanding shares of Series B redeemable convertible preferred stock were converted into 4,877,056 shares of common stock, the outstanding shares of Series C redeemable convertible preferred stock were converted into 5,820,020 shares of common stock and the outstanding shares of Series D redeemable convertible preferred stock were converted into 4,930,405 shares of common stock. In connection with the IPO, the outstanding warrants to purchase Series B redeemable convertible preferred stock were automatically exercised and the shares of Series B redeemable convertible preferred stock automatically converted into 40,797 shares of the common stock. As a result, the Company no longer recognizes accretion expense for preferred stock or non-operating income or expense for changes in the fair value of the warrant liability.

In connection with an employment agreement and director compensation agreement, the Company issued 53,333 shares of common stock in return for services in 2005. The shares will vest over three and four year periods. The Company recorded $0, $0.04 million and $0.1 million as compensation expense during 2005, 2006 and 2007, respectively, in connection with the issuance of these restricted shares.

In connection with the formation of the Company, the Company issued 232,266 shares of common stock to the Mount Sinai School of Medicine of New York University (MSSM) in exchange for exclusive license rights for certain intellectual property. The value of the shares was accounted for as in-process research and development; see “— Note 2. Summary of Significant Accounting Policies — In-Process Research and Development.” In October of 2006, the Company amended its license agreement 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 valued at $1.2 million to MSSM.

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Notes To Consolidated Financial Statements — (Continued)

Redeemable Convertible Preferred Stock

In June 2007 in connection with the IPO, all outstanding redeemable convertible preferred stock was converted to common stock. As of the IPO conversion date, Series A, Series B, Series C, and Series D were converted at their respected stated values (estimated fair value of $5.63 per share, $6.32 per share, $9.45 per share, and $12.17 per share, respectively, less issuance costs and accretion adjustments).

	Series A		Series B		Series C		Series D	
	Shares	Amount (in thousands)	Shares	Amount (in thousands)	Shares	Amount (in thousands)	Shares	Amount (in thousands)
Balance at February 4, 2002 (inception)	—	$ —	—	$ —	—	$ —	—	$ —
Issuance of Series A at $5.63 per share	444,443	2,500	—	—	—	—	—	—
Issuance costs	—	(95)	—	—	—	—	—	—
Accretion	—	10	—	—	—	—	—	—
Balance at December 31, 2002	444,443	2,415	—	—	—	—	—	—
Accretion	—	17	—	—	—	—	—	—
Balance at December 31, 2003	444,443	2,432	—	—	—	—	—	—
Issuance of Series B at $6.38 per share	—	—	2,823,523	18,000	—	—	—	—
Issuance cost	—	—	—	(122)	—	—	—	—
Issuance of warrants with Series B	—	—	—	(422)	—	—	—	—
Accretion	—	17	—	109	—	—	—	—
Balance at December 31, 2004	444,443	2,449	2,823,523	17,565	—	—	—	—
Issuance of Series B at $6.38 per share	—	—	2,039,211	13,000	—	—	—	—
Issuance cost	—	—	—	(6)	—	—	—	—
Issuance of Series C at $9.45 per share	—	—	—	—	2,910,010	27,500	—	—
Issuance cost	—	—	—	—	—	(178)	—	—
Accretion	—	17	—	110	—	12	—	—
Balance at December 31, 2005	444,443	2,466	4,862,734	30,669	2,910,010	27,334	—	—

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Notes To Consolidated Financial Statements — (Continued)

	Series A		Series B		Series C		Series D	
	Shares	Amount (in thousands)	Shares	Amount (in thousands)	Shares	Amount (in thousands)	Shares	Amount (in thousands)
Balance at December 31, 2005	444,443	2,466	4,862,734	30,669	2,910,010	27,334	—	—
Exercise of warrants with Series B at $6.38	—	—	14,322	91	—	—	—	—
Issuance of Series C at $9.45 per share	—	—	—	—	2,910,010	27,500	—	—
Issuance of Series D at $12.15 per share	—	—	—	—	—	—	2,953,878	35,947
Issuance cost	—	—	—	—	—	—	—	(76)
Accretion to redemption value	—	10	—	108	—	35	—	5
Balance at December 31, 2006	444,443	2,476	4,877,056	30,868	5,820,020	54,869	2,953,878	35,876
Issuance of Series D at $12.15 per share	—	—	—	—	—	—	1,976,527	24,053
Series B warrant exercise	—	—	40,797	98	—	—	—	—
Accretion to redemption value	—	24	—	126	—	130	—	71
Conversion of preferred stock to common	(444,443)	(2,500)	(4,917,853)	(31,092)	(5,820,020)	(54,999)	(4,930,405)	(60,000)
Balance at December 31, 2007	—	$ —	—	$ —	—	$ —	—	$ —

Warrants

During 2002, the Company issued 5,333 common stock warrants to a vendor as part of a capital lease agreement. These warrants were outstanding at December 31, 2005 and 2006. The warrants have an exercise price of $5.63 per share (adjusted for stock splits, stock dividends, etc.). The value of the warrants was calculated using the Black-Scholes option pricing model and was capitalized as debt issuance cost and amortized to interest expense over the term of the obligation. The value of the warrants and total charge to interest expense was not material for each of the years presented.

In 2003, the Company issued 133,332 common stock warrants to certain investors in connection with its Bridge Loans. The warrants had an exercise price of $0.56 per share (adjusted for stock splits, stock dividends, etc.). The value of the warrants of $0.2 million was calculated using the Black-Scholes option pricing model and was accounted for as debt discount and amortized to interest expense over the term of the loans. These same warrant shares were exercised in 2005. The total charge to interest expense was $0.1 million for the year ended December 31, 2004.

In 2004, the Company issued warrants to purchase 73,996 Series B shares to certain investors as part of the Series B financing. The warrants had an exercise price of $6.38 per share (adjusted for stock splits, stock dividends, etc.) and could have been exercised for cash or net shares at the option of the warrant holders. During 2006 there were 14,322 warrants exercised for Series B shares. In connection with the IPO, the remaining 40,797 warrants were automatically exercised and converted into 40,797 common shares. As the Series B Preferred shares underling the warrants had redemption rights, the warrants to purchase Series B shares were classified as a liability in accordance with FSP 150-5.

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The Company measures the fair value of its warrant liability using the Black-Scholes option pricing model with changes in fair value recognized in earnings. The value of the warrant liability at issuance was $0.4 million. The Company recognized changes in the fair value of the warrant liability as non-operating income or (expense) of $0, $(0.3 million), and $(0) in 2004, 2005, and 2006, respectively. In connection with the Company's IPO, the Series B warrants were exercised for 40,797 shares of Series B redeemable convertible preferred stock and automatically converted into common stock on a one-for-one basis.

7. 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). 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 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 the 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.

As of December 31, 2007, the Company reserved up to 3,168,000 shares for issuance under the 2002 Plan and 966,667 shares under the 2007 Plan.

The Company recognized stock-based compensation expense of $0.5 million, $3.3 million and $4.0 million in 2005, 2006 and 2007, respectively. In 2006, research and development expense and general and administrative expense include $1.7 million and $1.6 million of stock compensation expense, respectively. In 2007, research and development expense and general and administrative expense include $1.6 million and $2.4 million of stock compensation expense, respectively.

Upon adoption of SFAS No. 123(R) on January 1, 2006, the Company selected the Black-Scholes option pricing model as the most appropriate model for determining the estimated fair value for stock-based awards. The fair value of stock option awards subsequent to December 31, 2005 is amortized on a straight-line basis over the requisite service periods of the awards, which is generally the vesting period. 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. The Company 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 according to the SEC shortcut approach as described in SAB No. 107, which is 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 grant-date fair value per share of options granted during 2006 and 2007 were $10.20 and $9.45, respectively. The weighted average assumptions used in the Black-Scholes option pricing model are as follows:

	Years Ended December 31,	
	2006	2007
Expected stock price volatility	74.8%	78.3%
Risk free interest rate	4.7%	4.5%
Expected life of options (years)	6.25	6.25
Expected annual dividend per share	$0.00	$0.00

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Notes To Consolidated Financial Statements — (Continued)

The following table summarizes information about stock options outstanding:

	Number of Shares (in thousands)	Weighted Average Exercise Price	Weighted Average Remaining Contractual Life	Aggregate Intrinsic Value (in millions)
Options outstanding, December 31, 2004	426.6	$ 0.45		
Granted	1,010.2	$ 2.17		
Exercised	(97.2)	0.22		
Forfeited	(102.5)	$ 0.45		
Options outstanding, December 31, 2005	1,237.1	$ 2.10		
Granted	1,005.1	$ 6.00		
Exercised	(265.8)	$ 0.60		
Forfeited	(108.0)	$ 2.20		
Options outstanding, December 31, 2006	1,868.4	$ 4.27		
Granted	1,035.6	$ 13.16		
Exercised	(308.6)	$ 1.80		
Forfeited	(152.2)	$ 8.94		
Options outstanding, December 31, 2007	2,443.2	$ 8.08	8.3 years	$ 9.0
Vested and unvested expected to vest, December 31, 2007	2,214.0	$ 7.78	8.2 years	$ 7.1
Exercisable at December 31, 2007	746.6	$ 4.66	7.2 years	$ 4.5

The aggregate intrinsic value of options exercised during the years ended December 31, 2005, 2006, and 2007, was $0.1 million, $2.5 million and $2.7 million, respectively. As of December 31, 2007, the total unrecognized compensation cost related to non-vested stock options granted was $11.8 million and is expected to be recognized over a weighted average period of 2.8 years. Cash proceeds from stock options exercised during the years ended December 31, 2005, 2006 and 2007 was $0, $0.2 million and $0.5 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.

The following table summarizes the Company's restricted stock activity as of and for the year ended December 31, 2007:

	Restricted Stock	
	Number of Shares (in thousands)	Weighted Average Grant Date Fair Value
Unvested at December 31, 2006	51.1	$ 8.94
Granted	—	$ —
Vested	(16.1)	$ 8.88
Forfeited	—	$ —
Unvested at December 31, 2007	35.0	$ 8.96

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Upon vesting, 3,085 shares were surrendered by the Company to fund the Company's minimum statutory tax withholding requirements. There were no restricted stock awards in 2007 or prior to 2006. As of December 31, 2007, the total unrecognized compensation cost related to unvested restricted stock awards was $0.3 million. This cost is expected to be recognized over a weighted average period of 2.6 years. The total fair value of restricted stock awards which vested during 2007 was $0.1 million.

8. 401(k) Plan

The Company has a 401(k) plan (the Plan) covering all eligible employees. The Plan allows for a discretionary employer match. Through December 31, 2007, the Company has not made any match of employee contributions. 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.

9. Leases

Operating Leases

On May 12, 2005, the Company entered into a Sublease Agreement for its Corporate Office in Cranbury, NJ. The sublease term will expire on February 28, 2012 or on such earlier date upon mutual agreement of both parties. On August 14, 2006, the Company entered into another sublease agreement to expand office space in an adjacent building. This sublease term will expire on August 31, 2009 or on such earlier date upon mutual agreement of both parties. At December 31, 2007, aggregate annual future minimum lease payments under these leases are as follows (in thousands):

Operating Leases Years ending December 31:	
2008	$ 1,655
2009	1,527
2010	1,295
2011	1,307
2012	219
	$ 6,003

Rent expense for the years ended December 31, 2005, 2006 and 2007 were $1.0 million, $1.6 million and $1.8 million, respectively.

Capital Lease Facility

In August 2002, the Company entered into financing agreements that provides 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 2005. These financing arrangements include interest of approximately 9-12%, and lease terms of 36 or 48 months. Eligible assets under the lease lines include laboratory and scientific equipment, computer hardware and software, general office equipment, furniture, and leasehold improvements.

At December 31, 2006, the total amount available to the Company under these agreements is $1.4 million. The funding period of the facility expired by its own term in 2007.

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The remaining future minimum payments due for all non-cancelable capital leases as of December 31, 2007 are as follows (in thousands):

Capital Leases Years ending December 31:	
2008	$ 1,745
2009	957
2010	295
2011	42
	3,039
Less payments for interest	(318)
Total principal obligation	2,721
Less short-term portion	(1,527)
Long-term portion	$ 1,194

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

10. Income Taxes

In June 2006, the FASB issued FIN 48 to create a single model to address accounting for uncertainty in tax positions. FIN 48 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. FIN 48 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, disclosures and transition relating to the adoption of the new accounting standard. FIN 48 is effective for fiscal years beginning after December 15, 2006. The Company adopted FIN 48 as of January 1, 2007, as required and determined that the adoption of FIN 48 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, 2007 and did not accrue for interest or penalties as of December 31, 2007. The Company does not have an accrual for uncertain tax positions as of December 31, 2007. Tax returns for all years 2002 and thereafter are subject to future examination by tax authorities.

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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):

	For Years Ended December 31,		
	2005	2006	2007
Current deferred tax asset			
Non — cash stock issue to consultants	$ 64	$ 246	$ 283
Others	33	1,309	1,232
	97	1,555	1,515
Non — current deferred tax assets			
Amortization/depreciation	132	1,289	1,129
Research tax credit	1,344	3,611	5,403
Net operating loss carry forwards	14,464	27,257	42,282
Others	29	121	478
Total deferred tax asset	16,066	33,833	50,807
Non — current deferred tax liability			
Depreciation	(57)	—	—
Total net deferred tax asset	16,009	33,833	50,807
Less valuation allowance	(16,009)	(33,833)	(50,807)
Net deferred tax asset	$ —	$ —	$ —

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, 2005, 2006, and 2007, the Company recorded valuation allowances of $16.0 million, $33.8 million and $50.8 million, respectively, representing a change in the valuation allowance of $17.8 million and $17.0 million for the two previous fiscal year-ends, 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, 2007, the Company had federal and state net operating loss carry forwards of approximately $106 million and $103 million, respectively. The federal carry forward will begin to expire in 2024 and will end in 2028. The state carry forward will begin to expire in 2012 and will end in 2015. Utilization of the net operating loss carry forwards and credits may be subject to a substantial annual limitation due to the ownership change limitations provided by the Internal Revenue Code of 1986, as amended, and similar state provisions. The annual limitation may result in the expiration of net operating losses and credits before utilization. The Company is conducting an analysis to determine if there has been a “change in ownership” as defined by the Tax Reform Act of 1986. This analysis does not impact the 2007 financial statements.

The Company recognized a tax benefit of $0.6 million in connection with the sale of net operating losses in the New Jersey Tax Transfer Program during the year ended December 31, 2005.

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A reconciliation of the statutory tax rates and the effective tax rates for the years ended December 31, 2005, 2006 and 2007 are as follows:

	Years Ended December 31,		
	2005	2006	2007
Statutory rate	(34)%	(34)%	(34)%
State taxes, net of federal benefit	(6)	(6)	(5)
Permanent adjustments	1	1	3
Non deductible interest	—	—	—
R&D credit	(3)	(4)	(4)
Other	(1)	2	(1)
Benefit from sale of net operating loss	(3)	—	—
Valuation allowance	43	41	41
Net	(3)%	0%	0%

Income tax benefit consisted of the following components (in thousands):

	Years Ended December 31,		
	2005	2006	2007
Current benefit:			
Federal	$ —	$ —	$ —
State	(612)	—	—
Deferred:			
Federal	—	—	—
State	—	—	—
Income tax benefit	$ (612)	$ —	$ —

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 treatment of diseases which can be achieved by enhancing lysosomal enzyme activity 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 if a foreign patent is granted and 2018 otherwise, subject to any patent term extension that may be granted. Under this agreement, the Company has no milestone or future payments other than royalties on net sales. In conjunction with the $50 million upfront payment from Shire in November 2007, the Company recorded an accrual for its best estimate of royalties due to MSSM on the upfront payment.

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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 $29,500, 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 2 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 expects to pay royalties to all three licensors with respect to Plicera.

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. Development and Commercialization Agreement with Shire

In November 2007, the Company entered into a License and Collaboration Agreement with Shire. Under the agreement, the Company and Shire will jointly develop 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. The Company retains 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 will be shared 50/50 going forward. In addition, The Company is eligible to receive, for all three drug product candidates, aggregate potential milestone payments of up to $150 million if certain clinical and regulatory milestones are achieved for all three of the programs, and $240 million in sales-based milestones. The Company will also be eligible to receive tiered double-digit royalties on net sales of the products which are marketed outside of the U.S.

In accordance with the guidance in EITF 00-21, the Company determined that its various deliverables due under the collaboration agreement represent as a single unit of accounting for revenue recognition purposes. The initial, non-refundable upfront license fee payment of $50 million will be recognized on a straight line basis as Collaboration Revenue over the period of the performance obligations. The Company determined that the period of performance obligations is 18 years as contractually defined.

Under the collaboration agreement, the Company will also receive reimbursement of up to 50% of research and development costs incurred in the development of the products covered by the agreement. The Company will recognize revenue for reimbursed research and development costs in accordance with EITF Issue 99-19 as Research Revenue when the underlying costs associated with these reimbursable amounts are recorded in research and development expenses since the Company is currently and for the foreseeable future, conducting all of the research activities.

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As of December 31, 2007, the Company recorded $1.4 million in Research Revenue and deferred $1.0 million of reimbursed research and development costs to the current portion of deferred revenue.

As of December 31, 2007, the Company also recorded $0.4 million in Collaboration Revenue related to the $50 million upfront license fee payment from Shire and deferred $2.8 million to the current portion of deferred revenue and $46.8 million to long-term deferred revenue.

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

	Quarters Ended			
	March 31	**June 30**	**September 30**	**December 31**
2006				
Net loss	$(8,287)	$ (8,624)	$(11,643)	$(17,791)
Net loss attributable to common stockholders	(8,328)	(28,089)	(11,684)	(17,828)
Basic and diluted net loss per common share (1)	(15.43)	(39.04)	(15.01)	(19.77)
2007				
Net loss	(9,695)	(9,396)	(10,303)	(11,773)
Net loss attributable to common stockholders	(9,736)	(9,706)	(10,303)	(11,773)
Basic and diluted net loss per common share (1)	(10.21)	(1.37)	(0.46)	(0.53)

(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's issuing shares of its common stock during the year.

REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

The Board of Directors and Stockholders of
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, 2007 and 2006, and the related consolidated statements of operations, stockholders' (deficiency) equity and cash flows for each of the three years in the period ended December 31, 2007 and the period February 4, 2002 (inception) to December 31, 2007. 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. and subsidiary at December 31, 2007 and 2006, and the consolidated results of their operations and their cash flows for each of the three years in the period ended December 31, 2007, and the period February 4, 2002 (inception) to December 31, 2007, in conformity with U.S. generally accepted accounting principles.

As discussed in Note 2 to the consolidated financial statements, effective January 1, 2006, the Company adopted Statement of Financial Accounting Standards No. 123(R), "Share Based Payments," applying the prospective method.

/s/ Ernst & Young LLP

MetroPark, New Jersey
February 5, 2008

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

Rule 13a-15(e) under the Securities and Exchange Act of 1934, as amended (the "Exchange Act"), defines the term "disclosure controls and procedures" as those 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 recorded, processed, summarized and reported within the time periods specified in the Securities and Exchange Commission (SEC) rules and forms and that such information is accumulated and communicated to the company's management, including its principal executive and principal financial officers, or persons performing similar functions, as appropriate to allow timely decisions regarding required disclosure. Based on their evaluation as of December 31, 2007, our chief executive officer and chief financial officer have concluded that our disclosure controls and procedures (as defined by Rules 13a-15(e) and 15d-15(e) under the Exchange Act) were effective as of December 31, 2007.

There have been no changes in our internal controls over financial reporting during the fourth quarter of the year ended December 31, 2007 that have materially affected, or are reasonably likely to materially affect, our internal controls over financial reporting.

Exemption from Management's Report on Internal Control Over Financial Reporting for 2007

This annual report does not include a report of management's assessment regarding internal control over financial reporting or an attestation report of our registered public accounting firm due to a transition period established by rules of the SEC for newly public companies.

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 2008 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 1, 2008.

John F. Crowley has served as 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 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 Pharmaceuticals 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 at Genzyme Corporation in Regulatory Affairs and Manufacturing. Mr. Patterson received a B.A. in Biochemistry from Bowdoin College.

James E. Dentzer has served as Chief Financial Officer since October 2006. From November 2003 to October 2006, Mr. Dentzer was Corporate Controller at Biogen Idec Inc. From 2001 until the 2003 merger of Biogen, Inc. and IDEC Pharmaceuticals Corporation, Mr. Dentzer served as Corporate Controller of Biogen, Inc. Prior to that, he served in a variety of financial positions at E. I. du Pont de Nemours and Company, most recently as Chief Financial Officer of DuPont Flooring Systems. Mr. Dentzer received his B.A. from Boston College and his M.B.A. from the University of Chicago.

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.

David Palling, Ph.D., has served as Senior Vice President, Drug Development, since August, 2002. From September 1998 until August, 2002, Dr. Palling was with Johnson & Johnson, most recently serving as Vice President of Worldwide Assay Research and Development at Ortho Clinical Diagnostics, a subsidiary of Johnson & Johnson. Dr. Palling received B.Sc. and Ph.D. degrees in Chemistry from the University of London, King's College, and conducted post-doctoral research in Biochemistry at Brandeis University.

Gregory P. Licholai, M.D., has served as Vice President, Medical Affairs since December 2004. From November 2002 to December 2004, Dr. Licholai was with Domain Associates, a venture capital firm. From September 2000 to November 2002, he was director of Ventures and Business Associates for Medtronic Neurological, a division of Medtronic, Inc. Dr. Licholai received his B.A. from Boston College and completed Pre-Medical studies at Columbia University, his M.D. from Yale Medical School and his M.B.A. from Harvard Business School.

S. Nicole Schaeffer has served 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 Vice President, Business Planning since May 2007. 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.

Andrew Shenker M.D., Ph.D. has served as Vice President, Clinical Research since December 2007. From 2002 to 2007, Dr. Shenker was with Bristol-Myers Squibb where he served most recently as Medical Director in the Clinical Discovery Group. From 1995 to 2002, Dr. Shenker was Assistant Professor of Pediatrics, Molecular Pharmacology and Biological Chemistry at Northwestern University Medical School. Dr. Shenker obtained his Ph.D. in pharmacology and M.D. from the Mount Sinai School of Medicine in NYC, completed his internship and residency in Pediatrics at the Johns Hopkins Hospital and was a post-doctoral fellow at the National Institutes of Health.

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 March 30, 2008 (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 incorporates 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 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	Warrant to purchase shares of common stock, dated August 28, 2002	S-1 (333-141700)	3/30/07	4.6	
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	2007 Equity Incentive Plan and forms of option agreements	S-1/A (333-141700)	5/17/07	10.2	
+ 10.3	License Agreement, dated as of April 15, 2002, by and between the Registrant and Mount Sinai School of Medicine of New York University, as amended	S-1 (333-141700)	3/30/07	10.3	
+10.4	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	
+10.5	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	
10.6	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	
10.7	Amended and Restated Employment Agreement, dated as of April 28, 2006, by and between the Registrant and John F. Crowley	S-1 (333-141700)	3/30/07	10.7	
10.8	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	
10.9	Letter Agreement, dated as of July 27, 2006, by and between the Registrant and James E. Dentzer	S-1 (333-141700)	3/30/07	10.9	

Exhibit No.	Filed Exhibit Description	Incorporated by Reference to SEC Filing			Filed with this Form 10-K
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10.10	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	
10.11	Consulting Agreement, dated as of February 28, 2006, by and between the Registrant and Donald J. Hayden, Jr.	S-1 (333-141700)	3/30/07	10.15	
10.12	Form of Director and Officer Indemnification Agreement	S-1 (333-141700)	3/30/07	10.17	
10.13	Employment Agreement, dated as of September 11, 2006, by and between the Registrant and Donald J. Hayden, Jr.	S-1/A (333-141700)	4/27/07	10.19	
10.14	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	
10.15	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	
10.16	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	
10.17	2007 Director Option Plan and form of option agreement	S-1/A (333-141700)	5/17/07	10.23	
10.18	2007 Employee Stock Purchase Plan	S-1/A (333-141700)	5/17/07	10.24	
10.19	Severance and Change in Control Agreement, dated as of May 10, 2007, by and between the Registrant and Bradley L. Campbell	S-1/A (333-141700)	5/17/07	10.25	
++10.20	License and Collaboration Agreement, dated as of November 7, 2007, by and between Registrant and Shire Pharmaceuticals Ireland, Ltd.				
10.21	Severance and Change in Control Agreement, dated as of November 9, 2007, by and between the Registrant and David Lockhart, Ph.D.	Form 8-K Current Report	11/13/07	10.1	
10.22	Severance and Change in Control Agreement, dated as of November 12, 2007, by and between the Registrant and James E. Dentzer	Form 8-K Current Report	11/13/07	10.2	
10.23	Severance and Change in Control Agreement, dated as of November 12, 2007, by and between the Registrant and Matthew R. Patterson	Form 8-K Current Report	11/13/07	10.3	
10.24	Severance and Change in Control Agreement, dated as of November 9, 2007, by and between the Registrant and David Palling, Ph.D.	Form 8-K Current Report	11/13/07	10.4	
10.25	Severance and Change in Control Agreement, dated as of November 12, 2007, by and between the Registrant and Bradley L. Campbell	Form 8-K Current Report	11/13/07	10.6	

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10.27	Severance and Change in Control Agreement, dated as of November 12, 2007, by and between the Registrant and Mark Simon	Form 8-K Current Report	11/13/07	10.9	
10.28	Severance and Change in Control Agreement, dated as of November 9, 2007, by and between the Registrant and S. Nicole Schaeffer	Form 8-K Current Report	11/13/07	10.9	
21.1	Subsidiaries of the Registrant	S-1 (333-141700)	3/30/07	21.1	
23.1	Consent of Independent Registered Public Accounting Firm.				
31.1	Certification of Principal Executive Officer Pursuant to Rule 13a-14(a) of the Securities Exchange Act of 1934.				
31.2	Certification of Principal Financial Officer Pursuant to Rule 13a-14(a) of the Securities 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.				
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.				

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

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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 February 8, 2008.

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.

Signature	Title	Date
/s/ John F. Crowley (John F. Crowley)	Chief Executive Officer (Principal Executive Officer)	February 6, 2008
/s/ James E. Dentzer (James E. Dentzer)	Chief Financial Officer (Principal Financial and Accounting Officer)	February 6, 2008
/s/ Donald J. Hayden (Donald J. Hayden)	Chairman of the Board	February 6, 2008
/s/ Alexander E. Barkas, Ph.D. (Alexander E. Barkas, Ph.D.)	Director	February 6, 2008
/s/ Stephen Bloch, M.D. (Stephen Bloch, M.D.)	Director	February 6, 2008
/s/ P. Sherrill Neff (P. Sherrill Neff)	Director	February 6, 2008
/s/ Michael G. Raab (Michael G. Raab)	Director	February 6, 2008
/s/ Glenn Sblendorio (Glenn Sblendorio)	Director	February 6, 2008
/s/ James N. Topper, M.D., Ph.D. (James N. Topper, M.D., Ph.D.)	Director	February 6, 2008
/s/ Gregory M. Weinhoff, M.D. (Gregory M. Weinhoff, M.D.)	Director	February 6, 2008

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

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EXHIBIT 10.20

LICENSE AND COLLABORATION AGREEMENT

dated as of November 7, 2007

by and between

Amicus Therapeutics, Inc.

and

Shire Pharmaceuticals Ireland Ltd.

[***] INDICATES MATERIAL THAT HAS BEEN OMITTED AND FOR WHICH CONFIDENTIAL TREATMENT HAS BEEN REQUESTED. ALL SUCH OMITTED MATERIAL HAS BEEN FILED WITH THE SECURITIES AND EXCHANGE COMMISSION PURSUANT TO RULE 24b-2 PROMULGATED UNDER THE SECURITIES EXCHANGE ACT OF 1934, AS AMENDED.

TABLE OF CONTENTS

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EXECUTION COPY

LICENSE AND COLLABORATION AGREEMENT

This License and Collaboration Agreement (this "***Agreement***") is made as of November 7, 2007 (the "***Effective Date***"), by and between Amicus Therapeutics, Inc., a Delaware corporation ("***Amicus***"), and Shire Pharmaceuticals Ireland Ltd., a corporation organized under the laws of Ireland ("***Shire***" and each of Amicus and Shire, a "***Party***").

BACKGROUND

A. Amicus has developed a platform for the treatment of human genetic diseases comprising the use of small molecule drugs, referred to as pharmacological chaperones, which selectively bind to an active site of a target protein, thereby enhancing the protein's stability and ability to fold into the correct three-dimensional shape, to restore proper biological activity of the target protein. Amicus currently is conducting human clinical trials on three products containing such pharmacological chaperone compounds, which Amicus refers to as Plicera™, Amigal™ and AT2220.

B. Shire is an established pharmaceutical company which focuses its experience and expertise in the development and commercialization of pharmaceutical products in select areas, including among them, human genetic disorders.

C. Shire desires to acquire rights to the Licensed Products for commercialization outside the United States, and to collaborate with Amicus in the further Development of such Licensed Products, all on the terms and conditions set forth below in this Agreement.

Now, therefore, in consideration of the foregoing premises and the mutual covenants herein contained, and for other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, the Parties hereby agree as follows:

ARTICLE 1
DEFINITIONS

In addition to terms defined elsewhere in this Agreement, the following terms shall have the respective meanings set out below, and grammatical variations of such terms shall have corresponding meanings.

1.1 "***Affiliate***" means, with respect to a Party, any person, corporation or other entity which, directly or indirectly through one or more intermediaries, controls, is controlled by or is under common control with such Party, as the case may be. As used in this Section 1.1, "control" shall mean: (a) direct or indirect beneficial ownership of at least 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 voting stock or other ownership interest in such person, corporation or other entity; or (b) to possess, directly or indirectly, the power to affirmatively direct the management and

policies of such person, corporation or other entity, whether through ownership of voting stock or other ownership interest or by contract relating to voting rights or corporate governance.

1.2 "***Amicus IP***" shall mean the Amicus Know-How and Amicus Patent Rights, defined as follows:

1.2.1 "***Amicus Know-How***" means Know-How Controlled by Amicus as of the Effective Date or during the Term, and that is necessary, useful or actually used by Amicus to Develop, Manufacture or Commercialize Licensed Products in the Field.

1.2.2 "***Amicus Patent Rights***" means those Patent Rights listed on Appendix 1 and any and all other Patent Rights Controlled by Amicus during the Term that are necessary, useful or actually practiced by Amicus, to Develop, Manufacture or Commercialize a Licensed Product in the Field. Appendix 1 shall be updated from time to time as requested by either Party to reflect all additional Patent Rights within the Amicus Patent Rights.

1.3 "***Commercialization***" or "***Commercialize***" means activities directed to marketing, advertising, promoting, detailing, distributing, importing or selling a product, including Post-Marketing Studies, Manufacture of commercial supplies and education, planning, product support and medical efforts related to a product. For clarity, Manufacturing process development, scale-up and validation of Manufacturing with respect to a Licensed Product prior to the first Regulatory Approval in a Primary Market for such Licensed Product (or in connection with establishing second source manufacturers or manufacturing sites) shall not be deemed Commercialization and shall instead be considered Development (unless and to the extent, in the case of validation batches, such batches are used as commercial supplies), while further process development, scale-up and/or validation of Manufacturing after the first Regulatory Approval in a Primary Market for such Licensed Product shall be included within Commercialization.

1.4 "***Commercially Reasonable Efforts***" means, with respect to a Party, the efforts and resources which would be used by that Party relating to a certain activity or activities, consistent with its normal business practices for a product at a similar stage in its development and of similar market potential in a field of the biopharmaceutical industry of similar size as the Field that such Party is seeking to Develop and Commercialize in a reasonably expeditious manner.

1.5 "***Compound***" means the following chemical entities:

1.5.1 deoxygalactonojirimycin having the structure shown in Exhibit 1.5.1, and any **[***]** thereof ("***Deoxygalactonojirimycin***" or "***DGJ***");

1.5.2 deoxynojirimycin having the structure shown in Exhibit 1.5.2, and any **[***]** thereof ("***Deoxynojirimycin***" or "***DNJ***");

1.5.3 isofagomine having the structure shown in Exhibit 1.5.3, and any enantiomers, **[***]** thereof ("***Isofagomine***" or "***[***]***"); and

1.5.4 any other chemical entity that the Parties agree to add as a Compound under Section 6.3 or 6.4.4 below. If the Parties agree to add an additional Compound as described in this Section 1.5.4, the Parties shall attach to this Agreement an Exhibit 1.5.x, describing the chemical structure of such compound, it being understood that only the chemical entity so described, plus enantiomers, metabolites, salts and polymorphs thereof, shall be deemed so added as a Compound.

As used in this Agreement, any reference to a Compound shall be deemed to include enantiomers, metabolites, salts and polymorphs thereof.

1.6 "***Controlled***" means, with respect to any intellectual property right or other intangible property, the possession by license or ownership by a Party (or by an Affiliate (a) of such Party as of the Effective Date, (b) controlled, as defined in Section 1.1 above, by such Party or such an Affiliate, or (c) that first becomes an Affiliate after the Effective Date and is involved in the Development of the Compounds) of the ability to grant to the other Party access or a license or sublicense as provided herein without violating the terms of any written contract with any Third Party.

1.7 "***Data***" means any and all (a) research data, pharmacology data, chemistry, manufacturing and control data, preclinical data, clinical data and other similar technical and scientific data necessary, useful or actually used in the Development or Manufacture of Licensed Products within the Field or otherwise generated under the Development Plans and (b) all documentation and correspondence submitted, or required to be submitted, to a Regulatory Authority, or received from a Regulatory Authority, in connection with a Regulatory Approval for a Licensed Product within the Field in any country, including, without limitation, information in any drug master files or similar documentation.

1.8 "***Development***" means all activities related to (a) researching or developing a Licensed Product, or obtaining Regulatory Approvals for such products or indications (including Label Expansions and New Formulations within the Field pursuant to Section 6.1) in the Territory, including preclinical testing, toxicology, formulation, clinical trials, and regulatory affairs, as well as (b) Phase IV Clinical Trials and preclinical studies conducted after Regulatory Approval (such as carcinogenicity studies, preclinical studies to establish pediatric dosing and the like) that are required or requested by a Regulatory Authority to be conducted after Regulatory Approval, as a condition of or in connection with obtaining such Regulatory Approval. Development shall also include Manufacturing activities for the purposes of producing clinical supplies (or materials used in preclinical testing or research), as well as Manufacturing scale up, process development and validation for such a product prior the first Regulatory Approval of such a product in the first Primary Market (including manufacturing batches for validation and registration purposes, to the extent such batches are not used as commercial supplies) and the establishment of second source manufacturers or manufacturing sites. Development shall not include Manufacture of commercial supplies or Commercialization. As used herein "Develop" shall also include such activities with respect to a Compound, Related Product or Back-Up Compound.

1.9 "***Development Costs***" means, except as otherwise expressly provided in this Agreement, the internal and external costs incurred by a Party or a Subsidiary in performing

Development activities in accordance with the applicable Development Plan, including, (a) costs of clinical trials for Licensed Products in the Field and related clinical trial materials, (b) costs of non-clinical studies and related study materials, (c) costs associated with preparing and submitting Regulatory Filings to obtain, maintain and/or expand Regulatory Approval of Licensed Products in the Field, (d) costs associated with establishing and validating Manufacturing facilities (including process development and optimization of Manufacturing processes) to Manufacture Licensed Products in the Field and (e) such other amounts as reflected in such Development Plan. For such purposes, costs for a Party's personnel performing the Development Plans shall, unless otherwise determined by the JSC and reflected in the applicable Development Plan, be calculated on the basis of the FTE Rate. Any dispute regarding Development Costs shall be referred to the JDC for resolution in accordance with the terms and conditions of this Agreement.

1.10 "***EMEA***" means the European Medicines Agency or any successor agency with responsibility for regulating the development, manufacture and sale of human pharmaceutical products in the European Union.

1.11 "***[***]***" means a meeting held with the responsible Party and the Regulatory Authority of a Primary Market Country to review the data and results of the Phase II Clinical Trials of a Licensed Product and to discuss with the Regulatory Authority such Party's plan to commence a Phase III Clinical Trial of such Licensed Product and plans to complete additional work (e.g., preclinical testing and manufacturing) in support of a future license application.

1.12 "***Ex-U.S. Platform Patent Rights***" means those Amicus Patent Rights listed on Appendix 2.

1.13 "***European Union***" means Austria, Belgium, Bulgaria, the Czech Republic, Cyprus, Denmark, Estonia, Finland, France, Germany, Greece, Hungary, Ireland, Italy, Latvia, Lithuania, Luxembourg, Malta, the Netherlands, Poland, Portugal, Romania, Slovakia, Slovenia, Spain, Sweden and the United Kingdom.

1.14 "***FDA***" means the United States Food and Drug Administration and any successor thereto.

1.15 "***Field***" means the diagnosis, treatment and/or prevention of (a) Gaucher Disease, Fabry Disease or Pompe Disease, (b) to the extent the Parties mutually agree, in accordance with Section 6.1.5 below, to include an additional indication beyond those described in (a) above, such additional indication and (c) if Shire duly exercises the **[***]**'s Option, then with respect to [***] for [***]'s (but only [***] for [***]'s), [***]'s.

1.16 "***First Commercial Sale***" means the first bona fide commercial sale of a Licensed Product for use in the Field within a country in the Territory following issuance of all applicable Regulatory Approvals required prior to commercial sale in such country.

1.17 "***FTE Rate***" means initially $**[***]** per FTE (i.e., a full-time equivalent person) per year, subject to adjustment as follows: Commencing as of January 1, 2009, the FTE Rate shall increase on January 1 of each year by the percentage increase in the Consumer Price Index, for All Urban Consumers, as published by the U.S. Department of Labor, Bureau of Labor Statistics, since the last such increase under this definition (or in the case of the first such increase, the Effective Date) and such increase shall be effective for the then-current and all subsequent Development Plans hereunder until further modified under this definition. Any dispute regarding adjustment of the FTE Rate shall be referred to the JDC for resolution in accordance with the terms and conditions of this Agreement.

1.18 "***[***] for [***]'s***" means a pharmaceutical product containing **[***]**, for the treatment or prevention of [***]'s. It is understood that references herein to an [***] for [***]'s shall be deemed limited to the use of such product only for the treatment and/or prevention of [***]'s, and shall not include any other use of such product.

1.19 "***IND***" means an Investigational New Drug Application filed with the FDA or the equivalent application or filing necessary to commence clinical trials in a foreign jurisdiction, as applicable.

1.20 "***Know-How***" means all information, results and Data of any type, in any tangible or intangible form pertaining to the Development, Manufacturing or Commercialization of Licensed Products within the Field, including without limitation databases, ideas, discoveries, inventions, trade secrets, practices, methods, tests, assays, techniques, specifications, processes, formulations, formulae, knowledge, know-how, skill, experience, materials, including pharmaceutical, chemical and biological materials, products and compositions, scientific, technical or test data (including pharmacological, biological, chemical, biochemical, toxicological and clinical test data), analytical and quality control data, stability data, studies, procedures, drawings, plans, designs, diagrams, sketches, technology, documentation or descriptions. Notwithstanding the foregoing, as used in this Agreement, "Know-How" (a) does not include Patent Rights in the foregoing and (b) does not include methods, assays, materials, techniques, or other items used or useful to perform drug discovery or research in the Field, to the extent such items are not reasonably necessary, useful or used to perform clinical trials or Manufacturing of Licensed Products, preclinical testing in support of such clinical trials and/or Manufacturing, or Commercialization of a Licensed Product within the Field.

1.21 "***Licensed Product***" means (a) Amigal, (b) AT2220, (c) Plicera and (d) any other pharmaceutical formulation of a Compound developed under a Development Plan, or by Shire as an Independent Project in accordance with Section 6.1 below or as part of a Combination Product in accordance with Section 4.5, containing a Compound. For such purposes, and as otherwise used herein:

1.21.1 "***Amigal***" means that certain pharmaceutical product containing the active chemical entity Deoxygalactonojirimycin, the formulation of which is described in IND number 68,456;

1.21.2 "***AT2220***" means that certain pharmaceutical product containing the active chemical entity Deoxynojirimycin, the formulation of which is described in IND number 76,268; and

1.21.3 "***Plicera***" means that certain pharmaceutical product containing the active chemical entity Isofagomine the formulation of which is described in IND number 73,475.

1.22 "***MAA***" means any marketing authorization application for a country or region, requesting approval from the applicable Regulatory Authority for commercial sale of a Licensed Product in the Field in such country or region, and all amendments and supplements filed to any such application.

1.23 "***Manufacture***" means manufacturing and related activities, including chemical synthesis, formulation, processing, testing, packaging, labeling, storing, warehousing, quality control, quality assurance, releasing, disposing, handling, shipping and all other activities undertaken or required to be undertaken in order to manufacture and supply a Compound or Licensed Product.

1.24 "***NDA***" means a New Drug Application for any product, as appropriate, requesting permission to place a drug on the market in accordance with 21 C.F.R. Part 314, and all supplements or amendments filed pursuant to the requirements of the FDA, including all documents, data and other information concerning a product which are reasonably necessary for FDA approval to market a product in the United States.

1.25 "***Net Sales***" means the gross amounts invoiced for sales of Licensed Products in the Shire Territory by Shire, its Affiliates and/or its Sublicensees to Third Parties, less deductions for the following costs actually allowed or incurred:

1.25.1 freight, postage and transportation charges on shipment of such Licensed Product to the customer, including handling and insurance on such shipment;

1.25.2 sales (such as VAT or its equivalent) and excise taxes, other consumption taxes, customs duties and other governmental charges imposed upon the sale of such Licensed Product to the customer;

1.25.3 charge-back payments, rebates, and similar product-specific payments paid to a governmental entity specifically with respect to sales of Licensed Products under a governmental rebate program;

1.25.4 trade, quantity and cash discounts actually granted to the customer with respect to the Licensed Product;

1.25.5 credits, rebates and charge-backs, and allowances or credits to the customer on account of damaged products, rejection or returns of Licensed Products or on account of retroactive price reductions affecting such Licensed Product;

1.25.6 actual bad debt expense not to exceed **[***]** of gross amounts invoiced; and

1.25.7 any item similar in character or substance to any of the foregoing prevailing at the time and customary in the pharmaceutical industry at the time as determined by the JSC.

Notwithstanding the foregoing, the amounts described in **[***]**. Sales among a Party and its Affiliates or permitted Sublicensees for resale shall be excluded from the computation of Net Sales; provided, however, that the subsequent resale shall be included in Net Sales hereunder. If a Licensed Product is sold for consideration other than cash, the Net Sales from such sale or transfer shall be deemed the then fair market value of such Licensed Product. For clarity, Net Sales shall include sales of a Licensed Product made pursuant to a pre-license sale through a named patient basis sales program or other special access sales program. The supply of Licensed Products without charge (x) as commercial samples, (y) as charitable donations or (z) for use in Development and Post-Marketing Studies shall be excluded from the computation of Net Sales.

In the event that a Licensed Product is sold as part of a Combination Product in accordance with Section 4.5, Net Sales from sales of such Combination Product shall be determined pursuant to Section 4.5.

1.26 "***[***]***.

1.27 "***Patent Rights***" means (a) all patents and patent applications (including provisional applications), and all patents issuing thereon (including utility, model and design patents and certificates of invention), (b) all reissue patents, patents of addition, divisions, renewals, continuations, continuations-in-part, substitutions, extensions (including supplemental protection certificates), registrations, confirmations, re-examinations and (c) foreign counterparts of any of the foregoing.

1.28 "***Phase II Clinical Trial***" means a human clinical trial of a Licensed Product conducted for purposes of preliminary determination of efficacy and/or preliminary establishment of appropriate dosage ranges for efficacy and safety in patients with the disease or condition being studied and that would satisfy the requirements under 21 C.F.R. §312.21(b).

1.29 "***Phase III Clinical Trial***" means a human clinical trial of a Licensed Product intended to be a pivotal trial for obtaining Regulatory Approval or to otherwise establish safety and efficacy in patients with the disease or condition being studied for purposes of filing an NDA with the FDA or an MAA with the EMEA and that would satisfy the requirements under 21 C.F.R. §312.21(c).

1.30 "***Phase IV Clinical Trial***" means a human clinical trial for a Licensed Product conducted after receipt of Regulatory Approval in the country for which such trial is being conducted and that is required or requested by a Regulatory Authority to be conducted after Regulatory Approval, as a condition of or in connection with obtaining and maintaining such Regulatory Approval.

1.31 "***Post-Marketing Studies***" means marketing studies, epidemiological studies, modeling and pharmacoeconomic studies, investigator sponsored clinical trials and post-marketing surveillance studies of a Licensed Product, other than Phase IV Clinical Trials, that are not intended for use as a basis for obtaining Regulatory Approval (e.g., for a further indication, label expansion or otherwise) with respect to such Licensed Product.

1.32 "***Primary Market***" means any one or more of the following: United States, France, Germany, Italy, Spain and the United Kingdom.

1.33 "***Product Marks***" means the product-specific trademarks, logos, trade dress, or other symbols which a Party uses to Commercialize a Licensed Product in its Territory, but excluding the Amicus and Shire company names, tradenames, logos, trade dress and the like.

1.34 "***Regulatory Authority***" means any federal, national, multinational, provincial, state or local regulatory agency, department, bureau or other governmental entity, within a regulatory jurisdiction in the Territory, with the authority to grant any approvals, licenses, registrations or authorizations necessary for the Development, Manufacture, use, Commercialization or coverage and reimbursement of a Licensed Product. For clarity, references in this Agreement to "Regulatory Authority of a Primary Market Country" shall be deemed to include the EMEA.

1.35 "***Regulatory Approval***" means, with respect to a particular country, all approvals (including, without limitation, where applicable, pricing and reimbursement approval and schedule classifications), licenses, registrations or authorizations by any Regulatory Authority necessary for the Development, Manufacture, use, storage, import, transport, Commercialization or sale of a Licensed Product in such country.

1.36 "***Regulatory Filings***" means all documents filed with a Regulatory Authority, including INDs, NDAs, MAAs, Drug Master Files and the like, as well as their counterparts in jurisdictions other than the United States.

1.37 "***Related Agreement***" means a Pharmacovigilance Agreement or other agreements entered into by the Parties pursuant to or in connection with this Agreement.

1.38 "***Sole Invention***" means either a Shire Invention or an Amicus Invention.

1.39 "***Sublicensee***" shall mean a Third Party to whom Shire (or a Sublicensee) has granted a right to make, use, sell, offer for sale, import or Commercialize a Licensed Product in the Shire Territory pursuant to Section 2.2; and "***Sublicense***" shall mean an agreement or arrangement granting such rights. As used in this Agreement, "Sublicensee" shall not include a wholesaler or reseller of a Licensed Product who does not market or promote such Licensed Product.

1.40 "***Territory***" means both the Amicus Territory and the Shire Territory, each as defined below:

1.40.1 "***Amicus Territory***" means the United States of America, including the District of Columbia and including all possessions, territories and protectorates thereof and shall include the European Union with respect to a Licensed Product upon the reversion of the European Union with respect to such Licensed Product in accordance with Section 7.2.5.

1.40.2 "***Shire Territory***" means the entire world excluding the Amicus Territory.

As used herein, the phrase "a Party's Territory" or "such Party's Territory" shall mean either the Amicus Territory or the Shire Territory, as the context indicates.

1.41 "***Third Party***" means any person or entity, including a governmental entity, other than Amicus, Shire or their respective Affiliates.

1.42 "***Valid Claim***" means a claim of a pending patent application or an issued and unexpired patent, within the Amicus Patent Rights that has not been held unpatentable, invalid or unenforceable by a court or other government agency of competent jurisdiction in an unappealed or unappealable decision (provided, however, that if the holding of such court or agency is later reversed by a court or agency with appropriate authority, the claim shall be reinstated as a Valid Claim) and has not been admitted to be invalid or unenforceable through reissue, re-examination, disclaimer or otherwise nor lost in an interference proceeding. Notwithstanding the foregoing, in the case of a pending but unissued patent application, a pending claim of such application shall not be deemed a Valid Claim if more than three (3) years have elapsed since the first priority date to which such claim takes priority; such claim shall thereafter not be deemed a Valid Claim until such claim issues in a patent and otherwise meets this definition.

1.43 The following terms have the meanings defined in the corresponding sections of this Agreement referenced below:

Defined Term	Section	Defined Term	Section
[***]	7.2.4(e)	Manufacturing Cost	7.3.3(b)(ii)
Acting Party	10.2.5	Materials	8.1
Alleged Infringement	10.3.1	Milestone	7.2.3
Alliance Manager	3.6	MSSM	16.14
Annual Net Sales	7.3.1	MSSM Agreement	16.14
Amicus Indemnitees	13.2	New Formulation	6.1.1
Amicus Invention	10.1.2(a)	Non-Developing Party	6.1.2(b)
Auditing Party	7.8.2	Notice Date	15.5.2(a)(ii)
Back-Up Compound	6.4.4(a)	Notice Period	15.5.2(a)(ii)
Back-Up Compound Notice	6.4.4(b)	Offer	6.2.2(a)
Back-Up Compound Opt In Exercise Period	6.4.4(c)	Opt-In Notice	6.1.3(a)
Back-Up Compound Opt In	6.4.4(a)	Opt-In Period	6.1.3(a)

Right			
Breach Notice	15.2	Opt-In Right	6.1.3
Business Day	16.9	Shire Indemnitees	13.1
[***]	7.2.4	Shire Invention	10.1.2(a)
Collaboration Results Publication	14.5	Shire IP	6.1.1(d)(ii)
Combination Product	4.5	[***]'s Option	6.2.1
Commercializing Party	10.6.2	[***]'s Option Exercise Fee	6.2.1(b)
Committee	3.4.1	[***]'s Option Notice	6.2.1(a)
Committee Co-Chair	3.5.4	[***]'s Option Period	6.2.1(a)
Committee Dispute	16.8.1	Permitted Overrun	7.4.2
[***]	7.2.4(d)	Pharmacovigilance Agreement	9.3
Confidential Information	14.1	Prosecuting Party	10.2.4
Confidentiality Agreement	14.3	prosecution and maintenance	10.2.6
Cooperating Party	14.6.2	Receiving Party	7.4.4(b)
Developing Party	6.1.1	Reimbursable Share	6.1.3(c)
Development Period	15.5.2(a)(ii)	Reimbursing Party	7.4.4(b)
Development Plan	4.2.1	Related Product	6.3.1
Excess Costs	7.4.2	Related Product Notice	6.3.1
Existing In-Licenses	10.6.1	Related Product-Opt In Period	6.3.2
Force Majeure Event	16.5	Related Product-Opt In Right	6.3.2
Forecast	4.2.1	Requesting Party	6.4.1
Generic Competition	7.3.2	Responding Party	7.8.2
Generic Version	7.3.2	Reverted Products	15.5.2(a)(i)
Gross Margin	7.3.3(b)(i)	RFR Acceptance	6.2.2(a)
[***]	2.2.2(b)	RFR Acceptance Period	6.2.2(a)
Indemnitee	13.3	Right of First Refusal	6.2.2
Indemnitor	13.3	Right of First Refusal Notice	6.2.2(a)
Independent Development Costs	6.1.2(c)(ii)	[***]	7.2.4(e)
Independent Project	6.1.2(a)	[***]	2.4.1
Independent Trial	4.3.2	Secondary Country	7.2.5
Initiating Party	14.6.2	Special Committee	3.4.1
[***]	7.2.4(c)	Spending Party	7.4.2
Inspected Party	9.5	Statement of Costs	6.1.2(c)(i)
JAMS	16.8.1(b)	Sublicensing Party	10.6.2
JCC	3.3	[***]	7.2.4(a)

[***] INDICATES MATERIAL THAT HAS BEEN OMITTED AND FOR WHICH CONFIDENTIAL TREATMENT HAS BEEN REQUESTED. ALL SUCH OMITTED MATERIAL HAS BEEN FILED WITH THE SECURITIES AND EXCHANGE COMMISSION PURSUANT TO RULE 24b-2 PROMULGATED UNDER THE SECURITIES EXCHANGE ACT OF 1934, AS AMENDED.

JDC	3.2	[***]	7.2.4(b)
Joint Commercialization Committee	3.3	Supply Agreement	8.2
Joint Development Committee	3.2	Target	6.3.1
Joint Inventions	10.1.2(a)	Term	15.1
Joint Patent Rights	10.2.3	Terminated Product	6.4.4(a)
Joint Steering Committee	3.1	Third Party Claim	13.1
JSC	3.1	Third Party Technology	10.6.2
JSC Proposal Notice	6.1.1	Trademark Licensee	11.1.3
Label Expansion	6.1.1	Trademark Licensor	11.1.3
Laws	16.9	[***]	2.4.3
Liabilities	13.1	**[***]**	15.5.2(a)(ii)
Licensors	10.6.1	Wind down Period	15.5.2(b)

ARTICLE 2
GRANT OF RIGHTS

2.1 Amicus Grant. Subject to the terms and conditions of this Agreement, Amicus hereby grants to Shire, under the Amicus IP:

2.1.1 an exclusive license to use, import and sell or Commercialize Licensed Products in the Field (excluding the treatment, prevention or diagnosis of Fabry Disease with Amigal) in the Shire Territory, subject to Section 2.1.3;

2.1.2 a sole license (with a right to sublicense) to use, import and sell or Commercialize Amigal for the treatment, prevention or diagnosis of Fabry Disease in the Shire Territory, subject to Section 2.1.3;

2.1.3 a co-exclusive (with Amicus and its contractors or licensees) license to Manufacture the Compounds and Licensed Products in the Territory for use, import, sale or Commercialization within the Field in the Shire Territory (specifically subject to Section 8.3 below) and to Develop the Licensed Products within the Field (specifically subject to Section 6.1 below); provided, that if a Party (or any other entity acting under authority of such Party) proposes to perform clinical trials of a Licensed Product for an indication in the Field in a country within the other Party's Territory, the conduct of such trial in such country shall be subject to such other Party's approval, not to be unreasonably withheld; and

2.1.4 the right to have the foregoing performed on its behalf by subcontractors in accordance with Section 4.4.

Shire agrees that neither it, nor any of its Affiliates, shall (a) practice the Amicus IP other than as expressly authorized under this Article 2 if such actions would constitute an infringement or misappropriation thereof nor (b) Develop or Commercialize in the Amicus Territory a product containing a Compound, except as a Licensed Product in accordance with this Agreement.

2.2 Sublicenses.

2.2.1 Affiliates. Shire may grant to one or more of its Affiliates a Sublicense in connection with Shire's Development, Manufacture and/or Commercialization of the Compounds and Licensed Products under this Agreement; provided that Shire shall remain responsible for the activities of such Affiliate to the same extent as if such activities were conducted by Shire.

2.2.2 Third Parties.

(a) Shire may also grant to Third Parties a Sublicense under the rights granted to Shire under Section 2.1 to one or more Licensed Products, to the extent not in conflict with Section 2.1 or this Section 2.2.2.

(b) Notwithstanding Paragraph (a) above, Shire may grant a Sublicense under this Section 2.2.2 (i) only to a Third Party that is not a "competitor of Amicus" and (ii) in a country where Shire or an Affiliate of Shire has direct commercial operations in **[***]**, only if Shire remains primarily responsible for conducting Commercialization activities in such country. For such purposes, a "competitor of Amicus" shall mean those companies listed on Appendix 3 or as appended thereto upon the written agreement of the Parties.

2.2.3 Conditions of Sublicenses. If Shire grants a Sublicense under its rights in Section 2.1, such Sublicense shall be at least as protective of the Compounds and Licensed Products as the terms and conditions of this Agreement. Shire shall remain responsible for the performance of any of its Sublicensees under such rights, and shall remain responsible for any payments due hereunder with respect to activities of the Sublicensee. Shire shall use Commercially Reasonable Efforts to ensure that its Sublicensees perform at the same level as Shire is obligated to perform hereunder and do not engage in activities that would be harmful to the Licensed Products or the business related to the Licensed Products, and to take appropriate measures to remedy any failure of a Sublicensee to comply with the foregoing. It is understood and agreed that, except as may be otherwise agreed in writing by the Parties, Sublicensees shall have no rights with respect to the Committees or with respect to the Development Plans, nor to exercise any provision of this Agreement other than the exercise of their rights pursuant to Section 2.1 above. Upon request, Shire shall provide to Amicus a copy of the Sublicense, provided that the agreement may be redacted to the extent not necessary for Amicus to understand the scope and terms of such Sublicense. For purposes of clarity, Shire shall have the right to redact all financial and other proprietary terms with

respect to any Sublicense agreement provided to Amicus as required hereunder to the extent not required to determine that such Sublicense complies with this Agreement.

2.3 Exchange of Data and Know-How.

2.3.1 By Amicus. Promptly following the Effective Date, Amicus will make available to Shire, at no cost or expense to Shire, all Amicus Know-How necessary, useful or used to Develop Licensed Products within the Field, including all Data for such Licensed Products within the Field that Amicus has of the Effective Date.

2.3.2 By Either Party. During the Term, Amicus shall provide to Shire additional Amicus Know-How developed pursuant to activities under the Development Plans necessary, useful or used to Develop Licensed Products within the Field, and Shire shall provide to Amicus any Know-How Controlled by Shire and developed pursuant to activities under the Development Plans necessary, useful or used to Develop the Licensed Products within the Field, in each case that has not previously been provided hereunder, promptly upon request by the other Party. The Party providing such Know-How shall provide the same in electronic form to the extent the same exists in electronic form, and shall provide copies as reasonably requested or an opportunity for the other Party to inspect (and copy) all other materials comprising such Know-How (including, for example, original patient report forms and other original source data, to the extent allowable under Laws). Except as expressly provided, neither Party shall be obligated under this Section 2.3.2 (or other provisions requiring disclosure of Know-How hereunder) to provide to the other Party (a) any of the providing Party's Confidential Information that does not relate to a Licensed Product within the Field, including competitive and marketing strategies generally applicable to the providing Party's products or (b) any information regarding Commercialization except as necessary to Develop or Commercialize the Licensed Products.

2.3.3 Provision of Data to JDC. Upon request by the JDC, each Party shall promptly provide the JDC with summaries in reasonable detail of all Data generated or obtained in the course of such Party's performance of activities under the Development Plans.

2.3.4 Level of Effort Required. Notwithstanding the foregoing, neither Party shall be considered to be in breach of this Section 2.3 for failure to disclose information, if, despite Commercially Reasonable Efforts, the identification of such information is impractical.

2.3.5 Right to Use. Each Party shall have the right to use Know-How to be provided to such Party under this Section 2.3, in connection with the Development and Commercialization of Compounds and Licensed Products hereunder.

2.4 **[***]**. **[***]**

[***] INDICATES MATERIAL THAT HAS BEEN OMITTED AND FOR WHICH CONFIDENTIAL TREATMENT HAS BEEN REQUESTED. ALL SUCH OMITTED MATERIAL HAS BEEN FILED WITH THE SECURITIES AND EXCHANGE COMMISSION PURSUANT TO RULE 24b-2 PROMULGATED UNDER THE SECURITIES EXCHANGE ACT OF 1934, AS AMENDED.

2.5 No Implied Licenses. No right or license under any either Party's Know-How, Patent Rights or other subject matter is granted or shall be deemed granted by implication, estoppel or otherwise. All such rights or licenses are granted only as expressly provided in this Agreement and the Related Agreements. Without limiting the foregoing, nothing herein shall be deemed to grant to Shire a right or license to any active pharmaceutical ingredient other than the Compounds.

ARTICLE 3
GOVERNANCE

3.1 Joint Steering Committee. Within thirty (30) days following the Effective Date, the Parties shall establish a Joint Steering Committee (the "***Joint Steering Committee***" or "***JSC***"). The JSC shall have the duties described in Section 3.1.1 below.

3.1.1 Duties. The Joint Steering Committee shall:

(a) review and approve the Development Plans, and any material changes thereto as shall be submitted by the JDC to the JSC for approval;

(b) determine whether to terminate the joint Development of one or more Licensed Products pursuant to Section 6.4 below;

(c) determine actions necessary to prevent importation or sales of Licensed Products sold by a Party into the other Party's Territory by a Third Party (e.g. determination that neither Party may sell to such Third Party);

(d) resolve any matters submitted to the JSC by the JDC in accordance with Section 16.8 below; and

(e) perform such other duties as are specifically assigned to the JSC in this Agreement.

3.1.2 Additional Activities. In addition, at the meetings of the JSC, the Parties will discuss the following matters as reasonably requested by either Committee Co-chair of the JSC:

(a) strategic direction for the Development, Manufacturing and Commercialization of Licensed Products;

(b) the progress of the Parties in executing the Development Plans; and

(c) any other matters pertaining to Development, Manufacturing and Commercialization of Licensed Products in the Field in the Territory, and the collaboration between the Parties. However, it is understood that the decision-making authority of the JSC is limited to those matters described in Section 3.1.1 above.

3.2 Joint Development Committee. The Parties shall form a Joint Development Committee (the "***Joint Development Committee***" or "***JDC***"), no later than thirty (30) days following the Effective Date. The JDC shall have the duties described in Section 3.2.1 below.

3.2.1 Duties. The Joint Development Committee shall:

(a) propose revisions to the Development Plans as needed, but no less frequently than annually;

(b) propose supplements or revisions to the applicable Development Plans with respect to Label Expansions and New Formulations and submit the same to the JSC for approval;

(c) review and approve clinical protocols for Licensed Products within the Field under the Development Plans;

(d) review and finalize the common registration dossier for each Licensed Product generated under a Development Plan;

(e) monitor the progress of the activities undertaken by each of the Parties pursuant to each Development Plan (including review of the conduct of clinical trials conducted by each Party pursuant to a Development Plan);

(f) monitor the rate of spending pursuant to activities under a Development Plan against the budget for such activities in the Development Plan; and

(g) perform such other duties as are specifically assigned to the JDC in this Agreement.

3.2.2 Additional Activities. In addition, at the meetings of the JDC, the Parties will discuss the following matters as reasonably requested by either Committee Co-Chair of the JDC:

(a) the progress of the activities undertaken by the Parties pursuant to each Development Plan in relation to the corresponding budgets and timelines;

(b) the flow of information with respect to Development of the Licensed Products within the Field;

(c) the overall strategy for all material filings with applicable Regulatory Authorities in the Primary Markets with respect to the Licensed Products in the Field in the Shire Territory, in accordance with the Development Plans, as well as regulatory strategy for Licensed Products in the Field in Japan;

(d) the Parties' scientific presentation and publication strategy relating to Licensed Products within the Field pursuant to Section 14.5 below, until such time as the JCC is

formed pursuant to Section 3.3 below, at which time such matters shall be deemed to be within the duties of the JCC under Section 3.3.1 below;

(e) impact of operational activities related to Manufacturing, (for example, forecast development, growth, changes, variances, manufacturing process improvements, equipment/new facility introduction, capacity improvements, cycle time and lead time reduction, improvement in shelf life, inventory management, complaints, and in-market quality/performance reports); and

(f) any other matters pertaining to Development of Licensed Products in the Field. However, it is understood that the decision-making authority of the JDC is limited to those matters described in Section 3.2.1 above.

3.3 Joint Commercialization Committee. The Parties shall form a Joint Commercialization Committee (the "***Joint Commercialization Committee***" or "***JCC***"), no later than thirty days following the Initiation of the First Phase III Clinical Trial for a Licensed Product.

3.3.1 Duties. The Joint Commercialization Committee shall serve as a forum for communication regarding Commercialization activities and shall discuss and review the following:

(a) any Post-Marketing Studies proposed to be conducted by either Party;

(b) coordination of global branding to the extent practicable;

(c) promotional and other Commercialization activities of the Parties under this Agreement in the Amicus Territory and the Shire Territory, including pre-launch and post-launch activities;

(d) proposed Product Marks and branding strategy;

(e) coordination of the participation of physicians who are key opinion leaders during Development and Commercialization to achieve consistent messaging and collaboration in connection with conferences and other marketing activities, provided, however, that each Party shall have control over Commercialization of Licensed Products in the Field in its respective Territory; and

(f) such other matters as appropriate to further the purposes of this Agreement as determined by the Joint Steering Committee.

For clarity, it is understood that the purpose of the JCC is to promote communication and coordination regarding the foregoing matters and that the JCC shall not have decision making authority.

3.4 Special Committees and Sub-Committees; Financial Procedures.

3.4.1 Special Committees and Sub-Committees. The JSC may from time to time establish one or more special committees (each, a "***Special Committee***"), each such Special Committee to consist of an equal number of representatives of each Party as determined by the JSC, to perform certain duties and exercise certain powers of the JSC as expressly delegated by the JSC to such Special Committee. For example, it is understood that, from time to time, the JSC may establish one or more Special Committees to coordinate intellectual property matters in accordance with Article 10 below (it being understood such Special Committee shall be for communication purposes and shall not have decision making authority). Each of the JSC, JDC, JCC and any such Special Committee is referred to herein as a "***Committee***." Each Committee may from time to time establish sub-committees to handle matters within the scope of its authority hereunder.

3.4.2 Certain Financial Procedures. In addition, the JSC may establish a Special Committee to approve procedures, formats and timelines consistent with this Agreement for reporting financial data and monitoring financial performance under this Agreement; and if the Parties, or such Special Committee, as applicable, are unable to agree upon any such procedures, formats or timelines, the matter shall be resolved as a Committee Dispute in accordance with the provisions of Section 16.8 below.

3.5 Committee Membership, Decision-Making and Operations.

3.5.1 Membership of Committees. Each Committee shall be composed of an equal number of representatives from each of Amicus and Shire. Unless the Parties otherwise agree, the number of representatives for each of Amicus and Shire shall be: (a) with respect to the JSC, three (3) representatives, (b) with respect to the JDC, three (3) representatives and (c) with respect to the JCC, three (3) representatives, and each of the above with ad hoc members as deemed necessary by the relevant committee. At least one representative of each Party on the JDC and JCC will be at the vice president level or above. All representatives of each Party on the JSC will be at the vice president level or above, subject to the next sentence, and at least one representative of each Party on the JSC will be at the senior vice president (or its equivalent) level or above. In addition, each Party's Alliance Manager will serve on the JSC, JDC and JCC in a nonvoting capacity. Each Party may replace any of its representatives on a Committee at any time upon written notice to the other Party, provided that such replacement is of comparable standing and authority within that Party's organization as the person he or she is replacing.

3.5.2 Committee Meetings. Each Committee shall hold regularly scheduled meetings at such times as it elects to do so, provided, however, that (a) the JSC shall meet at least twice every calendar year, (b) the JDC shall meet at least once every calendar quarter, unless the respective Committee members otherwise agree and (c) the JCC shall meet at least twice every calendar year. Each Party may also call for special meetings to resolve particular matters requested by such Party. The applicable Committee Co-Chair shall provide Committee members no less than fifteen (15) Business Days' notice of each regularly scheduled meeting, and no less than ten (10) Business Days' notice, or such shorter time period as a Committee Co-Chair deems appropriate under the circumstances, but in no event less than two (2) Business Days' notice, of any special

meetings called by either Party. Meetings may be held by audio or video teleconference with the consent of each Party, which shall not be unreasonably withheld, provided that unless otherwise agreed at least two (2) of the meetings of each of the JSC and JDC per calendar year shall be held in person. Locations for meetings held in person shall alternate between Amicus' facilities in Cranbury, New Jersey, and Shire's facilities in Cambridge, Massachusetts, or at such other locations as the Parties may otherwise agree. A reasonable number of other employees of each Party involved in the Development, Manufacture or Commercialization of Licensed Products may attend Committee meetings as nonvoting participants with the approval of the respective Committee, and, with the consent of each Committee Co-Chair, which consent shall not be unreasonably withheld, a reasonable number of consultants, representatives or advisors involved in the Development, Manufacture or Commercialization of Licensed Products may attend Committee meetings as nonvoting observers, provided that such consultants, representatives and advisors are under obligations of confidentiality and non-use applicable to the Confidential Information of each Party that are at least as stringent as those set forth in Article 14. Each Party shall be responsible for all of its own expenses of participating in the JSC, JDC, JCC and any Special Committee.

3.5.3 Decision-Making and Dispute Resolution. Decisions of each Committee shall be made at a duly called meeting of the applicable Committee. Shire's members of each Committee shall collectively have one (1) vote and Amicus' members of each Committee shall collectively have one (1) vote, with decisions made by unanimous vote (assuming a quorum of at least two (2) representative members from each Party, and with each Party's vote being cast by such Party's Committee Co-Chair of the relevant Committee). Each Committee may act on a specific issue without a meeting if it is documented in a written consent signed by each of the Co-Chairs of the applicable Committee from each Party. Notwithstanding anything herein to the contrary, no Committee shall have authority to amend, modify or waive compliance with this Agreement or the Related Agreements. If a Committee fails to reach consensus on an issue specifically designated in this Agreement for its decision, the matter shall be resolved under the procedures set forth in Section 16.8.

3.5.4 Committee Co-Chairs. Each calendar year, each Party shall appoint one of its representatives on each Committee to co-chair meetings of such Committee (the "***Committee Co-Chair***"). For each Committee, the Committee Co-Chairs shall coordinate and prepare the agenda, ensure the orderly conduct of meetings and prepare and issue minutes of each meeting within thirty (30) days thereafter. Such minutes will not be finalized until the Committee Co-Chair from each Party have reviewed and confirmed the accuracy of such minutes in writing. The Committee Co-Chairs will solicit agenda items from the members of the applicable Committee and provide an agenda along with appropriate information for such agenda reasonably in advance of each meeting. It is understood that such agenda will include all items requested by either Committee Co-chair for inclusion therein.

3.5.5 Reports. In addition, subject to the foregoing, each Party shall keep the other Party (through the relevant Committees) informed of Development, Manufacturing and Commercialization activities pertaining to Licensed Products in the Field in the applicable Territory

by such Party, including by providing regular reports to the relevant Committees summarizing such activities, and such other information as the other Party may reasonably request from time to time.

3.6 Alliance Managers. Within thirty (30) days following the Effective Date, each Party shall appoint a representative (an "***Alliance Manager***"), who will be at the director (or its equivalent) level or above, 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 Party may replace its Alliance Manager with an alternative representative satisfying the requirements of this Section 3.6 at any time with prior written notice to the other Party.

ARTICLE 4
DEVELOPMENT

4.1 Overall Efforts in Development. Amicus and Shire shall establish and implement the Development Plans in a prompt and expeditious manner with respect to each Licensed Product within the Field, and in a manner that harmonizes the Development of Licensed Products within the Field towards (a) a common registration dossier as a basis for license applications in the Primary Markets and, to the extent described in Section 4.2.5(e) below, Japan, and (b) Regulatory Approval for such Licensed Product in each of the Primary Markets. The Parties shall use Commercially Reasonable Efforts to ensure that each Development Plan provides at all times for adequate resources to achieve such result in an expeditious and efficient manner.

4.2 Development Plans.

4.2.1 General. The JDC shall establish a rolling three (3) calendar year plan and budget for the cooperative Development of each Licensed Product within the Field under this Agreement (as such may be amended from time to time in accordance with this Agreement, and as approved by the JSC, each a "***Development Plan***"). Each Development Plan shall include (a) a reasonably detailed written plan of Development activities and budget for the first thirteen (13) months of such period, together with the JDC's then-current preliminary estimate of the Development activities and budget for the final twenty-three (23) months of the rolling thirty-six (36) month period (such twenty-three (23) month estimated plan and budget, together with the items in clause (b) below for such 23-month period, being referred to below as the "***Forecast***"), (b) an allocation of Development activities between the Parties for the first thirteen (13) months of such period, including but not limited to the number of allocated full time equivalent personnel and the applicable FTE Rate and other out-of-pocket expenses to be incurred by each Party during such period, together with an overall allocation of responsibilities for activities to be conducted during the remaining twenty-three (23) months covered by such Development Plan, and (c) the overall program of Development for such Licensed Product within the Field, including clinical studies, regulatory strategies and other elements for obtaining Regulatory Approval of such Licensed Product in each country within the Primary Markets. It is understood that the JDC will modify and update the Forecast annually in connection with the procedure for amending and updating each Development Plan under Sections 4.2.3 and 4.2.4 below. In addition, the Parties shall cooperate to establish

additional non-binding forecasts of Development Costs for the rolling four (4) year period beyond the three (3) year term of each Development Plan (including as updated).

4.2.2 Initial Development Plans. The initial Development Plans for Plicera, Amigal and AT2220 are attached hereto as Appendix 4.

4.2.3 Amendments. Each Development Plan shall be updated by the JDC for approval by the JSC, not less than annually (as set forth in Section 4.2.4 below), or more frequently as needed to take into account completion, commencement or cessation of Development activities not contemplated by the then current Development Plan. The JDC will submit to the JSC for approval any material amendment to each Development Plan in advance of implementation of such amendment, including, without limitation, any amendment that effects a material increase of the budget or timeline in effect for the current year of such Development Plan, subject to Section 4.2.4 below.

4.2.4 Timing and Process for Amendments. With respect to each Development Plan, by September 15 of each calendar year after the Effective Date commencing in 2008, the JDC shall present to the JSC for its review and approval a proposed Development Plan for the next three (3) calendar years in the form described in Section 4.2.1 above. If such revised Development Plan is not approved by the JSC by January 1 of a calendar year, then, until such time as a revised Development Plan is either approved by the JSC or established pursuant to the dispute resolution procedure set forth in Section 16.8 below, (a) the preceding Development Plan (including the Forecast for the applicable period) shall continue to govern the Parties' Development activities, (b) each Party shall be permitted to conduct 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 Section 7.4.1 below, and (c) in any case each Party may continue any on-going clinical trials initiated by such Party in accordance with such preceding Development Plan, and the reasonable costs incurred by such Party in connection with such clinical trials shall continue to be shared by the Parties in accordance with Section 7.4.1 below.

4.2.5 Development Activities. In addition to the information described in Section 4.2.1 above, each Development Plan shall be as further described in this Section 4.2.5.

(a) Unless otherwise agreed by the Parties, each Development Plan shall allocate to Shire responsibility for (i) strategic and operational control of Regulatory Approval within the Shire Territory, including conducting meetings on programs relating to Regulatory Filings and meetings on pivotal study designs, and submissions leading up to and subsequent to the filings for Regulatory Approval (such as scientific advice and pre-MAA meetings), (ii) managing relationships with physicians and other key personnel at clinical trial sites in the Shire Territory in connection with such Development Plan, (iii) to the extent included in such Development Plan, any Development activities to be undertaken in Japan, and (iv) such other activities as the Parties mutually agree from time to time. In furtherance of the foregoing and as contemplated by Section

9.22, Amicus shall have the opportunity to accompany Shire to meetings with Regulatory Authorities in the Shire Territory.

(b) Unless otherwise agreed by the Parties, each Development Plan shall allocate to Amicus responsibility for (i) strategic and operational control of Regulatory Approval (including conducting meetings and other related activities as described in (a)(i) above) for the Amicus Territory, (ii) managing relationships with physicians and other key personnel at clinical trial sites in the Amicus Territory in connection with such Development Plan, (iii) control of clinical operations for all phase I clinical trials and Phase II Clinical Trials, and (iv) preclinical activities (including ongoing non-clinical testing).

(c) By January 15, 2008, the JSC shall establish the final versions of the initial Development Plans referenced in Section 4.2.2, including Development activities and budgets as contemplated by Section 4.2.1. Such final Development Plans shall set forth the allocation between the Parties of Development activities other than those described in (a) and (b) above. For clarity, it is understood that, until such time such final versions of the initial Development Plans are so established, the initial Development Plans referenced in Section 4.2.2 shall continue to govern the Parties' Development activities. Notwithstanding anything herein, the 2008 budget for Development shall not exceed **[***]**without the mutual consent of the Parties.

(d) Notwithstanding anything herein, within the **[***]** of the Effective Date no pivotal clinical trial or Phase III Clinical Trial under a Development Plan shall be conducted without the prior written consent of each Party.

(e) Each Development Plan shall be directed to those activities necessary to obtain Regulatory Approval of the applicable Licensed Product in the Field in the Primary Markets and, to the extent the Parties agree, Japan. In addition, the Parties may, from time to time, agree to include certain Post-Marketing Studies under a Development Plan and to share the costs thereof in accordance with Section 7.4 below; provided, however, that unless so included, the conduct of Post-Marketing Studies shall be as addressed in Section 4.3.

(f) It is understood that, from time to time, it may be necessary for Shire to conduct additional Development activities beyond that set forth in a Development Plan, in order to obtain Regulatory Approval for a Licensed Product in a country of the Shire Territory other than the Primary Markets. Shire may conduct such additional Development activities outside of the Development Plans at its own cost.

4.3 Post-Marketing Studies; Monitoring of Independent Trials

4.3.1 Post-Marketing Studies. For clarity, if a Party desires to conduct a Post-Marketing Study that has not been approved by the Parties for inclusion under the applicable Development Plan under Section 4.2.5(e) above, such Party may perform such Post-Marketing Study, at its own expense. Further, the Party conducting such Post-Marketing Study outside such Development Plan shall not be required to share the Data (other than safety Data, in accordance with

the Pharmacovigilance Agreement or as otherwise required by a governmental or a regulatory authority or applicable Laws) resulting from such Post-Marketing Study.

4.3.2 Notice; Suspension of Independent Trials. The Party proposing to conduct a Post-Marketing Study outside the applicable Development Plan in accordance with Section 4.3.1 above or any other clinical trial of a Licensed Product (other than **[***]** for [***]'s) but outside the Development Plan (each, an "***Independent Trial***") shall notify the other Party at least sixty (60) days prior to submitting a protocol to the Institutional Review Board or Ethics Committee, as applicable, for such Independent Trial (which notice shall include a synopsis, in reasonable detail, of the proposed protocol). The Party proposing to conduct such Independent Trial may proceed with such trial (as described in its notice to the other Party) after such sixty (60) day notice period unless such other Party reasonably and in good faith objects to such protocol on the grounds that it would cause, or would have an unreasonable risk of causing, a material adverse effect upon the Development or Commercialization of a Licensed Product containing the same Compound. In the case of such an objection, the objecting Party shall give written notice of its objection (including a reasonably detailed explanation of the basis therefor) to the JSC within such sixty (60) day period and the Party proposing such protocol shall not commence such trial pending the resolution of such matter pursuant to this Section 4.3.2. If the JSC is unable to reach consensus on such matter within thirty (30) days after such matter is referred to the JSC, then, upon written notice of either Party to the other Party, such matter shall be resolved as a Committee Dispute in accordance with the provisions of Section 16.8 below.

4.4 Subcontractors. Except as otherwise set forth in this Agreement, each Party may engage subcontractors to perform, under its direction, specific functions that are assigned to it hereunder or that it carries out in the exercise of its rights hereunder, in each case in accordance with this Section 4.4. Each Party shall be fully responsible under this Agreement for the performance hereof by its permitted subcontractors as if such Party so performed this Agreement itself.

4.5 Combination Products. For purposes hereof, a "***Combination Product***" means any Licensed Product containing a Compound combined with one or more other active ingredients (e.g., a co-formulation) or a product in which both a Licensed Product and one or more other products or components are packaged together and sold for a combined price. In the event that Shire desires to Develop or Commercialize a Combination Product:

4.5.1 if such Combination Product, in Shire's good faith determination, is not intended for Commercialization outside the Shire Territory and/or Shire does not Control the Patent Rights necessary to Develop and Commercialize such Combination Product outside the Shire Territory, then (a) Shire may Develop and Commercialize such Combination Product solely in the Shire Territory and (b) the **[***]**; and

4.5.2 if the Parties agree to Commercialize such Combination Product in the Amicus Territory and Shire Controls the Patent Rights necessary to Develop and Commercialize such Combination Product in the Amicus Territory, then the Parties **[***]**. It is understood that neither Party is obligated to agree on such terms and if the Parties cannot so agree the Combination

Product shall not be included in a Development Plan and Shire may pursue such Combination Product under Section 4.5.1.

4.6 Term of Ongoing Development and Committee Obligations. The Parties' obligations under Sections 4.1 and 4.2 (and the Development Plans), and to share Development Costs under Section 7.4.1 below, and Amicus' supply and Manufacturing obligations under Article 8 below, shall terminate eighteen (18) years after the Effective Date. At such time, all Committees will terminate. However, each Party will continue to have an approval right with respect to matters specified to be decided by such Committees 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 such Committee, the matter shall be determined by binding arbitration in accordance with the procedures in Sections 16.8.1(b) through (d) below.

ARTICLE 5
COMMERCIALIZATION in the Shire Territory

5.1 General. Subject to the terms and conditions of this Agreement, Shire shall have the sole right to control the Commercialization of the Licensed Products in the Field in the Shire Territory.

5.2 Diligence. Shire shall use Commercially Reasonable Efforts to obtain Regulatory Approval of, and to Commercialize, the Licensed Products in the Field in the Shire Territory.

5.3 Territory Compliance. Each Party shall use diligent efforts to take the actions necessary to prevent importation or sales of Licensed Products sold by such Party into the other Party's Territory by a Third Party, including any such actions as are determined by the JSC under Section 3.1.1(c).

5.4 Bundling. **[***]** in order to benefit sales or prices of other products offered for sale by Shire to such customer or that discounts the price of the Licensed Product disproportionately to the other products included in such multiple product offering.

ARTICLE 6
CERTAIN OTHER ACTIVITIES

6.1 Label Expansions and New Formulations within the Field.

6.1.1 Proposal to JSC. In the event that either Party (the "***Developing Party***") proposes to Develop (a) a Licensed Product for a label expansion within the Field (a "***Label Expansion***") or (b) a new pharmaceutical formulation containing a Compound for an indication within the Field (a "***New Formulation***"), such Party shall make a written proposal to the JSC for the Development thereof, including a proposed work plan, budget, timeline, any Third Party Technology under Section 10.6.2 and, in the case of Shire, any formulation technology under Section 6.1.2(d)(iii) (the "***JSC Proposal Notice***").

(a) Inclusion within Development Plan. If the JSC determines to include such Label Expansion or New Formulation in an applicable Development Plan or where applicable a new Development Plan, then such Development Plan shall include the work plan, budget and timeline proposed by the Developing Party, or as the JSC may otherwise determine. For purposes of the JSC determination of whether or not to include such Label Expansion or New Formulation under the applicable Development Plan, the Developing Party shall be deemed to have approved the proposal presented by it to the JSC as part of the JSC Proposal Notice under Section 6.1.1 above.

6.1.2 Independent Projects.

(a) Election. If the JSC does not determine to include such Label Expansion or New Formulation under the applicable Development Plan, then the Developing Party shall have the right, subject to the Opt-In Right in Section 6.1.3 below, to Develop and Commercialize such Label Expansion or New Formulation for such Licensed Product outside the Development Plans solely in the Developing Party's respective Territory (subject to the proviso in Section 2.1.3) at its own expense (it being understood that Development Costs incurred in connection therewith shall not be shared under Section 7.4.1 below), and such Label Expansion or New Formulation of such Licensed Product shall be referred to herein as an "***Independent Project***." Notwithstanding the above, the Developing Party shall have the right to conduct non-clinical Development of an Independent Project anywhere, including, but not limited to, outside its Territory.

(b) Provision of Development Update. Upon request by the non-Developing Party (the "***Non-Developing Party***"), the Developing Party shall provide to the Non-Developing Party a summary in reasonable detail of the Development results to date pertaining to such Independent Project.

(c) Statement of Independent Development Costs.

(i) Prior to sixty (60) days after the end of each calendar year of an Independent Project, the Developing Party shall provide the Non-Developing Party with a statement of the Independent Development Costs (as defined below) for the prior year (each, a "***Statement of Costs***") for such Independent Project.

(ii) As used herein, "***Independent Development Costs***" shall mean those internal and external costs actually incurred by the Developing Party outside the Development Plans that are specifically allocable to the Development of the applicable Independent Project. The allocation of such costs to an Independent Project shall be determined in a manner consistent with the manner in which costs are allocated to the Development Costs of Licensed Products under the Development Plans. For such purposes, the internal Development costs of the Developing Party shall be determined by applying the FTE Rate applied under the Development Plans for the same period.

(d) License; Disclosure of Know-How. If a Label Expansion or New Formulation is included in the Development Plan in accordance with Section 6.1.1(a) above, or if the

Non-Developing Party exercises the Opt-In Right with respect to an Independent Project in accordance with Section 6.1.3 below, the Developing Party shall promptly provide to the Non-Developing Party Know-How Controlled by the Developing Party that is necessary, useful or actually used to Develop such Label Expansion, New Formulation or Independent Project and to Develop and Commercialize such Licensed Product in accordance with this Agreement. In such event:

(i) For clarity, it is understood that the licenses granted to Shire under Section 2.1 above include the Licensed Product comprising such Label Expansion, New Formulation or Independent Project, as the case may be; and

(ii) Subject to Sections 6.1.2(d)(iii) and 10.6.2, Amicus shall have and is hereby granted a license, under Shire IP, to use, import, sell and Commercialize such Licensed Product in the Amicus Territory, and to Develop and Manufacture such Licensed Product, including the right to sublicense; provided, however, that Amicus shall not conduct any clinical trial of such Licensed Product in the Shire Territory without Shire's consent (not to be withheld unreasonably). As used herein, "Shire IP" shall mean (A) Know-How to be disclosed to Amicus under this Section 6.1, and (b) Patent Rights Controlled by Shire that are necessary or actually used to Develop, Manufacture or Commercialize such Licensed Product.

(iii) If a New Formulation is to be licensed under Section 6.1.2(d)(ii) above to Amicus and includes proprietary formulation technology Controlled by Shire, then Amicus shall pay to Shire a royalty on net sales of such New Formulation in the Amicus Territory pursuant to such license, based on commercially reasonable terms; provided that in the case of a New Formulation included in a Development Plan pursuant to Section 6.1.1(a) above, such formulation technology was generated or acquired by Shire (A) outside of this Agreement or (B) as part of a different Independent Project for which Amicus did not exercise its Opt-In Right under Section 6.1.1(a) and Shire thereafter conducted clinical Development. In addition such license shall be subject to Section 10.6.2. If the Parties are unable to agree on the rate and other terms of such royalty, the same shall be determined by the JSC. In such event, if Shire Manufactures such New Formulation, then Shire will supply such New Formulation to Amicus on reciprocal terms as provided in Article 8 below; in each case, mutatis mutandis. If the Parties are unable to agree on the application of any such terms, the matter shall be determined by the JSC. If Amicus' proposal for the royalty rate and other terms is not selected pursuant to Section 16.8, Amicus may revoke its exercise of the Opt-In Right for such New Formulation and such Opt-In Right for such New Formulation shall terminate as if not exercised.

6.1.3 Opt-In Right. In the case of an Independent Project, until the end of the Opt-In Period (as defined below), the Non-Developing Party shall have the right to opt-in for Development of the (a) the Label Expansion within the Field with respect to the particular Licensed Product in question and/or (b) the New Formulation of a Licensed Product, as applicable, comprising such Independent Project, as follows (the "***Opt-In Right***"):

(a) Notice. The Developing Party shall notify the Non-Developing Party of a submission of a request to a Regulatory Authority of a Primary Market country for the first [***] for an Independent Project, or the submission of the protocol for the first Phase III Clinical Trial for an Independent Project to an Institutional Review Board or Ethics Committee for approval, whichever occurs first (the "***Opt-In Notice***"). As used herein, the "***Opt-In Period***" with respect to an Independent Project shall mean the period beginning upon the date on which the Non-Developing Party elected not to jointly Develop such Label Expansion or New Formulation (as applicable) pursuant to Section 6.1.2(a) and ending on ninety (90) days after the later of (x) first [***] for such Independent Project or (y) the submission of the protocol to such Institutional Review Board or Ethics Committee for the first Phase III Clinical Trial of such Independent Project, provided, that in the event (x) does not occur then upon the dosing of the first patient in the first Phase III Clinical Trial for such Independent Project. Notwithstanding the foregoing, if so agreed by the Parties on a case-by-case basis, the Opt-In Period may be extended and if extended, the Non-Developing Party may exercise the Opt-In Right at such later time as is mutually agreed upon by the Parties. The Opt-in Notice shall include a good faith estimate of the date the Opt-In Period will expire, and notwithstanding the foregoing, the Opt-In Period shall not expire prior to such estimated date.

(b) Know-How. Promptly after a request by the Non-Developing Party made during the applicable Opt-In Period, the Developing Party shall provide to the Non-Developing Party reasonable access to the material Know-How of the Developing Party pertaining to such Independent Project, and shall cooperate to enable the Non-Developing Party to evaluate such Know-How, and Independent Project, in a prompt and efficient manner. In addition the Developing Party shall provide the Non-Developing Party safety data required in accordance with the Pharmacovigilance Agreement or as otherwise required by a governmental or a regulatory authority or applicable Laws. The Developing Party shall similarly provide to the Non-Developing Party promptly a statement of the total Independent Development Costs incurred by the Developing Party in performing such Independent Project. If additional Know-How is generated, or Independent Development Costs incurred, during the Opt-In Period after such request, the Developing Party shall promptly make such information available to the Non-Developing Party upon request to the extent necessary to enable the Non-Developing Party to evaluate such Know-How and Independent Project. Notwithstanding anything herein to the contrary, unless the Non-Developing Party exercises its Opt-In Right under Section 6.1.3 below with respect to such Independent Project, the Non-Developing Party shall not have the right to use, and shall not use, such Know-How or Data pertaining to such Independent Project provided to it pursuant to this Section 6.1.2(b) other than as safety data as allowed pursuant to the Pharmacovigilance Agreement or as otherwise required by a governmental or a regulatory authority or applicable Laws.

(c) Exercise. To exercise the Opt-In Right with respect to an Independent Project, the Non-Developing Party shall deliver, prior to the expiration of the Opt-In Period for such Independent Project, written notice to the Developing Party of such exercise and shall promptly reimburse the Developing Party for the Non-Developing Party's Reimbursable Share (as defined below) of the Independent Development Costs incurred by the Developing Party in performing such Independent Project (through the date of such exercise). For such purpose, the Non-Developing

Party's "***Reimbursable Share***" shall mean **[***]** of the Independent Development Costs for the Independent Project. In the event of a dispute as to the calculation of Independent Development Costs to be reimbursed, the Non-Developing Party shall promptly reimburse the Developing Party the amount described above with respect to any undisputed Independent Development Costs, and shall promptly reimburse any remainder (or if applicable the Developing Party shall promptly refund any amounts that were so paid but subsequently found not to be due) upon resolution of such dispute by the JSC and in the event that the JSC is unable to resolve such dispute, the matter shall be resolved by an audit in accordance with provisions of Sections 7.8.2 and 7.8.3. For purposes of clarity, the Non-Developing Party shall be the Auditing Party in accordance with Section 7.8.2.

6.1.4 Further Development and Commercialization of Independent Projects.

(a) Generally. If the Non-Developing Party does not exercise its Opt-In Right for an Independent Project during the Opt-In Period, then the Developing Party shall have the right to continue the Development and Commercialization of such Independent Project solely in its respective Territory (subject to the proviso in Section 2.1.3), provided the Developing Party shall have the right to conduct non-clinical Development anywhere, including, but not limited to, outside its Territory. It is understood that Net Sales of Licensed Products in the Shire Territory resulting from such Label Expansion or New Formulation shall be included in Net Sales calculations for purposes of Sections 7.2.2 and 7.3 below.

(b) Clinical Trials. The conduct of clinical trials as part of an Independent Project shall be subject to Section 4.3.2 above.

(c) Manufacturing. If Shire elects to Develop and Commercialize a New Formulation as an Independent Project, Amicus will supply to Shire in accordance with Article 8 below additional quantities of the applicable Compound for use in such New Formulation. Unless otherwise agreed, however, Amicus will not be required to arrange for supplies of final formulated Licensed Products incorporating such New Formulation, and Shire shall have the right to Manufacture and have Manufactured such supplies of final formulated Licensed Products incorporating such New Formulation.

6.1.5 Activities Outside the Field. The application of Sections 6.1.1–6.1.4 is limited to activities within the Field with respect to the Licensed Products. In this regard nothing herein shall be deemed to grant to Shire the right to Develop or Commercialize Licensed Products (including New Formulations) outside the Field. The Parties may discuss from time to time expanding the Field to include indications outside the Field for a given Compound or Licensed Product, and if the Parties so agree, then the Field shall be deemed to include such additional indication with respect to Licensed Products containing such Compound. Unless so agreed, however, during the Term (a) Amicus shall not, directly or indirectly, Develop (other than as a Licensed Product in accordance with this Agreement) or Commercialize in the Shire Territory a product containing a Compound other than an [***] for [***]'s and (b) Shire shall not outside the Field, directly or indirectly, Develop or Commercialize in the Shire Territory a product containing a Compound,. Subject to (a) Sections 6.2.1 and 6.2.2, Amicus may, directly and/or indirectly,

Develop or Commercialize an [***] for [***]'s in the Shire Territory and (b) Section 4.3.2, Amicus may, directly and/or indirectly, Develop outside the Field a product containing a Compound in the Shire Territory for Commercialization in the Amicus Territory.

6.2 **[***]** for [***]'s.

6.2.1 [***]'s Option. Subject to Section 6.2.1(e) below, Shire shall have and Amicus hereby grants to Shire an option to expand the Field with respect to products containing **[***]** as set forth below in this Section 6.2.1 (the "***[***]'s Option***").

(a) Notice. Upon the scheduling of the first [***] with respect to an [***] for [***]'s, Amicus shall notify Shire in writing. Promptly following such [***] with respect to an [***] for [***]'s, Amicus shall provide to Shire written notice of such [***], together with the data package submitted by Amicus to the FDA for purposes of such [***] (the "***[***]'s Option Notice***"). Shire shall have the right to exercise the [***]'s Option within sixty (60) days after its receipt of the [***]'s Option Notice (the "***[***]'s Option Period***") by providing written notice to Amicus of such intent to exercise the [***]'s Option. Amicus shall promptly provide Shire any additional Data or information (e.g. minutes of the [***] and response letter from the FDA with respect to the [***]) that it has after providing the [***]'s Option Notice up to the exercise or expiration of the [***]'s Option. For purposes of clarity, Amicus shall not grant to a Third Party any license or other rights to Commercialize an [***] for [***]'s in the Shire Territory prior to the expiration of the [***]'s Option Period and further subject to Section 6.2.2 below.

(b) Exercise. Upon exercise of the [***]'s Option within the [***]'s Option Period and payment of the exercise fee (the "***[***]'s Option Exercise Fee***") within ten (10) Business Days [***], the Field with respect to **[***]** (and any other **[***]** for [***]'s) shall be deemed to include the diagnosis, treatment and/or prevention of [***]'s and the JDC shall promptly propose, for approval by the JSC, modifications to the applicable Development Plan to include Development of such [***] for [***]'s. Following exercise of the [***]'s Option, Amicus shall continue performing further activities related to the Development of such an **[***]** for [***]'s in accordance with its own Development plans for a period of up to one (1) year after exercise of the [***]'s Option, or until such earlier time as the JSC approves such a Development Plan therefor, and thereafter the further Development of such [***] for [***]'s shall be conducted in accordance with such Development Plan, as modified by the JSC from time to time. All costs reasonably incurred by Amicus in performing such activities (i.e., those after the exercise of the [***]'s Option but prior to the JSC's establishment of a modified Development Plan), and those conducted pursuant to the modified Development Plan so established, shall be shared in accordance with Section 7.4.1 below.

(c) [***]'s Option Exercise Fee and Milestone Payments. In the event Shire exercises the [***]'s Option in accordance with the foregoing, [***] to be made by Shire with respect to the Development and Commercialization of Licensed Products for [***]'s hereunder. It is understood that the amount of such [***]'s Option Exercise Fee and milestone payments shall [***].

(d) Cooperation. From the Effective Date until the end of the [***]'s Option Period, Amicus shall keep Shire informed of Amicus' progress in Developing [***] for [***]'s and shall cooperate with Shire to facilitate Shire's understanding of the [***] for [***]'s, including, but not limited to, providing access to all Data generated regarding [***] for [***]'s promptly upon Shire's written request.

(e) Amicus' Election Not to Proceed. If, prior to the [***]'s Option Notice, Amicus notifies Shire in writing that it has made the election not to further Develop for and/or seek Regulatory Approval for Commercialization in the Shire Territory, or Commercialize, an [***] for [***]'s in the Shire Territory, the [***]'s Option, and this Section 6.2.1, shall terminate and not apply; provided, that if Amicus makes such election under this Section 6.2.1(e), Amicus shall not (directly or indirectly) Commercialize an [***] for [***]'s in the Shire Territory (for so long as [***] is a Compound hereunder).

6.2.2 Right of First Refusal. Amicus hereby grants to Shire a right of first refusal to Develop and exclusively Commercialize one or more [***] for [***]'s in the Shire Territory as set forth below in this Section 6.2.2 (the "***Right of First Refusal***").

(a) Procedure. Prior to granting to a Third Party a license or other rights to Commercialize an [***] for [***]'s in the Shire Territory, Amicus shall so advise Shire in writing setting forth the terms and conditions under which Amicus would grant such rights (an "***Offer***") and including a copy of the proposed definitive agreement to grant such rights to Shire (a "***Right of First Refusal Notice***"). **[***]** of the date of the Right of First Refusal Notice (the "***RFR Acceptance Period***"), Shire shall notify Amicus in writing whether Shire desires to enter into the transaction in the Right of First Refusal Notice in accordance with the terms set forth therein, together with a fully executed copy of the definitive agreement included in such Right of First Refusal Notice (a "***RFR Acceptance***"). In the event that Shire shall have failed to deliver a RFR Acceptance **[***]**, then Shire shall be deemed to have waived its Right of First Refusal under this Section 6.2.2, and Amicus shall be free to execute and close on the Offer with a Third Party solely on the terms provided in the Right of First Refusal Notice (which for purposes of clarity shall mean the execution by a Third Party of the definitive agreement offered to Shire with such non-substantive changes as are necessary to substitute such Third Party for Shire); provided that if such definitive agreement is not consummated within **[***]** after the end of the RFR Acceptance Period, or if, during such **[***]** period Amicus proposes to grant to a Third Party rights to Commercialize an [***] for [***]'s on terms that are different than those set forth in the Offer (any terms different than those provided in the definitive agreement offered to Shire), Amicus shall be obligated to follow the procedures set forth in this Section 6.2.2 prior to granting to a Third Party any right or license to Commercialize an [***] for [***]'s.

(b) No Implied Obligations. The only obligations of Amicus under this Section 6.2.2 and Section 6.2.1 above are as expressly stated therein, and there are no further implied obligations relating to the matters contemplated therein. Without limiting the foregoing, it is understood that (i) Amicus is not obligated to identify the Third Party(ies) to whom Amicus would

grant rights to [***] for [***]'s, (ii) modifications or improvements may be made to such [***] after the date of the Right of First Refusal Notice and (iii) a transfer of rights in connection with an assignment of this Agreement in accordance with Section 16.1 below shall not be subject to this Section 6.2.2, provided, that this Section 6.2.2 shall apply to such assignee.

(c) Termination. Section 6.2.2 shall terminate for all purposes upon the first grant to a Third Party by Amicus of rights to Commercialize an [***] for [***]'s in accordance with this Section 6.2.2; and in any case this Section 6.2 (including both Sections 6.2.1 and 6.2.2 but not Section 6.2.1(e)) shall expire **[***]** after the First Commercial Sale of **[***]** (or, if earlier, another Licensed Product containing **[***]**) within the Field in a Primary Market country in the Shire Territory, after which time Amicus shall have no further obligation and Shire shall have no further rights under this Section 6.2.

6.3 Related Products.

6.3.1 Related Product Notice. At least thirty (30) days prior to dosing the first patient in the first human clinical trial of a Related Product within the Field, Amicus shall notify Shire in writing, which notice shall include a copy of the IND submitted to the FDA or other applicable Regulatory Authority for such trial (a "***Related Product Notice***"), subject to Section 6.3.6. For purposes hereof, "***Related Product***" is defined as a small molecule that selectively binds to the active site of (a) a-galactosidase A for the treatment or prevention of Fabry Disease, (b) b-glucocerebrosidase for the treatment or prevention of Gaucher Disease, (c) a-glucosidase for the treatment or prevention of Pompe Disease or (d) to the extent the Parties agree pursuant to Section 6.1.5 to expand the Field for a Compound and agree to add an additional target for such Compound, such other target for such indication, and in each such case whose primary therapeutic activity results from such selective binding, provided, that the restrictions of this Section 6.3 shall not apply to [***] for [***]'s or a Licensed Product being Commercialized pursuant to this Agreement. Each of the enzymes referenced under clauses (a), (b), (c) and (d) of this Section 6.3.1 is referred to herein as a "***Target***."

6.3.2 Related Product Opt-In Right. Shire shall have the right, exercisable **[***]** after receiving the Related Product Notice (the "***Related Product Opt-In Period***"), to designate that (a) the active pharmaceutical ingredient of such Related Product shall be added as a Compound hereunder, (b) the formulation described in the IND included in the Related Product Notice shall be added as a Licensed Product hereunder and (c) the Field with respect to such Licensed Product shall be limited to the specific disease for which such Related Product is being developed (i.e., Gaucher Disease, Fabry Disease or Pompe Disease or such other indication as is then included in the Field under Section 6.1.5, as applicable) (the "***Related Product Opt-In Right***").

6.3.3 Exercise. To exercise the Related Product Opt-In Right with respect to a Related Product, Shire shall deliver written notice to Amicus of such exercise within the Related Product Opt-In Period for such Related Product, and shall promptly reimburse Amicus for **[***]** of the costs incurred by Amicus outside the Development Plans that are specifically attributable to the

Development of such Related Product prior to such exercise, determined in accordance with Section 6.5.4.

6.3.4 Development Plan. Upon such timely exercise of the Related Product Opt-In Right, the JDC and JSC shall promptly establish a new Development Plan for such Related Product (i.e., as a Licensed Product).

6.3.5 Milestone Payments and Royalties. Following exercise of the Related Product Opt-In Right, Shire shall pay to Amicus royalty payments under Section 7.3 with respect to such Related Product as a Licensed Product. In addition, Shire shall pay to Amicus an additional set of Milestone payments for such Related Product, in an amount equal to **[***]** of the amounts for the achievement of the same events as provided for the first Licensed Product Developed for the same Field under Section 7.2.1, provided, that such payments shall not be due until the first Regulatory Approval of the first Licensed Product for the same disease within the Field as that of such Related Product. For clarity, upon Regulatory Approval of the first Licensed Product in a particular Field, payments for Milestones (as provided in this Section) for a Related Product previously achieved shall be due to Amicus.

6.3.6 Related Products to be Commercialized Solely in the Amicus Territory. Notwithstanding the foregoing, the Related Product Opt-In Right shall not apply with respect to a particular Related Product that Amicus elects to Commercialize solely in the Amicus Territory throughout the Term. Accordingly, if Amicus notifies Shire in writing that it has made the election not to Commercialize such Related Product in the Shire Territory, the Related Product Opt-In Right, and this Section 6.3, shall not apply to such Related Product; provided that if Amicus makes such election under this Section 6.3.6, Amicus shall not Commercialize such Related Product (for so long as the same remains a Related Product) in the Shire Territory prior to the end of the **[***]** period following Regulatory Approval in the Shire Territory of the first Licensed Product for such disease within the Field.

6.4 Termination by JSC; Back-Up Compounds. The JSC may, from time to time, terminate the Development of a Licensed Product for the Shire Territory or the Amicus Territory , in accordance with Section 6.4.1.

6.4.1 Termination of Development in Shire Territory or Amicus Territory. If, at the request of a Party (the "***Requesting Party***"), the JSC determines that it is not commercially reasonable to continue to Develop and/or to Commercialize a Licensed Product in the Requesting Party's Territory (regardless of whether it is commercially reasonable to do so in the other Party's Territory), then such Licensed Product shall be terminated in the Requesting Party's respective Territory (i.e., the Shire Territory if Shire is the Requesting Party and the Amicus Territory if Amicus is the Requesting Party). Upon such termination:

(a) If such termination is with respect to the Amicus Territory only, then: (i) the Licensed Product that was the subject of such termination shall remain a Licensed Product for all purposes of this Agreement and shall not revert to Amicus by reason of such termination, and the

provisions of Sections 6.4.2 and 6.4.3 shall not apply with respect to such termination; (ii) Shire shall have the right to continue Developing and to Commercialize such Licensed Product as an Independent Project outside the Development Plans in the Shire Territory at its own expense (which costs shall not be shared under Section 7.4.1); (iii) Amicus' obligations under Sections 4.1 and 4.2, and under the Development Plan for such Licensed Product (to the extent such Development Plan applies to such Licensed Product), shall terminate, subject to Amicus using Commercially Reasonable Efforts to transition any such Development activities that relate to Development and/or Commercialization of such Licensed Product in the Shire Territory to Shire; and (iv) Amicus' obligation to supply such Licensed Product under Article 8 shall terminate on the second anniversary of such termination or such earlier date as Shire establishes an alternative source of supply for such Licensed Product, during which period Amicus shall cooperate reasonably to transition to Shire or its designee the Manufacture of such Licensed Product. In the event of termination for the Amicus Territory only under this Section 6.4.1(a), Amicus shall not Develop or Commercialize such Licensed Product or the Compound contained therein anywhere in the Territory during the remaining Term for so long as the same remains a Licensed Product; or

(b) If such termination is with respect to the Shire Territory, then such Licensed Product shall cease to be a Licensed Product and the provisions of Sections 6.4.2, 6.4.3 and 6.4.4 shall apply (in which case such Licensed Product shall be deemed a Terminated Product), and Shire shall use Commercially Reasonable Efforts to transition any Development activities that relate to Commercialization of such Licensed Product in the Amicus Territory to Amicus. For clarity, Shire shall have no further obligations under this Agreement with respect to such Licensed Product under Sections 4.1 and 4.2, the Development Plan for such Licensed Product, and Article 5.

6.4.2 Intentionally Omitted.

6.4.3 Reversion.

(a) In the event a Licensed Product ceases to be a Licensed Product pursuant to Section 6.4.1(b) or 7.2.5, or Section 15.3.2, (i) such Licensed Product shall be deemed a "Reverted Product" under Section 15.5.2, (ii) the Compound contained therein shall cease to be a Compound for all purposes of this Agreement to the extent no other Licensed Product containing such Compound is actively being Developed (including clinical Development) or Commercialized by Shire hereunder, (iii) Shire shall have no further rights under Section 4.3.2 with respect to clinical trials involving such Licensed Product, (iv) Amicus shall have no further obligations under Article 8 with respect to such Licensed Product, (v) Shire shall have no further prosecution, maintenance and enforcement rights under Sections 10.2 and 10.3 with respect to Patent Rights specifically directed to such Licensed Product, (vi) Shire shall have no further rights under Section 14.5 with respect to such Licensed Product, and (vii) the provisions of Sections 15.5.2(a), (c), (d), (e), (f), (g) and (h) shall then apply with respect to such Licensed Product as if this Agreement had terminated under Section 15.3.1. For clarity, Shire shall have no further obligations under this Agreement with respect to such Licensed Product under Sections 4.1 and 4.2, the Development Plan for such Licensed Product, and Article 5.

(b) In addition, subject to subsection (iv) below, in the event of such reversion:

(i) with respect to Amigal, then Fabry Disease (plus any additional indication for DGJ included within the Field pursuant to Section 6.1.5) shall thereafter be excluded from the definition of Field; and the compounds and products described in Section 6.3.1(a) shall cease to be Related Products;

(ii) with respect to Plicera, then Gaucher Disease (plus any additional indication for [***] included within the Field pursuant to Section 6.1.5 or Section 6.2) shall thereafter be excluded from the definition of Field; the obligations under Sections 6.2.1 and 6.2.2 shall terminate; and the compounds and products described in Section 6.3.1(b) shall cease to be Related Products; and

(iii) with respect to AT2220, then Pompe Disease (plus any additional indication for DNJ included within the Field pursuant to Section 6.1.5) shall thereafter be excluded from the definition of Field; and the compounds and products described in Section 6.3.1(c) shall cease to be Related Products; in each of subsections (i) and (ii) above and this subsection (iii), for all purposes of this Agreement.

(iv) Notwithstanding the foregoing, if (x) a salt, enantiomer, metabolite or polymorph of such Compound is then actively being Developed (including clinical Development) or being Commercialized, or (y) an additional Compound has been added to this Agreement under Sections 6.3 or 6.4.4 (or 1.5.4) for a disease described in subsection (b)(i), (ii) or (iii) above and such Development is continuing under a Development Plan or as an Independent Project (or a Licensed Product is being Commercialized hereunder for such disease), then in either such case such disease shall not be deemed excluded from the Field under this Section 6.4.3 unless such Compound becomes a Reverted Product; and in the case of subsection (ii) if another Licensed Product containing [***] is actively being Developed (including clinical Development) or Commercialized hereunder, then Sections 6.2.1 and 6.2.2 shall not terminate pursuant to subsection (ii) above.

6.4.4 Back-Up Compounds.

(a) Back-Up Compound Opt-In Right. If a Licensed Product is terminated pursuant to Section 6.4.1(b) or 6.4.2 (a "***Terminated Product***") and at such time there is no other Licensed Product containing the same Compound as the Terminated Product being Developed hereunder for the disease within the Field to which such Terminated Product was directed, then upon request by Shire, the JSC shall take the actions set forth in this Section 6.4.4 to select a Back-Up Compound (as defined below) to be added as a Compound hereunder (the "***Back-Up Compound Opt-In Right***"). For purposes hereof, a "***Back-Up Compound***" shall mean an active pharmaceutical ingredient Controlled by Amicus:

(i) that is a Related Product for the same Target and disease within the Field as the applicable Terminated Product (e.g., a Related Product described in Section 6.3.1(a), if the Terminated Product was being Developed for Fabry Disease); and

(ii) that is either (x) referenced on Exhibit 6.4.4 hereto, (y) (A) for which Amicus has performed or is performing human clinical trials for such disease within the Field, or (B) for which Amicus first initiates such a human clinical trial within three (3) years after the date of such termination, or (z) a pre-clinical compound for which Amicus has performed at least one (1) study in an animal model in the Field that Amicus has not designated as a candidate for Development outside the Field.

(b) Notice. Promptly following termination of a Licensed Product as described in this Section 6.4.4(a)Amicus shall provide the JSC and Shire with a description of the Back-Up Compound(s) in existence at the time of such notice. If Shire exercised the Back-Up Compound Opt-In Right but the JSC does not select any of the Back-Up Compounds in existence at such time, and Amicus within three (3) years after the date of such termination initiates a human clinical trial of a Back-Up Compound within the Field, then Amicus shall notify the JSC at least thirty (30) days before initiating the first such trial or conducting the first such pre-clinical animal study of the first such Back-Up Compound (each of the notices in (i) and (ii), a "***Back-Up Compound Notice***").

(c) Exercise. To exercise the Back-Up Compound Opt-In Right, Shire shall notify Amicus and the JSC in writing of such exercise within **[***]** after its receipt of the Back-Up Compound Opt-In Notice (the "***Back-Up Opt-In Exercise Period***"). Upon Shire's timely exercise of the Back-Up Compound Opt-In Right, the JSC may select one such Back-Up Compound to be included in the Development Plan pursuant to such Back-Up Compound Opt-In Right provided, that the JSC may select multiple Back-Up Compounds from those referenced in Exhibit 6.4.4. It is understood that the JSC (rather than Shire) shall select the Back-Up Compound to be added as a Compound pursuant to such Back-Up Compound Opt-In Right. Upon the JSC's selection, and Shire's acceptance, of the Back-Up Compound:

(i) such Back-Up Compound shall become a Compound hereunder, and the Field with respect to Licensed Products containing such Compound shall be the same disease within the Field for the applicable Terminated Product;

(ii) Shire shall promptly reimburse Amicus for **[***]** of the costs incurred by Amicus outside the Development Plans that are specifically attributable to the Development of such Back-Up Compound during the period prior to such exercise, determined in accordance with Section 6.5.4;

(iii) any unpaid milestone payments under Section 7.2.1 with respect to the applicable Terminated Product shall be payable upon achievement of such Milestone by a Licensed Product containing such Back-Up Compound; and

(iv) Amicus may elect, within **[***]** following such Back-Up Compound becoming a Compound, to terminate Development and Commercialization of such Compound and any Licensed Product containing such Compound in the Amicus Territory provided, Amicus shall not have the right to terminate a Back-Up Compound selected by the JSC, if Amicus had proposed that the JSC select such Back-Up Compound.

(d) Failure to Exercise. If (i) Shire does not exercise the Back-Up Compound Opt-In Right with respect to a particular Terminated Product within the applicable Back-Up Opt-In Exercise Period, (ii) no Back-Up Compound is designated as a Compound under this Section 6.4.4 with respect to the Terminated Product within three (3) years following the date such Terminated Product is terminated under Section 6.4.1(b) or 6.4.2, or (iii) Shire does not accept the Back-Up Compound selected by the JSC within **[***]** of the selection of the JSC, then in any such case the Back-Up Compound Opt-In Right, and the provisions of this Section 6.4.4, shall terminate with respect to that Terminated Product and all Back-Up Compounds directed to the disease within the Field with respect to which such Terminated Product was directed, provided, Shire shall be deemed to have accepted the Back-Up Compound selected by the JSC, if Shire had proposed that the JSC select such Back-Up Compound.

6.5 Additional Terms Regarding Related Product/Back-Up Compound Opt-In Rights.

6.5.1 Confirmation of Termination. In the case of a JSC vote to terminate a Licensed Product under Section 6.4.1 or 6.4.2, each Party's vote on the JSC must be reflected in a written notice signed by both the Committee Co-Chair and the Chief Executive Officer of such Party.

6.5.2 Additional Know-How. During the Related Product Opt-In Period and the sixty (60) day period after each Back-Up Compound Notice, Amicus shall provide Shire with reasonable access to other Data and Know-How Controlled by Amicus with respect to the Related Product or Back-Up Compound(s) contained in the applicable Related Product Opt-In Notice or Back-Up Compound Notice. However, notwithstanding anything herein to the contrary, unless such Related Product or Back-Up Compound becomes a Licensed Product or Compound in accordance with Section 6.3 or 6.4, Shire shall not have the right to use, and shall not use, such Know-How pertaining to such Related Product or Back-Up Compound provided to it pursuant to this Section 6.5.2 or otherwise to obtain Regulatory Approval of, or to Commercialize, any product.

6.5.3 Transition of Development Following Exercise of Opt-In Right. Following exercise of the Related Product Opt-In Right or the selection of a Back-Up Compound upon exercise of the Back-Up Compound Opt-In Right, Amicus shall continue performing further activities related to the Development of such Related Product or Back-Up Compound, as applicable, in accordance with Amicus' own Development plans for a period of up to one (1) year after the exercise of such Related Product Opt-In Right or selection, respectively, or until such earlier time as the JSC establishes such new Development Plan. Thereafter, the further Development of such Licensed Product shall be conducted in accordance with such Development Plan, as modified by the JSC from time to time. All costs reasonably incurred by Amicus in performing such activities (i.e., those after

Shire's exercise of the applicable Opt-In Right, but prior to the JSC's establishment of a new Development Plan), and those conducted pursuant to the new Development Plan so established, shall be shared in accordance with Section 7.4.1. Following exercise of the Related Product Opt-In Right or Back-Up Compound Opt-In Right, and adoption of the new Development Plan, the Parties shall use Commercially Reasonable Efforts to ensure an orderly transition and uninterrupted Development of the Related Product or Back-Up Compound that was the subject of such Opt-In Right, respectively.

6.5.4 Regarding Amount of Reimbursement. With respect to the calculation of costs to be reimbursed under Sections 6.3.3, 6.4.4(c)(ii) or 6.5.3, internal Development costs shall be determined by applying the FTE Rate used under the Development Plans for the same period. In the event of a dispute as to the costs to be reimbursed under such Sections, the matter shall be resolved by the JSC; and in such case, prompt reimbursement shall be made in the amount described above with respect to any undisputed amounts and any disputed amounts shall be promptly reimbursed upon resolution of such dispute by the JSC or pursuant to Section 16.8.

6.5.5 Term of Related Product and Back-Up Compound Opt-In Rights. Within thirty (30) days after the first Regulatory Approval of a Licensed Product in the first Primary Market of the Shire Territory, Section 6.3 and Section 6.4.4 shall each terminate with respect to all Related Products, and all Back-Up Compounds, directed to the same disease within the Field as such Licensed Product. For example, upon the first Regulatory Approval for Amigal, (a) Shire would have no further Related Product Opt-In Right with respect to any small molecule that selectively binds to the active site of a-galactosidase A for the diagnosis, treatment or prevention of Fabry Disease and (b) Shire would have no further Back-Up Compound Opt-In Right if Amigal (or any other Licensed Product being developed for Fabry Disease) thereafter becomes a Terminated Product.

6.6 Independent Development and Commercialization of Related Products.

6.6.1 Neither Party shall, directly or indirectly, Commercialize a Related Product within the Field in the Shire Territory until **[***]** after the First Commercial Sale in the first Primary Market country in the Shire Territory of the Licensed Product directed toward the same Target as such Related Product. In addition, unless the Parties agree otherwise, Shire shall not and, solely with respect to a Related Product for which Amicus has elected solely to Commercialize in the Amicus Territory under Section 6.3.6, Amicus shall not, directly or indirectly, Develop such a Related Product prior to such First Commercial Sale; provided that such restriction shall apply neither to non-clinical Development activities, nor to clinical trials in healthy volunteers, with respect to a Related Product.

6.6.2 For clarity, it is understood that Section 6.3 through 6.5 above shall not apply with respect to [***] for [***]'s or other products outside the Field.

6.7 Reservation. For clarity, is understood that, except as expressly provided in Section 4.3.2, this Agreement shall not be deemed to limit Amicus' right to Manufacture, Develop,

Commercialize and otherwise use, import or exploit (a) Compounds or Licensed Products for use within or outside the Field within the Amicus Territory, or (b) subject to Sections 6.2.1 and 6.2.2 above, an [***] for [***]'s for use in the Shire Territory.

ARTICLE 7
PAYMENTS, ROYALTIES AND THE SHARING OF DEVELOPMENT COSTS

7.1 License Fee. Shire shall pay to Amicus a license fee in the amount of Fifty Million Dollars ($50,000,000), within three (3) Business Days after the Effective Date, in accordance with Section 7.6. The license fee set forth in this Section 7.1 shall not be refundable or creditable against any future milestone payments, royalties or other payments by Shire to Amicus under this Agreement.

7.2 Milestone Payments

7.2.1 Development Milestone Payments. Shire shall pay to Amicus the Milestone payments set forth below following the first achievement by Amicus or Shire, or any of their Affiliates or Sublicensees, of the corresponding Milestone event set forth below with respect to a Licensed Product:

Milestone Event	Milestone Payment Amount
[***]	**$[***]**
[***]	**$[***]**
[***]	**$[***]**
[***]	**$[***]**
[***]	**$[***]**
[***]	**$[***]**
[***]	**$[***]**
[***]	**$[***]**
[***]	**$[***]**
[***]	**$[***]**

For purposes of clarity, the above payments for achievement of a Milestone shall only be paid once under this Section 7.2.1 irrespective of how many Licensed Products achieve such Milestones. For clarity, additional milestones may be due pursuant to Sections 6.2.1(c) and 6.3.5.

7.2.2 Net Sales Milestone Payments. In addition, Shire shall pay to Amicus the Milestone payments set forth below at such time as the aggregate annual Net Sales of all Licensed Products during a particular calendar year first reaches the following amounts in the Shire Territory:

Aggregate Annual Net Sales of All Licensed Products in the Shire Territory	Milestone Payment Amount
$[***]	$[***]
$[***]	$[***]
$[***]	$[***]
$[***]	$[***]

Notwithstanding anything herein, in no event shall any two Milestone payments under this Section 7.2.2 be due in the same calendar year; **[***]**. For purposes of clarity, the above payments for achievement of a Milestone under this Section 7.2.2 shall only be paid once under this Agreement.

7.2.3 Reports and Payments. Each Party shall notify the other Party of its achievement of any of the foregoing milestone events under Sections 7.2.1 and 7.2.2 (each, a "***Milestone***") and Amicus shall provide Shire an invoice of such Milestone achievement, and payment of the amount corresponding to such Milestone, except as expressly provided otherwise in Section 7.2.2, shall be due within fourteen (14) Business Days of receipt by Shire of an invoice of such achievement. For the avoidance of doubt, the Milestone payments set forth in this Section 7.2 shall not be creditable against any future Milestone payments, royalties or other payments by Shire to Amicus under this Agreement.

7.2.4 Certain Terms. For purposes of the milestone payments due under Section 7.2.1:

(a) **[***]**.

(b) **[***]**.

(c) **[***]**.

(d) **[***]**.

(e) **[***]**.

[***] INDICATES MATERIAL THAT HAS BEEN OMITTED AND FOR WHICH CONFIDENTIAL TREATMENT HAS BEEN REQUESTED. ALL SUCH OMITTED MATERIAL HAS BEEN FILED WITH THE SECURITIES AND EXCHANGE COMMISSION PURSUANT TO RULE 24b-2 PROMULGATED UNDER THE SECURITIES EXCHANGE ACT OF 1934, AS AMENDED.

(f) For purposes of this Article 7, a **[***]** shall be deemed to constitute a **[***]** if the JDC determines to proceed with filing an **[***]** based upon the use of Data from such **[***]** as a pivotal trial.

(g) A Milestone shall be deemed achieved for Fabry Disease, Gaucher Disease or Pompe Disease if the Milestone is met for an indication involving such disease.

(h) If, upon first achievement of a particular Milestone under Section 7.2.1 above with respect to a particular Licensed Product, the amounts corresponding to any Milestone with respect to such Licensed Product that precede such Milestone in the table under Section 7.2.1 above have not been previously paid, then such previous amounts that have not been paid shall also become due and payable subject to Section 7.2.2.

7.2.5 Reversion. Notwithstanding the foregoing, if at the time the first Regulatory Approval is obtained for a Licensed Product in the first Primary Market country in the Shire Territory where (a) neither the sale nor use of such Licensed Product is covered by a Valid Claim and (b) such Licensed Product has no regulatory or orphan drug exclusivity in such Primary Market country, Shire may within thirty (30) days of such Regulatory Approval elect to (i) terminate this Agreement in its entirety with respect to such Licensed Product, under Section 15.3.2, in which case the Milestone payment for Regulatory Approval under Section 7.2.1 shall not be due for such Licensed Product irrespective of Section 7.2, (ii) terminate this Agreement with respect to such Licensed Product for the European Union only by providing notice as such and further no Milestone payment for such Regulatory Approval shall be due at such time for such Licensed Product irrespective of Section 7.2 or (iii) pay the Milestone payment for Regulatory Approval and continues as otherwise provided in the Agreement. In the event Shire elects subsection (ii) above, upon Regulatory Approval in the (x) first Secondary Country, Shire shall pay **[***]**, (y) second Secondary Country, Shire shall pay **[***]** and (z) remaining Secondary Country, Shire shall pay **[***]**, in each case of the Milestone payment that would have been paid upon the Regulatory Approval of such Licensed Product had Shire not made its election to terminate this Agreement with respect to such Licensed Product for the European Union. "***Secondary Country***" shall mean any one of the following: Japan, Brazil or Israel. In the event Shire elects (ii), then the European Union shall thereafter be included in the Amicus Territory with respect to such Licensed Product; and Section 6.4.3 above shall apply to such Licensed Product with respect to the European Union (and in the event of any dispute as to how Section 6.4.3 would apply in such case, the matter shall be determined by the JSC).

7.3 Royalties. Subject to this Section 7.3, Shire shall pay to Amicus royalties at the following rates on the Annual Net Sales (as defined below) by Shire, its Affiliates and its Sublicensees of each Licensed Product:

Royalty Tier	Annual Net Sales of Each Licensed Product	Royalty Rate
1	Portion up to and including $**[***]**	**[***]**%
2	Portion above $[***] and up to and including $**[***]**	**[***]**%
3	Portion above $**[***]** and up to and including $**[***]**	**[***]**%
4	Portion above $**[***]**	**[***]**%

7.3.1 Calculation. For such purposes, "***Annual Net Sales***" means total Net Sales of a particular Licensed Product in a particular calendar year for which royalties are due pursuant to this Agreement. For purposes of this Agreement, units of Licensed Products shall be considered sold, and the corresponding Net Sales shall be deemed to have accrued, when revenue is recognized in accordance with U.S. GAAP.

7.3.2 Generic Competition Reduction. Notwithstanding the foregoing, if there is Generic Competition in a Calendar Quarter in a country within the Shire Territory with respect to a Licensed Product, the royalties due on sales of Licensed Product for such Calendar Quarter in such country shall be reduced as follows:

Percentage of Generic Competition in a Country	Royalty Reduction on Net Sales from such Country
>**[***]**% <**[***]**%	**[***]**%
= **[***]**% <**[***]**%	**[***]**%
= **[***]**%	**[***]**%

"***Generic Competition***," with respect to a Licensed Product in a country in the Shire Territory, shall exist after one or more Generic Versions of such Licensed Product are being marketed in such country. The percentage of Generic Competition in a country shall be calculated by dividing the aggregate unit volume of Generic Version(s) of such Licensed Product by the total prescription unit volume of such Licensed Product and such Generic Version(s) combined, in the aggregate, in such country in a calendar quarter (as measured by a Scott-Levin Associates audit or other mechanism determined by the JSC); and "***Generic Version***" shall mean a product that: (x) (1) with respect to the European Union or Japan **[***]** and (2) with respect to all other countries in the Shire Territory has the same active ingredient as the Compound in the Licensed Product and is legally substituted by pharmacies in such country for the Licensed Product **[***]**.

The adjustment under this Section 7.3.2 shall be reconciled at the end of each calendar year to take into account the total volume for the year of the particular Generic Version and such Licensed Product and the royalty rate applicable for such calendar year under this Section 7.3.2 as

determined by the JSC (such that total adjustment for the year shall be calculated on annual basis for such calendar year).

7.3.3 Gross Margin Adjustment.

(a) In the event that Shire's average Gross Margin (as defined below) for a particular Licensed Product during any calendar quarter is less than **[***]%**, the difference between such average Gross Margin and **[***]%** shall be offset against the royalties otherwise payable with respect to such Licensed Product during such calendar quarter pursuant to Section 7.3 above, provided, however, that in no event shall: (a) the total royalty rate under Section 7.3 be reduced by more than **[***]%**, or (b) the royalty rate under Royalty Tier 1 be reduced to less than **[***]%**; and in any event there shall be no such reduction, under this Section 7.3.3, of the royalty rates under Royalty Tiers 3 and 4.

(b) For purposes hereof:

(i) "***Gross Margin***" is defined as the Net Sales of such Licensed Product less the Manufacturing Cost (as defined below) of such Licensed Product for the units of Licensed Product whose sale generated the Net Sales for the period in question, provided, however, that for purposes of calculating Shire's Gross Margin under this Section 7.3.3, Shire's Manufacturing Cost shall be deemed not to exceed Amicus' Manufacturing Cost for the same Licensed Product during the same period; and

(ii) "***Manufacturing Cost***" is defined as, with respect to a Licensed Product sold by a Party: (A)(1) the amount paid by such Party for such Licensed Product manufactured by a Third Party or (2) the cost of direct materials and direct labor for such Licensed Product manufactured by such Party or its Affiliate, plus a reasonable allocation of direct manufacturing overhead, not to exceed **[***]** of such materials and labor costs; (B) the net cost or credit to such Party of any value-added taxes or duties actually paid or utilized on account of such materials; (C) out-of-pocket transportation costs, including clearance and storage of such materials (if necessary), and transit insurance on account of such materials; (D) the costs of reasonable quantities of material destroyed in quality control testing on account of such Licensed Product (as such costs are calculated under clauses (A) through (C) above); and (E) in the case of a Licensed Product manufactured by a Third Party, the internal costs of such Party pertaining to the procurement of such materials (e.g., for quality assurance and quality control activities), not to exceed **[***]** of the Manufacturing Cost of such Licensed Product.

7.3.4 Third Party Royalties. If Shire is required (a) to enter into an agreement to license or acquire rights under Third Party Patent Rights that are necessary to use, import, Manufacture or Commercialize (i) a Compound contained in a Licensed Product or (ii) the Licensed Products as existing as of the Effective Date and (b) pursuant to such agreement, to pay to a Third Party royalties or other payments in order to use, import, Manufacture or Commercialize a Licensed Product within the Field in a country of the Shire Territory, then Shire may deduct up to **[***]** of the commercially reasonable royalty or other payments payable to such Third Party from the royalty

thereafter payable to Amicus under Section 7.3 above with respect to such Licensed Product in such country, provided that the royalty paid to Amicus shall not be **[***]** of what Amicus would have received otherwise pursuant to the terms of this Agreement; provided, further, that this Section 7.3.4 shall not apply to Patent Rights listed on Schedule 7.3.4. In the event of a dispute between the Parties whether such Third Party license is required, such dispute shall be resolved by the JSC based upon the well reasoned opinion of qualified independent patent counsel retained by the JSC.

7.3.5 Reports and Royalty Payment. Within ten (10) Business Days after the end of each calendar quarter, Shire shall deliver to Amicus a report setting out all details necessary to calculate the royalty payments due under this Section 7.3 with respect to Net Sales made in that calendar quarter, including:

(a) units of Licensed Products sold in the Shire Territory during the relevant calendar quarter **[***]**;

(b) gross sales of Licensed Products in the Shire Territory in the relevant calendar quarter **[***]**, including the gross sales price of each Licensed Product;

(c) Net Sales of Licensed Products in the relevant calendar quarter on a country-by-country basis;

(d) all relevant deductions in accordance with Section 1.25 and this Section 7.3;

(e) all relevant exchange rate conversions; and

(f) such other information as requested by Amicus regarding the Commercialization of Licensed Products in the Shire Territory as necessary to satisfy Amicus' reporting obligations to its licensors under the Existing In-Licenses.

Any amounts due under this Section 7.3 for such calendar quarter shall be paid within thirty (30) days after the end of such calendar quarter.

7.3.6 Royalty Term. The obligation of Shire to pay royalties pursuant to this Section 7.3 shall continue for each Licensed Product, on a Licensed Product-by-Licensed Product and country-by-country basis, until the latest of:

(a) Expiration of the last to expire Valid Claim covering the use, importation, or sale of such Licensed Product in the country of sale;

(b) The loss of all regulatory exclusivity (including orphan drug exclusivity) to market such Licensed Product in such country; or

(c) Fifteen (15) years after the First Commercial Sale of such Licensed Product in such country.

7.4 Development Cost Sharing7.4.1 Development Costs. Subject to the terms and conditions of this Agreement, each Party shall be responsible for fifty percent (50%) of all Development Costs incurred in accordance with the Development Plans.

7.4.2 Cost Overruns. With respect to Development Costs to be shared by the Parties under Section 7.4.1 above, if the total Development Costs incurred by a Party (such party, the "***Spending Party***") in a calendar year exceed the costs budgeted for such Party in the applicable then current Development Plan, then the other Party shall continue to bear its share of such costs under Section 7.4.1 above, notwithstanding such overrun, up to its share of **[***]** of the budgeted amounts (the "***Permitted Overrun***"), but such other Party shall not be responsible for its share of such aggregate costs in excess of the Permitted Overrun (the "***Excess Costs***"), without the approval of the JSC, which shall not be unreasonably withheld.

7.4.3 Commercialization Costs. For clarity, it is understood that each Party shall be responsible for all Commercialization costs incurred by such Party.

7.4.4 Procedure for Sharing Development Costs.

(a) Each Party shall calculate and maintain records of all Development Costs incurred by it in accordance with Section 7.8.

(b) So that the Parties will share equally the Development Costs incurred in accordance with the Development Plans pursuant to Section 7.4.1 above, balancing payments shall be made as follows: On or before the fifteenth day of each month, the Party who is budgeted to incur the lesser amount of Development Costs during such month (as reflected in the then current Development Plans) (the "***Reimbursing Party***") shall pay to the other Party (the "***Receiving Party***") an amount equal to (i) fifty percent (50%) of the Development Costs budgeted to be incurred by the Receiving Party during such month, less (ii) fifty percent (50%) of the Development Costs to be incurred by the Reimbursing Party during such month, all in accordance with the Development Plans then in effect. Unless otherwise specified in the applicable Development Plan, amounts budgeted thereunder for a period exceeding the length of a month will be deemed budgeted in equal amounts for each month within such period. Within ten (10) days following the last day of each month during any period in which activities are being performed under a Development Plan hereunder, each Party shall provide to the other a good faith estimate of the Development Costs actually incurred by such Party during the month then ended, in a form determined by the JDC.

(c) The Parties shall use good faith efforts to keep each other apprised of variances in Development budgets and to notify each other with respect thereto.

(d) Within thirty (30) days following the last day of each calendar quarter (March 31, June 30, September 30, and December 31) during the Term, the Parties shall reconcile the advanced payments made in accordance with paragraph (b) above with the Development Costs actually incurred to date by each Party, and within such thirty (30) day period the Parties shall make such balancing payment(s) as are necessary to achieve the result that each Party has funded fifty

percent (50%) of the total Development Costs through such March 31, June 30, September 30 or December 31 date, as applicable.

(e) Any dispute regarding Development Costs under this Section 7.4 shall be resolved by the JDC. The existence of any dispute regarding Development Costs or any other matter under this Section 7.4 shall not relieve a Party of its obligation to continue to pay prospectively each month its allocated portion of Development Costs in accordance with this Section 7.4 and the Development Plans. In the event of any such dispute, any undisputed amount shall be paid within the time periods specified in this Section 7.4, and the Party disputing any amount shall pay to the other Party within such time periods **[***]** of the disputed amount.

7.4.5 Funding Under Initial Development Plans. It is understood that, beginning upon the Effective Date, the Parties will fund in accordance with this Section 7.4 the Development Costs incurred by Amicus under the initial Development Plans attached as Appendix 4. The Parties acknowledge, however, that the number of FTEs reflected in such initial Development Plans has not been agreed as of the Effective Date. Accordingly, if the final Development Plans established in accordance with Section 4.2.5(b) provide for fewer FTEs than those reflected in such initial Development Plans (or if the Parties sooner agree on a lower number of FTEs), then Shire shall receive a credit for its portion of the costs of such excess FTEs reimbursed by Shire under this Section 7.4, in the amount of one half of the FTE Rate multiplied by the number of such excess FTEs, prorated for the period of time from the Effective Date until the date such final Development Plans are so approved. Such credit shall be applied in equal monthly amounts over the first six months following the approval of such final Development Plans.

7.5 Payments under Existing In-Licenses.

7.5.1 Generally. Other than as specifically provided in Sections 7.5.2, 7.5.3. 10.2.1 and 10.2.2 below, Shire shall have no responsibility for Third Party payments due with respect to the Development, Manufacture or Commercialization of the Licensed Products under the Existing In-Licenses, and such payments shall be borne solely by Amicus.

7.5.2 Royalty Reductions. Amicus shall be responsible for paying any royalties owed to Third Parties under Amicus' Existing In-Licenses. However, during the royalty term (as determined under Section 7.3.6) where a reduction of royalties due to Amicus under Section 7.3 with respect to a Licensed Product in a country of the Shire Territory occurs under Sections 7.3.2, 7.3.3, 7.3.4, 7.3.6 or 15.5.4 of this Agreement **[***]** with respect to sales of such Licensed Product in such country, Shire shall promptly reimburse Amicus **[***]**. If, with respect to a Licensed Product, upon the **[***]** Amicus continues to be obligated to make royalty payments to a Third Party under an Existing In-License with respect to sales of such Licensed Product in such country, then (x) Amicus shall promptly notify Shire of the amount of such royalty rate for sales of such Licensed Product and the period for which it is obligated to make such payments in such country and (y) within thirty (30) days after receipt of such notice Shire shall notify Amicus of its election to (1) terminate its sublicense under such Existing In-License in which case such sublicense will terminate whereby no further royalties shall be due from Shire for sales of such Licensed Product in such country or (2)

make royalty payments to Amicus for the amounts owed by Amicus under such Existing In-License for the period so specified in the notice provided under subsection (y) above and at the lower of the royalty rate so specified in such notice and the applicable rate specified in subsection (b) above.

7.5.3 Sublicenses. In the event that the granting of a Sublicense under the rights granted to Shire under Section 2.1 triggers any payment obligation by Amicus under any Existing In-License (other than the payment of running royalties), Shire shall be responsible for, and shall promptly pay to Amicus upon request, any such amounts when they become due.

7.6 Other Payment Terms.

7.6.1 Payment Method. All payments between the Parties under this Agreement (including, without limitation, the payments due under this Article 7) shall be made by bank wire transfer in immediately available funds to an account designated by the Party to which such payments are due. All payments due under this Agreement which are not timely paid shall bear interest to the extent permitted by Law at a rate equal to the U.S Prime Lending Rate, as quoted in *The Wall Street Journal* (U.S. Eastern Edition), effective for the date on which the payment was due. This Section 7.6.1 shall in no way limit any other remedies available to the Parties.

7.6.2 Currency. All dollar amounts in this Agreement are stated in, and all payments under this Agreement shall be made in, United States Dollars. With respect to amounts invoiced or incurred in a currency other than United States Dollars, (i) for calculation of Net Sales, the amount in foreign currencies shall be converted into United States Dollars using the average rate of exchange for such currencies for the relevant month as sourced from www.oanda.com, and (ii) for calculation of all other sums due under this Agreement, the amount in foreign currencies shall be converted into United States Dollars using the exchange rate for such currencies for the date of the respective invoice and where such exchange rate shall be the mid-price exchange rate taken from Bloomberg as published on the date of the relevant invoice or such other publication as may be mutually agreed between the Parties.

7.7 Taxes

7.7.1 Notice. Each Party will provide the other Party with reasonable advance notice of tax withholding obligations to which it reasonably believes that it is subject. Any withholding or other taxes that either Party is required by law to withhold or pay on behalf of the other Party, with respect to any payments to such other Party hereunder or any of the Related Agreements, shall be deducted from such payments and paid to the appropriate tax authority contemporaneously with the remittance to the other Party, provided, however, that the withholding Party shall furnish the other Party with proper evidence of the taxes so paid. Each Party shall cooperate with the other and furnish the other Party with appropriate documents to secure application of the most favorable rate of withholding tax under Law (or exemption from such withholding tax payments, as applicable), and, at the request of the payee Party, payment of any amount subject to withholding shall be deferred until the appropriate documents have been furnished.

7.7.2 Certain Taxes.

(a) Notwithstanding Section 7.7.1 above, subject to compliance with Section 7.7.2(b) and (c) below any payments to Amicus hereunder shall be made free and clear of, and Shire shall indemnify and hold Amicus harmless against, any Irish tax (whether withheld at the source or otherwise) imposed on or in relation to any payment made under this Agreement that would not have arisen had the paying entity been a corporation formed under the laws of the United States; provided, however, that Shire shall not be required to indemnify Amicus for Irish taxes imposed on the net income of Amicus by reason of Amicus (or its successors under Section 16.1 below) having (or having attributable to it) a permanent establishment, branch, agency or Irish tax resident corporation in Ireland, other than a permanent establishment, branch, agency or Irish tax resident corporation created as a result of Amicus entering into this Agreement or undertaking any action required under this Agreement.

(b) Amicus agrees to use diligent efforts to obtain, as promptly as practicable after the Effective Date, a completed US tax Form 6166 from the IRS and shall forward it with a completed Irish tax Form IC3/6166 to Shire requesting that the Irish Revenue Commissioners issue a direction to Shire to make all payments under this Agreement without withholding or deduction for or on account of Irish tax. In addition, Amicus agrees to use diligent efforts, upon request and at the expense of Shire, to recover from the Irish Revenue Commission any refundable tax, and/or to challenge an assessment of any other Irish tax, indemnified by Shire under Section 7.7.2(a) above.

(c) Amicus warrants that as of the Effective Date it is, and the indemnity and agreement not to withhold under Section 7.7.1 above is conditioned upon Amicus (and its successors under Section 16.1) being on the date of any payment under this Agreement, a resident in the United States for the purposes of the Convention between the Government of Ireland and Government of the United States of America for the avoidance of double taxation and the prevention of fiscal evasion with respect to taxes on income and capital gains signed 28th day of July, 1997.

7.8 Records Retention; Audits.

7.8.1 Record Retention. Each Party will maintain complete and accurate books, records and accounts used for the determination of expenses incurred in connection with the performance of Development of Licensed Products within the Field (and of [***] for [***]'s) or otherwise relevant for the calculation of Net Sales, in sufficient detail to confirm the accuracy of any payments required under this Agreement, which books, records and accounts will be retained by such Party for five (5) years after the end of the period to which such books, records and accounts pertain, or longer as is required by Law.

7.8.2 Request. Upon the written request of a Party (the "***Auditing Party***") and not more than once each calendar year, the other Party (the "***Responding Party***") shall permit the Auditing Party, accompanied by an independent certified public accounting firm of internationally recognized standing, selected by the Auditing Party and reasonably acceptable to the Responding Party, to have access during normal business hours to the records of the Responding Party as may be

reasonably necessary to verify the accuracy of the financial reports and calculations made under this Article 7 for any quarter ending not more than five (5) years prior to the date of such request.

7.8.3 Discrepancies. If, as a result of such audit, it is established that additional amounts were owed by the Responding Party for the audited period, such Party shall pay such additional amounts within thirty (30) days after the date such discrepancy is established. The determination by such public accounting firm shall be definitive and final. The fees charged by such accounting firm shall be paid by the Auditing Party; provided, however, that if the audit establishes that the aggregate amounts payable by the Responding Party for the period covered by the audit are more than one hundred five percent (105%) of the aggregate amounts actually paid for such period, then the Responding Party shall pay the reasonable fees and expenses charged by such accounting firm. The Auditing Party shall treat all financial information subject to review under this Section 7.8 as confidential, and shall cause its accounting firm to retain all such financial information in confidence.

ARTICLE 8
MANUFACTURING AND SUPPLY

8.1 General. It is understood that Amicus procures supplies of Licensed Products from Third Party contractors. Accordingly, subject to the terms and conditions of this Agreement, Amicus shall cooperate with Shire to obtain from such Third Party contractors quantities of Licensed Products and Compounds (collectively, the "***Materials***") to supply Shire's reasonable requirements for Materials for the Shire Territory. In furtherance of the preceding sentence:

8.1.1 Procedures for Clinical Supply. The JDC shall establish as soon as practicable following the Effective Date procedures for the supply of Licensed Products to Shire for use in performing Shire's Development activities under the Development Plan or Independent Projects.

8.1.2 Interim Procedures. Pursuant to such procedures:

(a) Amicus shall procure Materials on behalf of and as reasonably requested in writing by Shire, consistent with Amicus' arrangements with its suppliers; and

(b) Materials ordered by Amicus from such supplier on behalf of Shire shall be charged to the Development Costs, or Shire (in the case of an Independent Project) in an amount equal to the amount paid by Amicus for such Materials, plus (i) any other documented out-of-pocket costs incurred by Amicus directly in connection with procuring such Materials, and (ii) any internal costs of Amicus pertaining to the procurement of such Materials (e.g., for quality assurance and quality control activities), provided that such internal costs do not exceed **[***]** of the overall cost of such Materials.

8.1.3 Form. The Materials supplied to Shire pursuant to Section 8.1 above shall be supplied in the same form as like Materials are supplied to Amicus, or as otherwise established by

the JDC or by agreement of the Parties, it being understood that any differences in such form requested by Shire shall be at Shire's expense.

8.1.4 Shortage of Supply. The procedures and arrangements to be established by the JDC under Section 8.1.1 above shall include reasonable mechanisms to avoid shortage of supply, including procedures for buffer stock inventories to be maintained by the Parties on a proportionate basis, and/or establishing second source manufacturers or manufacturing sites. If, prior to the establishment and implementation of such procedures, Shire's requirements of Materials cannot be fulfilled in accordance with this Article 8, the total available quantities of Licensed Products shall be allocated among the Parties on a reasonable worldwide basis (based upon the needs of the Development Plans and any Independent Projects, and if such Licensed Product is then being Commercialized, sales history and reasonably forecasted demand for such Licensed Product), as determined by the JSC.

8.2 Supply Agreement. Upon Shire's written request, the Parties shall use good faith efforts to enter into a commercial supply agreement (a "***Supply Agreement***") within six (6) months of such request consistent with this Article 8 on commercially reasonable terms documenting the arrangement by which Amicus shall supply Shire's reasonable requirements for Materials at cost for the Shire Territory, which Supply Agreement shall contain forecasting and ordering procedures (including lead times), product specifications, delivery terms and other appropriate provisions and be consistent with Amicus' contractual arrangements with its Third Party suppliers.

8.3 Limitation; Manufacturing by Shire. Amicus shall (a) cooperate fully with Shire to make available for the benefit of Shire the benefits of Amicus' supply agreements and/or arrangements with its Third Party suppliers of Materials and (b) administer such agreements or arrangements diligently and pursue its rights and remedies thereunder. It is understood, Amicus shall not be liable for a default or a failure of supply due to default of the Third Party suppliers. Notwithstanding anything in this Agreement, after the first Phase II Clinical Trial for a Licensed Product, Shire shall have the right to Manufacture, or engage a Third Party to Manufacture, Shire's requirements of Materials for the Shire Territory. Promptly following Shire's request, Amicus shall transfer, or cause to be transferred, to Shire or such Third Party manufacturer all Amicus Know-How that is necessary, useful or actually used for such Manufacture of Materials (and the cost of such transfer of Amicus Know-How shall be shared equally by the Parties), and shall make personnel of Amicus reasonably available to assist Shire and/or its contractor in implementing the Amicus Know-How necessary to Manufacture such Materials. In any event the Parties shall cooperate to use the same process in Manufacturing Materials for use in Development.

ARTICLE 9
REGULATORY MATTERS

9.1 Regulatory Responsibilities. Unless otherwise agreed between the Parties:

9.1.1 Amicus Territory Regulatory Responsibility. As between the Parties, Amicus shall be responsible for filing, obtaining and maintaining, in its own name, Regulatory Filings and

Regulatory Approvals for Development and Commercialization of Licensed Products in the Amicus Territory except as otherwise provided in the Development Plan.

9.1.2 Shire Territory Regulatory Responsibility. As between the Parties, Shire shall be responsible for filing, obtaining and maintaining, in its own name, Regulatory Filings and Regulatory Approvals for Development and Commercialization of Licensed Products in the Field in the Shire Territory except as otherwise provided in the Development Plan.

9.2 Filings and Meetings with Regulatory Authorities.

9.2.1 Regulatory Filings and Correspondence. The Party with responsibility for regulatory matters in a Primary Market country shall provide the other Party's representatives on the JDC with copies of all Regulatory Filings and all minutes of any meetings, telephone conferences and/or discussions with the Regulatory Authority of such Primary Market country, and shall promptly notify the other Party's representatives on the JDC with respect to any material changes or material matters that may arise in connection with Regulatory Approvals of Licensed Products within such Primary Market country, in each case to the extent it has the right to do so. Each Party will provide the other Party with translations of such documents into English to the extent prepared or obtained for its own use and requested by the other Party. Notwithstanding the foregoing, during the Term, Amicus shall not have the right to use for purposes of obtaining Regulatory Approval of Amigal for Fabry Disease in the Shire Territory any Regulatory Filing generated under the applicable Development Plan by Shire, for so long as Amigal remains a Licensed Product hereunder.

9.2.2 Meetings. The Party with primary responsibility for regulatory matters in a Primary Market within the Shire Territory shall, to the extent reasonably practicable: (a) promptly provide the JDC with reasonable advance written notice of any material Regulatory Filings, meetings, telephone conferences and/or other discussions with the Regulatory Authority of such country (including in all cases the EMEA), scheduled or unscheduled, that pertain to Licensed Products in the Field, (b) afford representatives of such other Party an opportunity to comment on such Regulatory Filings, and accept such comments or notify such other Party of the reason for not accepting any such comments, and (c) afford representatives of such other Party an opportunity to attend all such meetings, telephone conferences and/or discussions with the Regulatory Authority of such country solely in an observatory capacity. For clarity, Shire shall be the Party with primary responsibility for regulatory matters in the Shire Territory unless otherwise provided in the Development Plan.

9.3 Adverse Events and Post-Market Surveillance. With respect to adverse drug experiences, as defined by 21 C.F.R. Section 314.80 and/or 600.80 (as applicable), and IND safety reports, as referenced in 21 C.F.R. Section 312.32, and like regulations of other Regulatory Authorities, the Party with regulatory responsibility for a country shall be responsible for and shall establish operating procedures to report to the appropriate Regulatory Authority in that country all adverse drug experiences in accordance with the Laws of the relevant countries and agencies. The Parties agree to implement, as soon as reasonably practicable, a separate agreement setting forth the

pharmacovigilance responsibilities and procedures for safety information exchange (the "***Pharmacovigilance Agreement***"). The Pharmacovigilance Agreement shall contain such terms as are reasonable and customary for arrangements of this type, and shall in all events include such terms as are necessary to ensure that both Parties are able to comply with applicable Laws pertaining to adverse events and safety reporting and provide that there shall be one global safety database maintained by Amicus or its designee and accessible to both Parties. The JDC shall determine standard operating procedures by which the Parties shall have access to such global safety database.

9.4 Common Registration Dossier. A primary objective of each Development Plan is to Develop and produce a common registration dossier to serve as a basis for license applications to be filed in each of the Primary Market countries. Unless otherwise determined by the JDC, Amicus shall be responsible for preparing such common registration dossier, and the costs incurred in connection therewith shall be deemed to be Development Costs for purposes of this Agreement.

9.5 Regulatory Inspections. If either Party or its Affiliates or contractors (an "***Inspected Party***") are to be inspected by a Regulatory Authority regarding the Development or Manufacture of a Licensed Product, in each case within the Field, the Inspected Party shall promptly notify the JDC of the inspection in advance. The Inspected Party shall, where practicable, permit representatives of each of Shire and Amicus to participate as observers with respect to such inspection, and shall provide the JDC with a written report of any such inspection, noting with specificity any records or documents reviewed by the regulatory inspector, and including copies of any FDA 483s (or their foreign equivalent) or written communications provided by any Regulatory Authority relating to such inspection. The Inspected Party shall also provide an opportunity for the JDC to assist in responding to any issues or concerns relating to such inspections, and shall provide copies of all communications to and from any Regulatory Authority relating thereto to the JDC. The Parties shall cooperate in good faith and otherwise mutually support any such inspections by the FDA or other Regulatory Authority of facilities, clinical investigators or contract manufacturers. In furtherance of the preceding sentence, if the Inspected Party receives a request by a Regulatory Authority to inspect any facilities of the other Party, such other Party shall cooperate with and makes its facilities available for such inspection.

9.6 Audit Rights. Each Party shall have the right, during normal business hours, and no more than once per year, with more frequent audits upon agreement of the Parties, to inspect and audit: (a) those portions of the facilities of each Party, Affiliate, Sublicensee, subcontractor and investigator site used in the performance of the applicable Development Plan or the Manufacturing of Materials to be supplied hereunder, to ascertain compliance with Laws and Regulatory Approvals, including cGLP, cGCP and cGMP, and conformance with the applicable specifications and quality assurance standards, provided that the inspecting Party shall on such occasions be accompanied by a representative of the other Party; and (b) any of the other Party's documentation or its Affiliates', Sublicensees', subcontractors' or investigators' documentation relating to such Development Plan or Manufacturing of the Materials to be supplied hereunder, including, to the extent permitted by Law and any privacy policies, the medical records of any patient participating in any clinical study under the Development Plan. A Party's audit rights shall be limited by pre-existing bona fide Third Party

agreements or confidentiality obligations, provided, however, that each Party shall use its reasonable efforts to (1) obtain audit rights for the other Party under such pre-existing agreements and (2) ensure such other Party is granted audit rights to the same extent which a Party has audit rights in any future agreements.

ARTICLE 10
INTELLECTUAL PROPERTY

10.1 Ownership.

10.1.1 Clinical Data. To the extent a Party has the right to do so, each Party shall assign to the other Party a joint ownership interest in all Data with respect to Licensed Products generated in the course of performing clinical trials conducted under the Development Plans, excluding any inventions and Patent Rights therein (which are addressed in Section 10.1.2 below). For clarity, it is understood that the foregoing applies only to clinical data, and does not apply, for example, to related Regulatory Filings.

10.1.2 Inventions; Patent Rights.

(a) Subject to Section 10.1.2(b) below, title to all inventions, and all Patent Rights therein, made solely by Shire personnel in connection with this Agreement shall be owned by Shire ("***Shire Invention***"); title to all inventions, and all Patent Rights therein, made solely by Amicus personnel in connection with this Agreement shall be owned by Amicus ("***Amicus Invention***"); and title to all inventions, and all Patent Rights therein, made jointly by personnel of Amicus and Shire in connection with this Agreement shall be jointly owned by Amicus and Shire ("***Joint Inventions***"). For such purposes, a Party's personnel shall include personnel of such Party's Affiliates who are engaged in the Development or Commercialization of Licensed Products (to the extent such personnel are carrying out activities in connection with this Agreement).

(b) Notwithstanding paragraph (a) above, Shire hereby each grants and agrees to grant, to Amicus an exclusive license, with the right to sublicense, under Shire Inventions, and Shire's interest in Joint Inventions, that are made in connection with the Development or use of Compounds or Licensed Products, in each case that are improvements to the chaperone composition technology covered by the Amicus Patent Rights, or the manufacture, testing or use thereof, or that constitute a modification of a Compound, to make, use, sell, offer for sale and import and otherwise exploit such inventions subject to the inclusion of such inventions in the licenses granted in Article 2.

(c) In addition, Shire hereby grants to Amicus a non-exclusive license, with right to sublicense, to practice and otherwise exploit Patent Rights in Shire Inventions made by Shire in connection with the Development or use of a Compound or Licensed Product.

10.1.3 Joint Ownership.

(a) Except as expressly provided in this Agreement (including Section 10.1.3(b) below), it is understood that neither Party shall have an obligation to obtain approval of, nor pay a share of the proceeds to, the other Party to practice, enforce, license, assign or otherwise exploit Joint Inventions and Data jointly owned pursuant to Section 10.1 above, and each Party hereby waives any right it may have under the applicable Laws of any jurisdiction to require such approval or accounting. The Parties shall reasonably cooperate with each other and take any actions reasonably necessary to effect the purposes of this Section 10.1.3.

(b) Notwithstanding the foregoing, neither Party shall, without the consent of the other Party (which consent shall not be unreasonably withheld), grant to a Third Party a license under Patent Rights in a Joint Invention, other than (i) in connection with a collaboration with such Third Party, (ii) for use with products developed in whole or in part by such Party, or (iii) together with other technology or intellectual property Controlled by such Party.

10.2 Patent Filing, Prosecution, and Maintenance10.2.1 By Shire. Shire shall have the right to control the filing for, prosecution and maintenance of the Amicus Patent Rights in the name of Amicus (or Amicus' Licensor), excluding the Ex-U.S. Platform Patent Rights, solely with respect to the Licensed Products in the Field in all jurisdictions outside the United States using counsel of its choice, and Amicus shall reimburse Shire for **[***]** of the out-of-pocket expenses reasonably incurred by Shire in performing such activities.

10.2.2 By Amicus. Amicus shall have the right to control the filing for, prosecution and maintenance of: (a) the Amicus Patent Rights in the United States and (b) the Ex-U.S. Platform Patent Rights in all jurisdictions outside the United States, using counsel of its choice, and Shire shall have the option to either reimburse Amicus for fifty percent (50%) of the out-of-pocket expenses reasonably incurred by Amicus in performing the activities under (b) above or, within sixty days of a request for such reimbursement, opt to have such Patent Rights excluded from the licenses granted herein (in which case such Patent Rights shall be excluded from Amicus Patent Rights hereunder and Section 7.5.2 shall not apply for amounts due by Amicus to a Third Party under such Existing In-License where all Amicus Patent Rights under such Existing In-License have been excluded from the licenses granted herein and no such Patent Rights cover Manufacturing of Materials by Amicus for Shire).

10.2.3 Jointly Owned Patent Rights. The filing for, prosecution and maintenance of Patent Rights covering Joint Inventions (the *"**Joint Patent Rights**"*) shall be as determined by the Parties, and the out-of-pocket costs thereof shall be shared equally by the Parties.

10.2.4 Right to Consult and Advise. During the Term, each Party filing for, prosecuting and/or maintaining Patent Rights pursuant to this Section 10.2 (the "***Prosecuting Party***") shall copy the other Party, or have the other Party copied, on all material or substantive documents regarding such Patent Rights, in each case that are directly related to Licensed Products in the Field or cover Joint Patent Rights, which are received from or to be filed in any patent office in the Territory, promptly following receipt from the patent office and within a reasonable time prior to filing with the patent office (but not less than thirty (30) days), as applicable, including copies of

each patent application, office action, response to office action, declaration, information disclosure statement, request for terminal disclaimer, request for patent term extension and request for reexamination. Consistent with the foregoing, such other Party shall have the right to comment on the prosecution of such Patent Rights, in each case that are directly related to Licensed Products in the Field or are Joint Patent Rights, by the Prosecuting Party and provide such comments to the Prosecuting Party's patent counsel, and the Prosecuting Party shall consider all such comments in good faith. If such other Party fails to provide its comments with respect to the prosecution by the Prosecuting Party of such patent application or patent reasonably in advance of the deadline for filing or otherwise responding to the relevant matter in the relevant patent office (but not less than five (5) Business Days), the Prosecuting Party shall be free to act without consideration of such other Party's comments, provided that the Prosecuting Party has provided the other Party with the relevant information not less than ten (10) Business Days prior to such deadline or response.

10.2.5 Abandonment of Prosecution. The Prosecuting Party will notify the other Party in the event it desires to abandon its efforts with respect to the prosecution and maintenance of any Patent Rights being prosecuted by the Prosecuting Party under this Section 10.2, to the extent such Patent Rights pertain to the Licensed Products in the Field in the Shire Territory or the Joint Patent Rights. Notification will be given within a reasonable period (i.e., with sufficient time for such other Party to take whatever action may be necessary) prior to the date on which such Patent Rights will lapse, go abandoned (other than to file continuation application for the same subject matter) or otherwise diminish (but not less than sixty (60) days). Such other Party (the "***Acting Party***") will then have the right, exercisable upon written notification to the Prosecuting Party, to assume full responsibility, at its discretion and its sole cost and expense, to file, prosecute, maintain or conduct any interferences, re-examinations, reissues and oppositions in the Shire Territory, or in the case of Joint Patent Rights, any country or countries; provided that, in the case of Shire being the Acting Party with respect to Patent Rights (other than Joint Patent Rights) Controlled by Amicus, such right shall be limited to the extent such Patent Rights pertain to a Licensed Product in the Field in the Shire Territory.

10.2.6 Scope of Activities. For the purposes of this Section 10.2, "***prosecution and maintenance***" shall mean, with respect to a patent, the preparing, filing, prosecuting and maintenance of such patent, as well as re-examinations, reissues and requests for patent term extensions and the like with respect to such patent, together with the conduct of interferences, the defense of oppositions and other similar proceedings and appeals thereof with respect to a patent, but shall not include enforcement litigation or the defense of declaratory judgment actions. Also, as used in this Section 10.2, to "abandon" particular Patent Rights shall include deciding not to defend against an opposition, not to defend an interference or similar proceeding, not to pursue an appeal of an adverse decision or not to pursue particular claims, in each case with respect to such Patent Rights in the United States Patent & Trademark Office or a corresponding patent examining authority in another country.

10.3 Enforcement Against Third Parties.

10.3.1 Notice. If either Party reasonably believes that a Third Party is conducting any activities in the Shire Territory that may constitute actual or potential infringement of the Amicus Patent Rights, in each case with respect to a Generic Version of a Licensed Product or a Related Product in the Field in the Shire Territory (each, an "***Alleged Infringement***"), such Party shall promptly notify the other Party of such activities.

10.3.2 Shire's First Right to Enforce. Except as otherwise agreed, Shire shall have the first right to bring and control any action or proceeding under such Patent Rights in respect to an Alleged Infringement occurring in the Shire Territory. If Shire fails to bring an action or proceeding with respect to an Alleged Infringement occurring in the Shire Territory within one hundred twenty (120) days following a request by Amicus to do so, Amicus shall have the right to bring and control any such action or proceeding with respect to Amicus Patent Rights.

10.3.3 Cooperation. The Parties shall reasonably cooperate with each other in all actions or proceedings described in this Section 10.3, to the extent pertaining to an Alleged Infringement. The non-controlling Party agrees to be joined as a party plaintiff if necessary to prosecute the action or proceeding and shall provide all reasonable cooperation (including any necessary use of its name) required to prosecute such litigation; provided that the controlling Party shall reimburse the non-controlling Party for out-of-pocket expenses reasonably incurred in providing such cooperation at the controlling Party's request. The non-controlling Party will be entitled to be represented by counsel of its own choice at its own expense.

10.3.4 Recoveries. Any recovery obtained by any Party as a result of any proceeding described in this Section 10.3, by settlement or otherwise, shall be applied in the following order of priority: (a) first, to reimburse each Party for all litigation costs in connection with such proceeding paid by that Party and not otherwise recovered (on a pro rata basis based on each Party's respective litigation costs, to the extent the recovery was less than all such litigation costs); and (b) second, the remainder shall be shared in the ratio of two-thirds (2/3) to Shire and one-third (1/3) to Amicus.

10.4 Defense of Infringement Claims. If a Licensed Product becomes the subject of a Third Party's claim or assertion of infringement of a patent relating to the making, using, sale, offer for sale or importation of such Licensed Product within the Field in the Shire Territory, the Party first having notice of the claim or assertion shall promptly notify the other Party, and the Parties shall promptly confer to consider the claim or assertion and the appropriate course of action. If the claim or assertion names Shire as Defendant, then Shire shall have the right to control the defense of any proceeding, and Amicus shall have right to join in such defense at its own expense. Unless the Parties otherwise agree in writing, each Party shall have the right to defend itself against a suit that names such Party as a defendant, and the other Party shall have the right to join in such defense at its own expense. Neither Party shall enter into any settlement of any action described in this Section 10.4, or otherwise consent to an adverse judgment in any such action, that imposes a financial obligation on the other Party, or that admits the infringement or validity of any Third Party Patent without the other Party's written consent, which consent shall not be unreasonably withheld. In any event, each Party shall reasonably assist the other Party and cooperate in connection with any

litigation in which such Party is not named as a defendant, at the defending Party's request and expense.

10.5 Patent Marking. Shire agrees to mark, and require their contractors and permitted Sublicensees to mark, all Licensed Products sold or distributed for the Shire Territory pursuant to this Agreement in accordance with the applicable patent statutes or regulations in the country or countries of Manufacture or sale thereof, to the extent required by Law.

10.6 License of Third Party Rights. .

10.6.1 Existing Third Party Technology. It is understood that certain Patents Rights for the Shire Territory within the Amicus Patent Rights have been in-licensed pursuant to certain existing in-license agreements listed in Exhibit 10.6.1 hereto (the "***Existing In-Licenses***"). As required for the furtherance of the objectives of this Agreement, Amicus shall maintain the Existing In-Licenses and timely pay all fees due thereunder. In addition, it is understood by the Parties that their respective rights under Sections 10.2 and 10.3 above with respect to the Amicus Patent Rights licensed to Amicus under the Existing In-Licenses are subject to and limited by the applicable terms and conditions of the Existing In-Licenses. Amicus agrees to use Commercially Reasonable Efforts to persuade its licensors pursuant to such licenses ("***Licensors***") to fully cooperate with Shire in the defense or prosecution of any proceedings hereunder, and shall use reasonable efforts to cause the Licensors not to enter into any settlement, agreement, consent judgment or other voluntary final disposition of any proceeding or threatened proceeding which would adversely affect Shire or its rights and licenses hereunder.

10.6.2 Third Party Technology Acquired after Effective Date. In addition, if after the Effective Date, Amicus or Shire (the "***Sublicensing Party***") acquire rights from a Third Party that are to be licensed to the other Party under this Agreement, including with respect to a Related Product or Back-Up Compound under Section 6.3 or 6.4.4, respectively ("***Third Party Technology***"), but that is subject to royalty or other payment obligations to the Third Party, then the following shall apply: The licenses granted to the other Party (the "***Commercializing Party***") hereunder with respect to such Third Party Technology shall be subject to the Commercializing Party's promptly reimbursing the Sublicensing Party for any milestone payments, royalties or other amounts that become owing to such Third Party by reason of the Commercializing Party's exercise of such license or sublicense to the Third Party Technology. To the extent that any such payments made by a Party under an agreement to acquire Third Party Technology are not attributable to either Territory, but are attributable to the acquisition of rights to a Third Party Technology used for a Licensed Product, a reasonable portion of such amounts as determined by the JSC shall be deemed Development Costs to be shared under Section 7.4.1 (unless excluded from the licenses hereunder as provided in this Section 10.6.2). At the inception of the inclusion of any Third Party Technology in such license under this Agreement and thereafter upon request by the Commercializing Party, the Sublicensing Party shall disclose to the Commercializing Party a true, complete and correct written description of such payment obligations, and the Commercializing Party's obligation to reimburse such amounts following such request shall be limited to those payment obligations as so disclosed by

the Commercializing Party. In the event that the Commercializing Party does not promptly reimburse the Sublicensing Party for such amounts upon request (such amounts as determined by the JSC in accordance with this Agreement, to the extent so provided above), then such Third Party Technology shall thereafter be deemed excluded from the licenses or other subject matter licensed hereunder. Notwithstanding the above, this Section 10.6.2 shall not apply to Patent Rights licensed by Shire from a Third Party and covered by Section 7.3.4 above.

ARTICLE 11
TRADEMARKS AND COPYRIGHTS

11.1 Product Marks

11.1.1 Display. All packaging materials, labels and promotional materials for the Licensed Products in the Shire Territory shall be at the sole discretion of Shire, provided, that such packaging materials and labels shall display the Amicus trade name in reasonable size and prominence, as determined by the JCC in accordance with applicable Laws and applicable local regulations.

11.1.2 Selection; Title. Within the framework of the JCC, the Parties shall work together and seek to agree on the selection of Product Marks for each Licensed Product in the Territory. Notwithstanding the foregoing, each Party shall have final decision on the selection of Products Marks for each Licensed Product in its Territory. Each Party shall own rights to any Product Mark which is created by it or on its behalf and used in the Commercialization of a Licensed Product in the Territory, including all goodwill arising out of the use of such Product Mark. Each Party agrees that it will not use marks in the other Party's Territory for any other products other than the Licensed Product that are confusingly similar to such Product Mark.

11.1.3 Grant of License. Subject to the terms and conditions of this Agreement, each Party (the "***Trademark Licensor***") hereby grants to the other Party (the "***Trademark Licensee***") an exclusive license to use the Trademark Licensor's Product Marks in Trademark Licensee's Territory for the packaging, marketing, sale and promotion of the applicable Licensed Product in accordance with the Trademark Licensor's reasonable trademark usage guidelines, provided, however, that in the event a Party, after the Effective Date, generates a new Trademark for Licensed Products, the other Party shall not have a license to such new Trademark unless it reimburses the other Party for fifty (50%) of the costs incurred to identify, design and register (including clearance and registerability searches) such Trademark.

11.1.4 Registration of Trademarks; Recordation. Each Trademark Licensee shall file, register and maintain for the Term appropriate registrations for the Trademark Licensor's Product Marks in the name of the Trademark Licensor, as mutually agreed by the Parties, in each country of the Territory in which the Licensed Products are or will be sold, at its own expense. In those countries where a trademark license must be recorded, the Trademark Licensor will provide to the Trademark Licensee, on the Trademark Licensee's written request, a separate trademark license for the Trademark Licensor's Product Marks and will arrange for the recordation of such trademark

license with the appropriate governmental agency, at the Trademark Licensee's expense, promptly following receipt of such license from the Trademark Licensor. Each Party shall cooperate in the preparation and execution of such documents

11.1.5 Approval of Materials. To the extent necessary to preserve the Trademark Licensor's legal rights in its Product Marks, the Trademark Licensee shall submit representative promotional materials, packaging and samples of a Licensed Product displaying the Product Marks for the Trademark Licensor's review and approval prior to the first use of such promotional materials, packaging or Licensed Product and prior to any subsequent change or addition to such promotional materials, packaging or Licensed Product; provided that if the Trademark Licensor has not responded within twenty (20) days after the Trademark Licensee's receipt of such promotional materials, packaging or Licensed Product, the Trademark Licensor's approval will be deemed to have been received. In any case, neither Party will permit the quality of the Licensed Products with which the other Party's Product Marks are used to deteriorate so as to affect adversely the goodwill associated with such Product Marks.

11.1.6 Enforcement. Amicus and Shire shall reasonably cooperate with each other to protect each other's Product Marks. The JSC shall determine whether and to what extent to institute and prosecute or defend any actions or proceedings involving or affecting the Trademark Licensor's Product Marks in the Trademark Licensee's Territory. The Parties shall reasonably cooperate in any action taken to enforce or defend the other Party's Product Marks in the Territory, including taking appropriate appeals.

ARTICLE 12
REPRESENTATIONS, WARRANTIES AND COVENANTS

12.1 Mutual Representations, Warranties and Covenants. Each Party hereby represents, warrants and covenants to the other Party as follows:

12.1.1 Due Organization. Such Party is a corporation duly organized, validly existing and in good standing under the laws of the jurisdiction of its incorporation, and is qualified to do business and is in good standing as a foreign corporation in each jurisdiction in which the conduct of its business or the ownership of its properties requires such qualification and failure to have such qualification would prevent it from performing its obligations under this Agreement.

12.1.2 Due Execution. The execution, delivery and performance by such Party of this Agreement have been duly authorized by all necessary corporate action and do not and will not (i) require any consent or approval of its stockholders, (ii) violate any provision of any Law, order, writ, judgment, injunction, decree, determination or award presently in effect having applicability to it or any provision of its charter or bylaws or (iii) conflict with or constitute a default under any other agreement to which such Party is a party.

12.1.3 Binding Agreement. This Agreement is a legal, valid and binding obligation of such Party, enforceable against it in accordance with the terms and conditions hereof (except as

enforceability may be limited by applicable bankruptcy, insolvency, reorganization, moratorium or similar laws affecting the enforcement of creditors' rights generally.

12.1.4 Authorizations. Such Party has obtained all authorizations, consents and approvals, governmental or otherwise, necessary for such Party to grant the rights and licenses granted by such Party under this Agreement, and to otherwise perform such Party's obligations under this Agreement.

12.1.5 Third Party Agreements. Such Party has not previously granted and, during the Term will not grant, any rights in conflict with the rights and licenses granted herein. As of the Effective Date, there are no existing agreements, options, commitments or rights with, of or to any person or entity to acquire or obtain any rights with respect to such Party's intellectual property, which are in conflict with the rights and licenses granted herein.

12.1.6 Debarment. Such Party has not been debarred or is subject to debarment and neither it not any of its Affiliates have used or will use in any capacity, in connection with the Development or Commercialization of Licensed Products, any person or entity who has been debarred pursuant to Section 306 of the United States Federal Food, Drug and Cosmetic Act, or who is subject of a conviction described in such Section 306. Further, such Party agrees to inform the other Party in writing immediately if it or any person or entity who is performing services hereunder is debarred or is the subject of a conviction described in such Section 306, or if any action, suit, claim, investigation or legal administrative proceeding is pending or, to the best of such Party's knowledge, is threatened, relating to the debarment of such Party, its Affiliates or any person or entity used in any capacity by such Party or its Affiliates in connection with the Development, Manufacturing or Commercialization of Licensed Products.

12.1.7 Development Activities. To the best of such Party's knowledge, such Party, its contractors and its consultants have conducted and shall continue to conduct, as applicable, all research and Development, including non-clinical studies and clinical studies of Licensed Products and all Manufacturing of Materials in accordance with all material provisions of applicable Laws or standards of the United States and other countries in which such activities are conducted. Such Party has conducted or is planning to conduct, as applicable, appropriate audits of its contract-manufacturer organizations and contract research organizations relating to compliance with Laws and has found no circumstances that such Party believes would be likely to have a material adverse effect on the Development, Manufacturing, use or Commercialization of Materials as contemplated by this Agreement. Neither such Party nor, to its knowledge, any officer, employee or agent of such Party has made or shall make, as applicable, an untrue statement of a material fact to any Regulatory Authority with respect to Licensed Products (whether in any submission to such Regulatory Authority or otherwise), or knowingly failed to disclose or shall knowingly fail to disclose, as applicable, a material fact required to be disclosed to any Regulatory Authority with respect to Licensed Products.

12.2 Amicus Additional Representations, Warranties and Covenants. Except as disclosed on Schedule 12.2 attached hereto, Amicus hereby represents, warrants and covenants to Shire as of the Effective Date as follows:

12.2.1 Existing In-License.

(a) The Existing In-Licenses are in full force and effect, and to the best of Amicus' knowledge as of the Effective Date, no Party to such agreements (including Amicus) is in breach or default thereunder. Amicus has not waived or allowed to lapse or terminate any of its rights relating to the Compounds or Licensed Products under the Existing-In-Licenses.

(b) Amicus has provided a true and complete copy of each Existing In-License to Shire.

(c) Amicus will not during the Term amend such Existing-In Licenses in a manner that would adversely affect the rights, obligations or economic interests of Shire under this Agreement without Shire's prior written consent.

(d) Amicus shall, or shall cause Licensors to, furnish Shire with copies of all notices received by Amicus relating to any alleged breach or default by Amicus under the Existing-In-Licenses within five (5) Business Days after Amicus' receipt thereof. In the event Amicus does not resolve any such alleged breach, it shall notify Shire within a sufficient period of time before the expiration of the cure period for such breach under such Existing-In-License such that Shire is able to cure or otherwise resolve such alleged breach. If Shire makes any payments to a Licensor in connection with the cure or other resolution of such alleged breach of Amicus, then Shire may credit the amount of such payments (to the extent such amount was actually due under the applicable Existing In-License) against any royalties or other payments payable to Amicus pursuant to this Agreement.

(e) Amicus shall promptly furnish Shire with copies of (a) all amendments of the Existing In-Licenses and (b) correspondence (or in the case of oral discussions, summary of such discussions) with or from and reports received from or provided to Licensors to the extent material to Shire or its rights granted under this Agreement.

(f) Amicus shall use Commercially Reasonable Efforts to obtain standby licenses in favor of Shire under each of the Existing-In Licenses as promptly as practicable following the Effective Date.

12.2.2 Intellectual Property. As of the Effective Date:

(a) The Amicus Patent Rights, Amicus Know-How and Product Marks licensed to Shire pursuant to this Agreement constitute all of the intellectual property that is Controlled by Amicus and used in the Development, Manufacture or Commercialization of Plicera, Amigal and AT2220 and, to the best of Amicus' knowledge, the Development, Manufacture or Commercialization of Plicera, Amigal and AT2220 in the Shire Territory do not infringe the intellectual property rights of any Third Party.

(b) To the best of Amicus' knowledge, Amicus Patent Rights and Amicus Know-How are the only intellectual property rights required in order to Manufacture, Develop, use, import and/or sell or Commercialize Plicera, Amigal and AT2220 in the Shire Territory.

(c) Amicus holds good title to and is the legal and beneficial owner or licensee of the Amicus Patent Rights and Amicus Know-How free and clear of any lien, mortgage, security interest, pledge, restriction on transferability, defect of title or other claim, charge or encumbrance (other than the terms of the Existing In-Licenses), and Amicus has not granted any

Third Party any right, title or interest in the Amicus Patent Rights or Amicus Know-How directly relating to the Development, Manufacture or Commercialization of Licensed Products in the Shire Territory.

(d) Appendix 1 sets forth all of the Amicus Patent Rights, including the legal and beneficial owner or applicant for registration of each Amicus Patent Right.

(e) All actions required to maintain the good standing of the Amicus Patent Rights (including payment of all applicable fees due and payable to a governmental authority before the Effective Date and timely compliance with filing, prosecution and maintenance requirements) have been taken.

(f) There are no claims, judgments or settlements against or owed by Amicus, nor any pending reissue, reexamination, interference, opposition or similar proceedings, with respect to the Amicus Patent Rights or Amicus Know-How, and Amicus has not received written notice as of the Effective Date of any threatened claims or litigation or any reissue, reexamination, interference, opposition or similar proceedings seeking to invalidate or otherwise challenge the Amicus Patent Rights or Amicus Know-How.

(g) To the best of Amicus' knowledge, there are no pending Third Party patent applications which, if issued, would materially adversely affect the right of Shire to practice under the Amicus Patent Rights in accordance with this Agreement.

(h) To the best of Amicus' knowledge, there have been no and there is no reason to believe that there will be any, inventorship challenges with respect to any of the Amicus Patent Rights.

(i) The Amicus IP is not and, to the best of Amicus knowledge during the Term, will not become subject to any rights granted in favor of a Third Party that are in conflict with or otherwise restrict the rights granted to Shire hereunder (subject to the Existing In-Licenses).

(j) All current and former employees and consultants of Amicus and its Affiliates who are or have been substantively involved in the design, review, evaluation or development of the Compounds and Licensed Products have executed written contracts or are otherwise obligated to protect the confidential status and value thereof and to vest in Amicus or its Affiliates exclusive ownership of the Compounds and Licensed Products (to the extent invented by such persons).

12.2.3 Regulatory, Clinical, Preclinical and Clinical Studies. As of the Effective Date:

(a) Regulatory Filings. Neither Amicus nor its Affiliates, nor, to the best of Amicus' knowledge, its subcontractors, has received any notice in writing or otherwise has knowledge of any facts which have led Amicus to believe that any of the Regulatory Filings relating

to Plicera, Amigal or AT2220 are not currently in good standing with the FDA or any other applicable Regulatory Authority.

(b) No Inquiries. Neither Amicus, nor to the best of Amicus' knowledge, its subcontractors has received written notice of any proceedings pending before or threatened by any Regulatory Authority with respect to Plicera, Amigal or AT2220 or any facility where any such product is Manufactured.

(c) Disclosure. Amicus has disclosed to Shire and/or made available to Shire for review all relevant data and documentation (including, without limitation, all relevant correspondence with Regulatory Authorities, both in the United States and outside the United States, related to the foregoing) in its possession or control, that would be material in order to assess the safety and efficacy of Licensed Products, including all such pre-clinical and clinical data and all such efficacy data regarding Licensed Products.

(d) Safety Issues. Amicus is not aware of any safety, efficacy, or regulatory issues, other than the information that has previously been made available to Shire, that would preclude Shire or Amicus, or their licensees and contract service organizations, from researching, Developing, Manufacturing or Commercializing Licensed Products in compliance with Laws, including but not limited to issues relating to the system for maintaining relevant documents, the internal audit systems, and any other regulatory-related matter.

12.2.4 Manufacture and Supply.

The JSC shall approve the terms and conditions of all manufacture and supply agreements and other arrangements under which Amicus procures Materials for use under the Development Plans or for supply to Shire under this Agreement. The Parties intend that (i) Shire and its Affiliates shall be third party beneficiary of such warranty and covenants in any manufacture and supply agreements and other arrangements under which Amicus procures Materials for use under the Development Plans or for supply to Shire under this Agreement and (ii) Amicus shall make available to Shire any benefits under indemnification provisions under any manufacture and supply agreements.

12.2.5 **[***]** for [***]'s.

(a) Amicus has disclosed and made available to Shire for review all material Data or summaries thereof (including, without limitation, all relevant correspondence with Regulatory Authorities, both in the United States and outside the United States, related to the foregoing) in Amicus' possession or Control with respect to **[***]** (including **[***]**) for the treatment, prevention and diagnosis of [***]'s, including, without limitation, all such pre-clinical and clinical data and all such efficacy data regarding **[***]** for [***]'s.

(b) Amicus shall allocate at least [***] full time equivalent personnel to identify, Develop and/or acquire a molecule as a substitute for [***] for the treatment and/or

prevention of [***]'s, provided, however, that Amicus may terminate such activities, and its obligation under this Section 12.2.5(b): (i) upon the first [***] with respect to an [***] for [***]'s, or if earlier, upon termination of the [***]'s Option under Section 6.2.1(e), or (ii) at such earlier time as the JDC, upon the request of Amicus, determines that Commercially Reasonable Efforts to find such substitute would not require the continuation of such activities. Amicus shall provide periodic updates regarding activities conducted by it pursuant to its obligations under this Section (including the results of such activities) at the request of Shire and further shall provide an annual report on such activities and results.

12.3 Disclaimer. EXCEPT AS SET FORTH IN THIS ARTICLE 12, AMICUS AND SHIRE EXPRESSLY DISCLAIM ANY OTHER WARRANTIES OR CONDITIONS, EXPRESS, IMPLIED, STATUTORY OR OTHERWISE, WITH RESPECT TO THE PATENT RIGHTS OR KNOW-HOW OR THE SUBJECT MATTER OF THIS AGREEMENT (INCLUDING WITH RESPECT TO LICENSED PRODUCTS AND ANY RESEARCH AND DEVELOPMENT ACTIVITIES RELATING THERETO), INCLUDING ANY IMPLIED WARRANTIES OF MERCHANTABILITY, NON-INFRINGEMENT OR FITNESS FOR A PARTICULAR PURPOSE.

ARTICLE 13
INDEMNIFICATION; INSURANCE

13.1 Indemnification of Shire. Amicus shall indemnify and hold harmless each of Shire, its Affiliates and the directors, officers and employees of such entities and the successors and assigns of any of the foregoing (the "***Shire Indemnitees***") from and against any and all liabilities, damages, penalties, fines, costs and expenses (including reasonable attorneys' fees and other expenses of litigation) (collectively, "***Liabilities***") resulting from claims, actions, suits or proceedings brought by a Third Party (a "***Third Party Claim***") that are incurred by any Shire Indemnitee, arising from or occurring as a result of: (a) the Development or Commercialization of any Licensed Product, or other product containing the Compound, in the Amicus Territory, in each case by or under authority of Amicus or its Affiliates, (b) any gross negligence or willful misconduct of Amicus, its Affiliates, or their officers, directors, employees, contractors, consultants, agents, representatives, or licensees in the exercise of any obligations under this Agreement or (c) any material breach by Amicus of any representations, warranties or covenants set forth in this Agreement, except to the extent such Third Party Claims fall within the scope of Shire's indemnification obligations set forth in Section 13.2.

13.2 Indemnification of Amicus. Shire shall indemnify and hold harmless each of Amicus, its Affiliates and the directors, officers 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 from any Third Party Claims incurred by any Amicus Indemnitee, arising from or occurring as a result of (a) the Development or Commercialization of any Licensed Product in the Shire Territory, in each case by or under authority of Shire or its Affiliates or Sublicensees, (b) any gross negligence or willful misconduct of Shire, its Affiliates or Sublicensees, or their officers, directors, employees, contractors, consultants, agents, representatives, or licensees in the exercise of any obligations under

this Agreement or (c) any material breach by Shire of any representations, warranties or covenants set forth in this Agreement, except to the extent such Third Party Claims fall within the scope of Amicus' indemnification obligations set forth in Section 13.1.

13.3 Procedure. A Party that intends to claim indemnification under this Article 13 (the "***Indemnitee***") shall promptly notify the other Party (the "***Indemnitor***") in writing of any Third Party Claim in respect of which the Indemnitee intends to claim such indemnification, and the Indemnitor shall have the right to control the defense and/or settlement thereof with counsel of its choice as long as such counsel is reasonably acceptable to the Indemnitee. The Indemnitee shall have the right to participate in such defense and/or settlement at its own expense with counsel of its choice. The indemnity arrangement in this Section 13.3 shall not apply to amounts paid in settlement of any action with respect to a Third Party Claim if such settlement is effected without the consent of the Indemnitor, which consent shall not be unreasonably withheld or delayed. 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 relieve such Indemnitor of any Liabilities that result from any delay in providing such notice which materially prejudices the defense of such Third Party Claim under this Section 13.3, but the omission to so deliver written notice to the Indemnitor shall not relieve the Indemnitor of any liability that it may have to any Indemnitee otherwise than under this Section 13.3. The Indemnitee under this Section 13.3 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 Article 13.

13.4 Insurance. Each Party shall procure and maintain insurance, including product liability insurance, which is consistent with normal business practices of prudent companies similarly situated at all times during which any Licensed Product is being clinically tested in human subjects or commercially distributed or sold by such Party and the insurance coverage shall in no event be less than: (a) prior to the First Commercial Sale of a Licensed Product anywhere in the world, $**[***]** per loss occurrence and $**[***]** in the aggregate, and (b) after such First Commercial Sale, $**[***]** per loss occurrence and $**[***]** in the aggregate. It is understood that such insurance shall not be construed to create a limit of either Party's liability with respect to its indemnification obligations under this Article 13. Each Party shall provide the other Party with written evidence of such insurance upon request. Each Party shall provide the other Party with written notice at least thirty (30) days prior to the cancellation, non-renewal or material change in such insurance or self insurance which materially adversely affects the rights of the other Party hereunder.

ARTICLE 14
CONFIDENTIALITY

14.1 Confidentiality; Exceptions. Except to the extent expressly authorized by this Agreement or otherwise agreed in writing, the Parties agree that the receiving Party shall keep confidential and shall not publish or otherwise disclose or use for any purpose other than as provided for in this Agreement any information and other confidential and proprietary materials furnished to it by the other Party pursuant to this Agreement collectively and except to the extent any of Sections

14.1.1. to 14.1.4 are applicable ("***Confidential Information***"), except to the extent that it can be established by the receiving Party that such Confidential Information:

14.1.1 was in the lawful knowledge and possession of the receiving Party prior to the time it was disclosed to, or learned by, the receiving Party, or was otherwise developed independently by the receiving Party, as evidenced by written records kept in the ordinary course of business, or other documentary proof of actual use by the receiving Party;

14.1.2 was generally available to the public or otherwise part of the public domain at the time of its disclosure to the receiving Party;

14.1.3 became generally available to the public or otherwise part of the public domain after its disclosure and other than through any act or omission of the receiving Party in breach of this Agreement; or

14.1.4 was disclosed to the receiving Party, other than under an obligation of confidentiality, by a Third Party who had no obligation to the disclosing Party not to disclose such information to others.

14.2 Authorized Disclosure. Except as otherwise expressly provided in this Agreement, each Party may use and disclose Confidential Information of the other Party as follows: (a) under appropriate confidentiality provisions substantially equivalent to those in this Agreement, in connection with the performance of its obligations or exercise of rights granted or reserved in this Agreement (including to grant licenses and sublicenses permitted hereunder, and in the case of Amicus, to Develop, Manufacture and Commercialize Licensed Products and Compounds for use in the Amicus Territory and, in the case of [***] for [***]'s, outside the Field), (b) to the extent such disclosure is reasonably necessary in filing or prosecuting patent, copyright and trademark applications, complying with the terms of licenses from Third Parties, prosecuting or defending litigation, complying with applicable governmental regulations, obtaining Regulatory Approval, conducting preclinical or clinical trials, or marketing Licensed Products, or otherwise required by Law (including securities Laws), provided, however, that if a Party is required by Law to make any such disclosure of the other Party's Confidential Information it will, except where impracticable for necessary disclosures (for example, in the event of medical emergency), give reasonable advance notice to the other Party of such disclosure requirement and, except to the extent inappropriate in the case of patent applications, use its reasonable efforts to secure confidential treatment of such Confidential Information required to be disclosed, (c) in communication with investors, consultants, advisors or others on a need to know basis, in each case under appropriate confidentiality provisions substantially equivalent to those of this Agreement, (d) in the case of Amicus, to the extent necessary to comply with its obligations to provide progress reports to its licensors under the Existing In-Licenses, under appropriate confidentiality provisions, or (e) to the extent mutually agreed to by the Parties.

14.3 Termination of Prior Agreement. This Agreement supersedes the Confidentiality Agreement between the Parties dated June 6, 2007 ("***Confidentiality Agreement***"), including all

modifications thereto. All Confidential Information (as defined in the Confidentiality Agreement) exchanged between the Parties under such agreement shall be deemed Confidential Information and shall be subject to the terms of this Article 14.

14.4 Disclosure of Terms. Each Party agrees not to disclose to any Third Party the terms of this Agreement without the prior written consent of the other Party, except as permitted for disclosures of Confidential Information pursuant to Section 14.2.

14.5 Publications. Except as required by applicable Law, each Party agrees that it shall not publish or present the results of Development work or Post-Marketing Studies conducted by such Party that are directed to any Licensed Product for an indication in the Field, including but not limited to studies or clinical trials carried out by such Party as part of a Development Plan under this Agreement (each, a "***Collaboration Results Publication***"), without providing the other Party the opportunity for prior review, it being understood, however, that publication of such Collaboration Results Publication shall not require approval of the other Party. Each Party shall provide to the other Party the opportunity to review any of the submitting Party's proposed abstracts, manuscripts or presentations (including information to be presented verbally) comprising such a Collaboration Results Publication (including any proposed Third Party publication submitted to the submitting Party for review and approval, to the extent the applicable terms of any agreement with such Third Party permit) at least fifteen (15) days prior to their intended presentation or submission for publication. Once such abstracts, manuscripts or presentations have been reviewed by each Party, the same information contained in such abstracts, manuscripts or presentations does not have to be provided again to the other Party for review for a later submission for publication. Each Party shall also have the right to require that its Confidential Information be removed from any such approved Collaboration Results Publication. Notwithstanding the foregoing, any Collaboration Results Publication shall be delayed upon the request of Party for a period not less than sixty (60) days, if such Party requests such delay in order to allow for filing a patent application or taking such measures as such Party deems appropriate to establish and preserve its proprietary rights.

14.6 Press Releases and Announcements

14.6.1 Initial Release. On the Effective Date or, if mutually agreed, promptly after the Effective Date, each Party shall have the right to release a press release announcing this Agreement and the relationship of the Parties, provided each Party's such press release will be in the form provided to the other Party prior to the Effective Date. Thereafter, Shire and Amicus may each disclose to Third Parties the information contained in each such press release without the need for further approval by the other.

14.6.2 Further Publicity. The Parties acknowledge the importance of supporting each other's efforts to publicly disclose results and significant developments regarding Licensed Products in the Shire Territory and other activities in connection with this Agreement in the Shire Territory that may involve Confidential Information of the other Party generated or obtained in connection with this Agreement pertaining to the Licensed Products, 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. Such disclosures may include, without limitation, achievement of Development milestones, significant events in the Development and regulatory process with respect to Licensed Products, Commercialization activities and the like. When a Party (the "***Initiating Party***") elects to make any such public disclosure under this Section 14.6.2, it will give the other Party (the "***Cooperating Party***") at least five (5) Business Days notice to review and comment on such statement, it being understood that if the Cooperating Party does not notify the Initiating Party in writing within such five (5) Business Day period of any objections, such disclosure shall be deemed approved, and in any event the Cooperating Party shall work diligently and reasonably to agree on the text of any approved disclosure in an expeditious manner. The principles to be observed in such disclosures shall include 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 Initiating Party's business.

ARTICLE 15
TERM AND TERMINATION

15.1 Term. The term of this Agreement (the "***Term***") shall begin on the Effective Date and shall continue on a Licensed Product by Licensed Product basis until the expiration of the royalty term as per Section 7.3.6 above for such Licensed Product, unless and until earlier terminated as permitted under this Agreement. Upon expiration (but not earlier termination) of this Agreement with respect to a Licensed Product, Shire shall have a fully paid up, license to Develop, use and Commercialize such Licensed Product within the Field in the Shire Territory, provided, that Shire's license to Develop and Manufacture shall be a worldwide license.

15.2 Termination for Breach. In the event of a material breach of this Agreement, the non-breaching Party shall have the right to give written notice (the "***Breach Notice***") to the breaching Party, specifying the breach in reasonable detail. The breaching Party shall have **[***]** after the Breach Notice to cure any such breach. If, at the end of such **[***]** period, the breach remains uncured, then the non-breaching Party shall have the right to terminate this Agreement upon written notice, in its entirety or on a Licensed Product-by-Licensed Product basis.

15.3 Termination by Shire.

15.3.1 Generally. Subject to Section 15.3.4 below, Shire may terminate this Agreement for any reason upon **[***]** prior written notice to Amicus

15.3.2 Licensed Product by Licensed Product. In addition, subject to Section 15.3.4 below, Shire may terminate this Agreement as to any Licensed Product, upon **[***]** prior written notice to Amicus.

15.3.3 Safety. In addition, if Shire exercises its right of termination under this Section 15.3 because it reasonably believes that a Licensed Product is unsafe for human use, then Shire shall promptly provide Amicus with reasonable evidence of such safety concern and notwithstanding Section 6.4.2 and Section 15.5 below, Shire may cease to conduct any further

Development or Commercialization of such Licensed Product. If Shire invokes this Section 15.3.3 without a reasonable basis, it is understood that doing so shall be deemed a breach of this Agreement, and Amicus shall have the right to pursue any remedies available for such breach under applicable Law or this Agreement.

15.3.4 Initial Period. Notwithstanding the foregoing, Shire shall not issue any notice of termination under (a) Section 15.3.1 within **[***]** after the Effective Date or (b) Section 15.3.2 within **[***]** after the Effective Date, provided, that it shall not issue any notice of termination under Section 15.3.2 within **[***]** after the Effective Date with respect to more than two (2) Licensed Products.

15.4 Termination for 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 for the other Party or its assets, or if the other Party is served with an involuntary petition against it, filed in any insolvency proceeding, and such petition shall not be dismissed with ninety (90) days after the filing thereof, or if the other Party makes a general assignment for the benefit of creditors.

15.5 Effects of Expiration or Termination

15.5.1 Accrued Obligations. Expiration or termination of this Agreement for any reason shall not release either Party any obligation or liability which, at the time of such expiration or termination, has already accrued to the other Party or which is attributable to a period prior to such expiration or termination.

15.5.2 Termination by Amicus under Section 15.2 or Action of Shire under Section 15.3.1. If this Agreement is terminated by Amicus under Section 15.2 or by Shire pursuant to Section 15.3.1, then:

(a) Development.

(i) If, on the date of notice of such termination, Shire was conducting any ongoing clinical trials of one or more Licensed Products (collectively with all Licensed Products for which the Agreement is terminated, "***Reverted Products***"), then, to the extent and as requested by Amicus, Shire shall promptly transition such clinical trials to Amicus or (except for Independent Projects) continue to conduct such clinical trials for a period requested by Amicus up to **[***]** after the effective date of such termination. During this period, the out-of-pocket costs that Shire reasonably incurs in performing such clinical trials at Amicus' request shall be deemed Wind-Down Development Expenses for purposes of (and shall be shared by the Parties in accordance with) Section 15.5.2(a)(ii) below.

(ii) [***] (the "***Notice Period***") following the date of the notice of termination under Section 15.2 or 15.3.1, as applicable (the "***Notice Date***"), and (B) any **[***]**, or by **[***]** in performing activities at Amicus' request under Section 15.5.2(a)(i) above, during the **[***]** period following the Notice Period (the "***Development Period***") for clinical trials initiated prior to the Notice Date in accordance with the applicable Development Plan (collectively (A) and (B), the **[***]**) (for clarity such **[***]** shall not include any expenses for activities related to Independent Projects); provided that, with respect to the **[***]** described in (B) above, **[***]** shall be reduced from **[***].** Promptly following the end of each calendar quarter until the end of the Development Period, each Party shall provide written documentation of the actual **[***]** that have then been incurred by it and that reflect the Development conducted according to this Section 15.5.2(a)(ii) since the last such report, and the Parties shall **[***]** in accordance with Section 7.4.4(c) above, until all such Wind-Down Development Expenses have been so reported and paid. In the event the budget in the applicable Development Plan does not extend for the full duration of the Development Period, such budget shall be deemed extended until the end of the Development Period to include the costs of such continuing Development activities. As used herein, **[***]** shall mean (x) **[***]** to Third Party contractors performing work under the applicable Development Plan (such as CRO's or clinical trial sites), as well as (y) the **[***]** of Licensed Products consumed in performing such clinical trials.

(b) Commercialization. With respect to Licensed Products being Commercialized at the time of such termination, Shire, its Affiliates and permitted Sublicensees shall continue to sell the Reverted Products in each country in the Shire Territory for which Regulatory Approval has been obtained, in accordance with the terms and conditions of this Agreement, for a period requested by Amicus not to exceed the lower of **[***]** from the effective date of termination or upon the completion of the transfer of Regulatory Approvals allowing Amicus or its designee to sell such License Product (the "***Wind-down Period***"), provided that Amicus may terminate the Wind-down Period upon **[***]** written notice to Shire and provided, further, that Shire shall not be obligated to promote the sale of Reverted Products in the Shire Territory during the Wind-down Period. Notwithstanding any other provision of this Agreement, during the period from and after the notice of termination, Shire's and its Affiliates' and permitted Sublicensees' rights with respect to the Reverted Products in the Shire Territory shall be non-exclusive. All Net Sales from sales of Reverted Products sold or disposed by Shire in the Shire Territory during the Wind-down Period shall be paid to Amicus, less (x) a fixed distribution fee equal to **[***]** of such Net Sales and (y) the Manufacturing Cost of quantities of the Licensed Product included in such Net Sales. Except as provided in this Section 15.5.2(b), after termination, Shire and its Affiliates and Sublicensees shall not sell any quantities of Reverted Products produced or obtained pursuant to this Agreement.

(c) Transition Assistance. Shire shall cooperate with reasonable requests by Amicus to achieve, as promptly as reasonably practicable during the period from notice of termination until the end of the Wind-down Period, a smooth and orderly transition to Amicus of the Development and Commercialization of the Reverted Products in the Territories, including making its personnel and other resources reasonably available to Amicus. If Shire has entered into contracts with contractors (including contract manufacturers) or vendors that are necessary or useful for

Amicus to take over responsibility with respect to the Reverted Products in the Territories, then Shire shall, to the extent possible and requested in writing by Amicus, assign all of the relevant Third Party agreements to Amicus, or otherwise cooperate to make such arrangements available to Amicus or its designee for purposes of the Licensed Products.

(d) Assignment of Regulatory Filings and Regulatory Approvals. Shire shall assign and transfer, or cause to be assigned and transferred, to Amicus all Regulatory Filings and Regulatory Approvals solely for the Reverted Products made or owned by Shire and its Affiliates, and shall take such actions and execute such other instruments, assignments and documents as may be necessary to effect the transfer of rights under such Regulatory Filings and Regulatory Approvals to Amicus (or, if not so assignable or not solely related to Related Products, Shire shall take all reasonable actions to make available to Amicus the benefits of such Regulatory Filings and Regulatory Approvals). Shire shall require each of its Sublicensees and any other Third Party that holds Regulatory Filing or Regulatory Approvals under authority from Shire hereunder solely related to Reverted Products to transfer any such Regulatory Filings and Regulatory Approvals to Amicus if this Agreement terminates (or, if not so assignable or not solely related to Related Products, Shire shall take all reasonable actions to make available to Amicus the benefits of such Regulatory Filings and Regulatory Approvals). In each case, unless otherwise prohibited by any applicable Laws, the foregoing assignment (or availability) shall be made within **[***]** after termination of this Agreement.

(e) Data and Know-How Disclosure. Within **[***]** after the Notice Date, Shire shall disclose to Amicus (to the extent Shire has not already disclosed to Amicus) all Know-How in Shire's or its Affiliates' possession or Control with respect to the Reverted Products Developed under this Agreement. Such disclosure shall be in electronic form to the extent available and, if reasonably necessary in connection with Amicus' further Development, Manufacture or Commercialization of the Reverted Products, shall include original hardcopies or duplicate copies thereof to the extent available, as required. Amicus shall be free to use this Know-How in accordance with the license under Section 15.5.2(f) below.

(f) Licenses. Shire shall grant, and hereby grants, to Amicus, effective upon the Notice Date, a perpetual, fully paid-up non-exclusive license, with the right to grant and authorize sublicenses, to use Shire Inventions (including under all Patent Rights inherent thereto), the Know-How provided or to be provided to Amicus under this Agreement and all copyrighted materials Controlled by Shire, in each case pertaining to the Reverted Products, to Develop, Commercialize, Manufacture and otherwise exploit the Reverted Products, or other products containing a Compound (other than, in the case of termination under Sections 6.4.3 or 15.3.2, Compounds included in Licensed Products for which Shire retains its license under Section 2.1).

(g) Trademarks and Copyrights. Upon payment of the costs incurred to identify, design and register (including clearance and registerability searches) of Product Marks (to the extent not previously paid by Amicus pursuant to Section 11.1.3), Shire shall promptly assign to Amicus, at Amicus' sole reasonable expense (with no royalty obligations) all rights of Shire in and

to the Product Marks and other product-specific trademarks for Reverted Products, including applicable registrations and associated goodwill.

(h) Sublicenses. Each Sublicense granted by Shire or its Affiliates hereunder shall, at the request of Amicus and in its discretion, be assigned to Amicus to the furthest extent possible. In the event that such assignment is not requested by Amicus or is not approved by such Sublicensee, then the rights of such Sublicensee with respect to Reverted Products in the Shire Territory shall terminate upon termination of Shire's license with respect to the Shire Territory. Shire shall ensure that its Affiliates and Sublicensees (if the applicable Sublicense is not assigned to Amicus pursuant to this Section 15.5.2(h)) transition the Reverted Products back to Amicus in the manner set forth in this Section 15.5.2 as if such Affiliate or Sublicensee were named herein.

15.5.3 Partial Termination. If this Agreement is terminated under Section 15.2 or 15.3.2 with respect to one or more Licensed Products but not this Agreement in its entirety, it is understood that Section 6.4.3 shall apply with respect to such terminated Licensed Product.

15.5.4 Termination by Shire under Section 15.2. If this Agreement is terminated by Shire under Section 15.2, then:

(a) Licenses and Payments. Shire shall continue to retain the licenses and other rights granted to Shire under Article 2 and 11 above, provided that Shire continues to fulfill its payment obligations under Article 7 above, subject to any right of offset Shire may have under applicable Law for damages resulting from Amicus' breach of this Agreement; provided that any royalties due under Section 7.3 and milestone payments due under Section 7.2.1 that are achieved after the date of such termination shall be reduced by **[***]%,** provided, that in the event any Licensed Product is then being Commercialized, any royalty due under Section 7.3 for such Licensed Product shall be reduced by **[***]%**.

(b) Development. Notwithstanding anything herein to the contrary, commencing upon the effective date of termination, Shire shall be permitted to engage in Development activities outside of the applicable Development Plan(s) with respect to Licensed Products in the Field in the Shire Territory, and Shire shall not be required to provide to Amicus under Section 2.3 or Article 9 above the Data and other results of such activities nor permit Amicus to have access to or participate in regulatory matters in the Shire Territory related to such activities in accordance with Article 9.

(c) Data and Know-How Disclosure. Within **[***]** after the termination of this Agreement, Amicus shall, at Shire's sole reasonable expense, disclose to Shire (to the extent Amicus has not already disclosed to Shire) all Know-How in Amicus' or its Affiliates' possession or Control required to be disclosed under Section 2.3.2 above.

(d) Reservation of Rights. The exercise by Shire of its rights under this Section 15.5.4 shall in no way limit any other remedies available to Shire in connection with such termination.

15.5.5 Rights in Bankruptcy. All rights and licenses granted under or pursuant to this Agreement by one Party to the other are, and shall otherwise be deemed to be, for purposes of Section 365(n) of the U.S. Bankruptcy Code and other similar foreign Laws, licenses of rights to "intellectual property" as defined under Section 101 of the U.S. Bankruptcy Code or such foreign Laws. Each Party, as a Sublicensee of rights under this Agreement, shall retain and may fully exercise all of its rights and elections under the U.S. Bankruptcy Code and other similar foreign Laws.

15.5.6 Public Disclosure. The Parties shall use good faith efforts to coordinate any public disclosure regarding any termination under this Agreement, subject to compliance with applicable Laws, including securities Laws.

15.6 Survival.

15.6.1 Surviving Articles and Sections. Articles 1, and 16, and Sections 2.3.5 (with respect to Amicus' rights thereunder) 4.5.2 (with respect to licenses granted thereunder), 6.1.2(d) (with respect to licenses granted thereunder), 10.1, 13.1-13.3 and 14.1-14.4, 15.1, 15.5 and 15.6 shall survive expiration or termination of this Agreement for any reason. Except as otherwise provided in this Article 15, all rights and obligations of the Parties under this Agreement shall terminate upon expiration or termination of this Agreement for any reason.

15.6.2 Committee Decisions. To the extent that any provision of this Agreement that provides for a decision to be made by a Committee survives termination of this Agreement pursuant to this Article 15, such matter shall be decided by the Parties jointly, and any dispute between the Parties with respect to any such matter shall be resolved as if it were a Committee Dispute under Section 16.8 below.

ARTICLE 16
GENERAL PROVISIONS

16.1 Assignment. This Agreement shall not be assignable by either Party to any Third Party hereto without the written consent of the other Party hereto, except that (a) either Party may assign this Agreement without the other Party's consent to an entity that acquires substantially all of the business or assets of the assigning Party (or, in the case of Shire, Shire's **[***]** business), whether by merger, asset sale or otherwise, provided that the acquirer assumes this Agreement in writing or by operation of law; and (b) either Party may assign this Agreement to an Affiliate upon written notice to the non-assigning Party; provided that in the case of (b), (i) the assigning Party guarantees the performance of this Agreement by such Affiliate and (ii) if the non-assigning Party reasonably believes that assignment to such Affiliate would result in adverse tax consequences to the non-assigning Party, such assignment shall not be made without the non-assigning Party's consent, such consent not to be unreasonably withheld. Subject to the foregoing, this Agreement shall inure to the benefit of each Party, its successors and permitted assigns. Any assignment of this Agreement in contravention of this Section 16.1 shall be null and void.

16.2 Independent Contractors. The Parties are and shall at all time be independent contractors. In performing under this Agreement, neither Party is an agent, employee, employer, joint venturer or partner of the other. Neither Party shall incur or hold itself out to Third Parties as having the authority to incur any expenses, liabilities or obligations on behalf of the other Party. This Agreement is not a partnership agreement and nothing in this Agreement shall be construed to establish a relationship of co-partners or joint venturers between the Parties.

16.3 Third Party Beneficiaries. This Agreement shall not confer any third party beneficiary rights or remedies upon any Affiliate of a Party or any Third Party, except as otherwise provided in Section 16.4.

16.4 Waiver. No waiver by a Party in any one or more instances shall be deemed to be a continuing waiver, a further waiver, a waiver of any other provision of this Agreement or a waiver of this Agreement as a whole. No waiver of any right under this Agreement shall be effective unless it is documented in a writing signed by the Party providing the waiver.

16.5 Force Majeure. A failure by a Party to perform any obligation under this Agreement that is prevented by an occurrence beyond the reasonable control of the non-performing Party (and which did not occur as a result of its financial condition, negligence or fault), including acts of God, embargoes, fires, floods, explosions, riots, wars, civil disorders, terrorist acts, rebellion or acts of sabotage (a "***Force Majeure Event***"), shall not constitute a breach of this Agreement so long as that Party notifies the other Party as soon as practicable and uses Commercially Reasonable Efforts to resume performance as soon as possible. Neither Party shall be entitled to rely on a Force Majeure Event to relieve it from an obligation to pay money (including any interest for delayed payment) which would otherwise be due and payable under this Agreement.

16.6 Severability. If any term of this Agreement is held invalid, illegal or unenforceable in any jurisdiction, then, to the fullest extent permitted by Law (a) all other terms shall remain in full force and effect in such jurisdiction, (b) such invalidity, illegality or unenforceability shall not affect the validity, legality or enforceability of such provision in any other jurisdiction and (c) the Parties shall negotiate in good faith such terms as may be necessary in order to correct any imbalance of rights and obligations that results from such invalidity, illegality or unenforceability in the relevant jurisdiction.

16.7 Governing Law; Dispute Resolution. This Agreement shall be governed by and interpreted under, and any court action shall apply, the Laws of the State of New York, excluding its conflicts of Laws principles. Subject to Section 16.8, any dispute as to the performance, enforcement, termination, validity or interpretation of this Agreement shall be brought only in a federal court of competent jurisdiction (or a state court if no federal court has jurisdiction) located in New York, New York and the Parties hereby submit to the exclusive jurisdiction and venue of such courts.

16.8 Arbitration for Committee Disputes and Certain Other Disputes.

16.8.1 Committee Disputes. The Parties agree that the inability of the JSC, JDC, JCC or any Special Committee to reach consensus on a decision that is expressly designated in this Agreement to be made by such Committee (a "***Committee Dispute***") shall be resolved through the procedures set forth in this Section 16.8.

(a) In the event that the JDC, JCC or a Special Committee is unable to reach consensus on a decision within the authority of such Committee, such Committee Dispute shall be first referred to the JSC by a Co-Chair of the JDC, JCC or such Special Committee, as applicable, who has concluded in good faith that there has been sufficient discussion of the matter and that resolution is unlikely, and the JSC shall consider such matter within fourteen (14) days. If the JSC is unable to reach a unanimous decision as to such Committee Dispute, or to a Committee Dispute within the direct authority of the JSC, within such fourteen (14) day period, such Committee Dispute shall similarly be referred for joint and mutual resolution by the Chief Executive Officer (or his/her designee) of each Party. If such Committee Dispute is not resolved by the Chief Executive Officers (or their respective designees) within thirty (30) days after being referred for their joint and mutual resolution, then such Committee Dispute shall, upon written notice of either Party to the other Party, be resolved by final, binding arbitration in accordance with the provisions of Sections 16.8.1(b) through (d).

(b) The arbitration shall be conducted by the Judicial Arbitration and Mediation Services (or its successor entity) ("***JAMS***") under its rules of arbitration then in effect, except as modified in this Agreement. The arbitration shall be conducted in the English language, by a single arbitrator. If the Parties are unable to agree on an arbitrator, the arbitrator shall be selected in accordance with the JAMS rules, or if the JAMS rules do not provide for such selection, by the chief executive of JAMS. At his or her election, the arbitrator may engage an independent expert with experience in the subject matter of the dispute to advise the arbitrator, but final decision making authority shall remain in the arbitrator. The arbitrator shall determine what discovery will be permitted, consistent with the goal of reasonably controlling the cost and time that the Parties must expend for discovery, provided that the arbitrator shall permit such discovery as he or she deems necessary to permit an equitable resolution of the dispute.

(c) The Parties and the arbitrator shall use all reasonable efforts to complete any such arbitration within ninety (90) days, and such arbitration 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 a written report setting forth its final position with respect to the substance of the dispute and the arbitrator shall then select one of the Party's positions as his or her final decision. The arbitrator shall not have authority to render any substantive decision other than to so select the position of either Amicus or Shire. Further, to the extent applicable, the arbitrator shall make such decision based on the underlying agreement of the Parties that the Parties are equally sharing all costs for Development of Licensed Products (other than under an Independent Project) in order to achieve Regulatory Approval in each of the Primary Market countries.

(d) The Parties agree that the decision of the arbitrator shall be the binding remedy between them regarding the dispute presented to the arbitrator, and in the case of a Committee Dispute shall become the decision of the JSC on the matter. The arbitration proceedings and the decision of the arbitrator shall be deemed Confidential Information of both Parties under Article 14 above. Unless otherwise mutually agreed upon by the Parties, the arbitration proceedings shall be conducted in New York, New York. The Parties agree that they shall share equally the cost of the arbitration filing and hearing fees, the cost of the independent expert retained by the arbitrator and the cost of the arbitrator and administrative fees of JAMS. Each Party shall bear its own costs and attorneys' and witnesses' fees and associated costs and expenses.

16.8.2 Disputes Regarding the Right of First Refusal. The Parties agree that it is important to be able to resolve any disputes regarding Sections 2.4, 6.1.3, 6.2.1 6.2.2, 6.3.2, 6.3.3, 6.4.4 or 6.5.5 above quickly. In the event of a dispute under such provison, such dispute shall be resolved under binding arbitration in accordance with Section 16.8.1(b)-(d).

16.9 Construction. Unless the context of this Agreement clearly requires otherwise, (a) references to any gender include all genders, (b) "including" has the inclusive meaning frequently identified with the phrase "including but not limited to" or "including without limitation" and (c) references to "hereunder" or "herein" relate to this Agreement. The section and other headings contained in this Agreement are for reference purposes only and shall not control or affect the construction of this Agreement or the interpretation thereof in any respect. Section, subsection, Appendix and Schedule references are to this Agreement unless otherwise specified. Each accounting term used herein that is not specifically defined herein shall have the meaning given to it under U.S. GAAP, but only to the extent consistent with its usage and the other definitions in this Agreement. In addition: (a) "***Business Day***" means a day other than a Saturday, Sunday or a day that is a statutory holiday in the United Kingdom or a federal holiday in the United States; and (b) "***Laws***" means all laws, ordinances, rules, directives and regulations of any kind of any governmental or regulatory authority of a country in the Territory (including Regulatory Authorities), in each case to the extent applicable to the respective activities of a Party that are being performed.

16.10 Notices. All notices that are required or permitted hereunder shall be in writing and shall be sufficient if personally delivered or sent by registered or certified mail, Federal Express or other international business delivery service. Any notices shall be deemed given upon the earlier of the date when received at, or the third day after the date when sent by registered or certified mail or the day after the date when sent by Federal Express or other international business delivery service to, the address set forth below, unless such address is changed by notice to the other Party:

If to Shire:

Shire Pharmaceuticals Ireland Ltd.
5 Riverwalk
Citywest Business Campus

Dublin 24
Ireland
Fax: 00 353 1 429 7701
Attention: Legal Department

with a copy to:

Morgan, Lewis & Bockius LLP
502 Carnegie Center
Princeton, NJ 08540
Fax: (609) 919-6701
Attention: Randall B. Sunberg, Esq.

If to Amicus:

Amicus Therapeutics, Inc.
6 Cedar Brook Drive
Cranbury, NJ 08512
Fax: (609) 662-2001
Attention: President

with a copy to:

Wilson Sonsini Goodrich & Rosati
650 Page Mill Road
Palo Alto, CA 94304
Fax: (650) 493-6811
Attention: Kenneth A. Clark, Esq.

16.11 Amendment. This Agreement may be amended or modified only by a writing signed by each of the Parties.

16.12 Entire Agreement. This Agreement and the Related Agreements between the Parties constitute the entire understanding between the Parties as of the Effective Date with respect to the subject matter hereof and thereof and supersede all related prior or contemporaneous oral communications, agreements or discussions with respect to the subject matter hereof or thereof.

16.13 Execution in Counterparts; Facsimile Signatures. This Agreement may be executed in two counterparts, each of which counterparts, when so executed and delivered, shall be deemed to be an original, and both of which counterparts, taken together, shall constitute one and the same instrument even if both Parties have not executed the same counterpart. Signatures provided by facsimile transmission shall be deemed to be original signatures.

16.14 Provisions of Existing In-Licenses. Pursuant to Section 2(d) of the Licensed Agreement between Amicus and Mount Sinai School of Medicine of New York University

("***MSSM***") dated April 15, 2002, as amended (the "***MSSM Agreement***"), (a) Shire agrees to be bound by Sections 6, 9 and 10 of the MSSM Agreement, the text of which is attached hereto as Exhibit 16.14 and incorporated herein by reference, to the extent applicable to Shire in its capacity as a sublicensee thereunder and (b) MSSM shall be deemed to be a third party beneficiary of this Agreement for purposes of enforcing Sections 9 and 10 of the MSSM Agreement against Shire in its capacity as a sublicensee thereunder. In addition, Shire, in its capacity as a sublicensee under the Existing In-Licenses, agrees to comply with the audit rights applicable to sublicensees thereunder.

(The remainder of this page is intentionally left blank; the signature page follows.)

[***] INDICATES MATERIAL THAT HAS BEEN OMITTED AND FOR WHICH CONFIDENTIAL TREATMENT HAS BEEN REQUESTED. ALL SUCH OMITTED MATERIAL HAS BEEN FILED WITH THE SECURITIES AND EXCHANGE COMMISSION PURSUANT TO RULE 24b-2 PROMULGATED UNDER THE SECURITIES EXCHANGE ACT OF 1934, AS AMENDED.

IN WITNESS WHEREOF, each of the Parties, by their duly authorized officers, have executed this Agreement as of the Effective Date.

AMICUS THERAPEUTICS, INC.	SHIRE PHARMACEUTICALS IRELAND LTD.
By: /s/ John F. Crowley	By: /s/ Susan Connell
Name: John F. Crowley	Name: Susan Connell
Title: President and Chief Executive Officer	Title: Director

[***] INDICATES MATERIAL THAT HAS BEEN OMITTED AND FOR WHICH CONFIDENTIAL TREATMENT HAS BEEN REQUESTED. ALL SUCH OMITTED MATERIAL HAS BEEN FILED WITH THE SECURITIES AND EXCHANGE COMMISSION PURSUANT TO RULE 24b-2 PROMULGATED UNDER THE SECURITIES EXCHANGE ACT OF 1934, AS AMENDED.

APPENDIX 1

Amicus Patent Rights

Application No.	Patent No.	Country	Title	Legal Owner	Beneficial Owner
09/087804	6274597	US	Method Of Enhancing Lyosomal Alpha-Galactosidase A	Mount Sinai School of Medicine	Amicus Therapeutics, Inc.
[***]					
09/604053	6583158	US	Method For Enhancing Mutant Enzyme Activities In Lysosomal Storage Disorders	Mount Sinai School of Medicine	Amicus Therapeutics, Inc.
09/927285	6774135	US	Method Of Enhancing Lysosomal Alpha-Galactosidase A	Mount Sinai School of Medicine	Amicus Therapeutics, Inc.
09/948348	6599919	US	Method For Enhancing Mutant Enzyme Activities In Lysosomal Storage Disorders	Mount Sinai School of Medicine	Amicus Therapeutics, Inc.
10/172604	6589964	US	Method for Enhancing Mutant Enzyme Activities In Lysosomal Storage Disorders	Mount Sinai School of Medicine	Amicus Therapeutics, Inc.
10/304395	6916829	US	Method For Enhancing Mutant Enzyme Activity In Gaucher Disease	Mount Sinai School of Medicine	Amicus Therapeutics, Inc.
[***]					
10/989258	7141582	US	Method Of Enhancing Mutant Enzyme Activity In Gaucher Disease	Mount Sinai School of Medicine	Amicus Therapeutics, Inc.

[***] INDICATES MATERIAL THAT HAS BEEN OMITTED AND FOR WHICH CONFIDENTIAL TREATMENT HAS BEEN REQUESTED. ALL SUCH OMITTED MATERIAL HAS BEEN FILED WITH THE SECURITIES AND EXCHANGE COMMISSION PURSUANT TO RULE 24b-2 PROMULGATED UNDER THE SECURITIES EXCHANGE ACT OF 1934, AS AMENDED.

Application No.	Patent No.	Country	Title	Legal Owner	Beneficial Owner
[***]					
[***]					
[***]					
95911229.3	0749423	CH	Piperidines And Pyrrolidines	Novo Nordisk A/S	Amicus Therapeutics, Inc.
69531098.4	0749423	DE	Piperidines And Pyrrolidines	Novo Nordisk A/S	Amicus Therapeutics, Inc.
95911229.3	0749423	EP	Piperidines And Pyrrolidines	Novo Nordisk A/S	Amicus Therapeutics, Inc.
95911229.3	0749423	FR	Piperidines And Pyrrolidines	Novo Nordisk A/S	Amicus Therapeutics, Inc.
95911229.3	0749423	GB	Piperidines And Pyrrolidines	Novo Nordisk A/S	Amicus Therapeutics, Inc.
95911229.3	0749423	SE	Piperidines And Pyrrolidines	Novo Nordisk A/S	Amicus Therapeutics, Inc.
7-523172		JP	Use Of Hydroxy Alkyl Piperidine And Pyrrolidine Compounds To Treat Diabetes	Novo Nordisk A/S	Amicus Therapeutics, Inc.
[***]					

[***] INDICATES MATERIAL THAT HAS BEEN OMITTED AND FOR WHICH CONFIDENTIAL TREATMENT HAS BEEN REQUESTED. ALL SUCH OMITTED MATERIAL HAS BEEN FILED WITH THE SECURITIES AND EXCHANGE COMMISSION PURSUANT TO RULE 24b-2 PROMULGATED UNDER THE SECURITIES EXCHANGE ACT OF 1934, AS AMENDED.

APPENDIX 2

Ex-U.S. Platform Patent Rights

Application No.	Patent No.	Country	Title	Legal Owner	Beneficial Owner
09/087804	6274597	US	Method Of Enhancing Lyosomal Alpha-Galactosidase A	Mount Sinai School of Medicine	Amicus Therapeutics, Inc.
[***]					
09/604053	6583158	US	Method For Enhancing Mutant Enzyme Activities In Lysosomal Storage Disorders	Mount Sinai School of Medicine	Amicus Therapeutics, Inc.
09/927285	6774135	US	Method Of Enhancing Lysosomal Alpha-Galactosidase A	Mount Sinai School of Medicine	Amicus Therapeutics, Inc.
09/948348	6599919	US	Method For Enhancing Mutant Enzyme Activities In Lysosomal Storage Disorders	Mount Sinai School of Medicine	Amicus Therapeutics, Inc.
10/172604	6589964	US	Method for Enhancing Mutant Enzyme Activities In Lysosomal Storage Disorders	Mount Sinai School of Medicine	Amicus Therapeutics, Inc.
10/304395	6916829	US	Method For Enhancing Mutant Enzyme Activity In Gaucher Disease	Mount Sinai School of Medicine	Amicus Therapeutics, Inc.
[***]					

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Application No.	Patent No.	Country	Title	Legal Owner	Beneficial Owner
10/989258	7141582	US	Method Of Enhancing Mutant Enzyme Activity In Gaucher Disease	Mount Sinai School of Medicine	Amicus Therapeutics, Inc.
[***]					
08/404077	5863903	US	Use Of Hydroxy Alkyl Piperidine And Pyrrolidine Compounds To Treat Diabetes	Novo Nordisk A/S	Amicus Therapeutics, Inc.
95911229.3	0749423	CH	Piperidines And Pyrrolidines	Novo Nordisk A/S	Amicus Therapeutics, Inc.
69531098.4	0749423	DE	Piperidines And Pyrrolidines	Novo Nordisk A/S	Amicus Therapeutics, Inc.
95911229.3	0749423	EP	Piperidines And Pyrrolidines	Novo Nordisk A/S	Amicus Therapeutics, Inc.
95911229.3	0749423	FR	Piperidines And Pyrrolidines	Novo Nordisk A/S	Amicus Therapeutics, Inc.
95911229.3	0749423	GB	Piperidines And Pyrrolidines	Novo Nordisk A/S	Amicus Therapeutics, Inc.
95911229.3	0749423	SE	Piperidines And Pyrrolidines	Novo Nordisk A/S	Amicus Therapeutics, Inc.

[***]

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APPENDIX 3

Amicus Competitors

The companies to be listed on this Appendix 3 pursuant to Sections 2.2.2(b) and 2.4.4 are as set forth in that certain letter from Douglas A. Branch to Gary Clements dated as of the Effective Date.

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APPENDIX 4

Initial Development Plan

[***]

[***] INDICATES MATERIAL THAT HAS BEEN OMITTED AND FOR WHICH CONFIDENTIAL TREATMENT HAS BEEN REQUESTED. ALL SUCH OMITTED MATERIAL HAS BEEN FILED WITH THE SECURITIES AND EXCHANGE COMMISSION PURSUANT TO RULE 24b-2 PROMULGATED UNDER THE SECURITIES EXCHANGE ACT OF 1934, AS AMENDED.

EXHIBIT 1.5.1

Deoxygalactonojirimycin

[***]

[***] INDICATES MATERIAL THAT HAS BEEN OMITTED AND FOR WHICH CONFIDENTIAL TREATMENT HAS BEEN REQUESTED. ALL SUCH OMITTED MATERIAL HAS BEEN FILED WITH THE SECURITIES AND EXCHANGE COMMISSION PURSUANT TO RULE 24b-2 PROMULGATED UNDER THE SECURITIES EXCHANGE ACT OF 1934, AS AMENDED.

EXHIBIT 1.5.2

Deoxynojirimycin

[***]

[***] INDICATES MATERIAL THAT HAS BEEN OMITTED AND FOR WHICH CONFIDENTIAL TREATMENT HAS BEEN REQUESTED. ALL SUCH OMITTED MATERIAL HAS BEEN FILED WITH THE SECURITIES AND EXCHANGE COMMISSION PURSUANT TO RULE 24b-2 PROMULGATED UNDER THE SECURITIES EXCHANGE ACT OF 1934, AS AMENDED.

EXHIBIT 1.5.3

Isofagomine

[***]

[***] INDICATES MATERIAL THAT HAS BEEN OMITTED AND FOR WHICH CONFIDENTIAL TREATMENT HAS BEEN REQUESTED. ALL SUCH OMITTED MATERIAL HAS BEEN FILED WITH THE SECURITIES AND EXCHANGE COMMISSION PURSUANT TO RULE 24b-2 PROMULGATED UNDER THE SECURITIES EXCHANGE ACT OF 1934, AS AMENDED.

EXHIBIT 6.4.4

The Back-up Compounds referenced in §6.6.4(c)(ii) are as set forth in Appendix A of that certain letter from Douglas A. Branch to Gary Clements dated as of the Effective Date.

[***] INDICATES MATERIAL THAT HAS BEEN OMITTED AND FOR WHICH CONFIDENTIAL TREATMENT HAS BEEN REQUESTED. ALL SUCH OMITTED MATERIAL HAS BEEN FILED WITH THE SECURITIES AND EXCHANGE COMMISSION PURSUANT TO RULE 24b-2 PROMULGATED UNDER THE SECURITIES EXCHANGE ACT OF 1934, AS AMENDED.

EXHIBIT 10.6.1

Existing In-Licenses

1) Agreement, dated as of April 15, 2002, as amended, by and between Amicus and Mount Sinai School of Medicine of New York University

2) Exclusive License Agreement, dated as of June 8, 2005, by and between Amicus and Novo Nordisk, A/S

[***] INDICATES MATERIAL THAT HAS BEEN OMITTED AND FOR WHICH CONFIDENTIAL TREATMENT HAS BEEN REQUESTED. ALL SUCH OMITTED MATERIAL HAS BEEN FILED WITH THE SECURITIES AND EXCHANGE COMMISSION PURSUANT TO RULE 24b-2 PROMULGATED UNDER THE SECURITIES EXCHANGE ACT OF 1934, AS AMENDED.

EXHIBIT 16.14

Sections 6, 9 and 10 of the MSSM Agreement

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.

[***] INDICATES MATERIAL THAT HAS BEEN OMITTED AND FOR WHICH CONFIDENTIAL TREATMENT HAS BEEN REQUESTED. ALL SUCH OMITTED MATERIAL HAS BEEN FILED WITH THE SECURITIES AND EXCHANGE COMMISSION PURSUANT TO RULE 24b-2 PROMULGATED UNDER THE SECURITIES EXCHANGE ACT OF 1934, AS AMENDED.

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.

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.

[***] INDICATES MATERIAL THAT HAS BEEN OMITTED AND FOR WHICH CONFIDENTIAL TREATMENT HAS BEEN REQUESTED. ALL SUCH OMITTED MATERIAL HAS BEEN FILED WITH THE SECURITIES AND EXCHANGE COMMISSION PURSUANT TO RULE 24b-2 PROMULGATED UNDER THE SECURITIES EXCHANGE ACT OF 1934, AS AMENDED.

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 7.3.4

U.S. Patent No. 6,344,475

U.S. Patent No. 6,270,954

U.S. Patent No. 6,541,195

U.S. Patent No. 5,900,360

Australian Patent No. AU 775 575 B2

Australian Patent No. AU 734 905 B2

[***]

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SCHEDULE 12.2

This Schedule 12.2 is made and given pursuant to Section 12.2 of the Agreement. The section numbers below correspond to the section numbers of the Agreement. Nothing in this Schedule 12.2 is intended to broaden the scope of any representation or warranty contained in the Agreement or to create any covenant. The information contained in this Schedule 12.2 is provided solely for purposes of making disclosures to Shire under the Agreement. In disclosing such information, Amicus does not waive any attorney-client privilege associated with such information or any protection afforded by the work-product doctrine with respect to any of the matters disclosed or discussed in this Schedule 12.2.

[***]

[***] INDICATES MATERIAL THAT HAS BEEN OMITTED AND FOR WHICH CONFIDENTIAL TREATMENT HAS BEEN REQUESTED. ALL SUCH OMITTED MATERIAL HAS BEEN FILED WITH THE SECURITIES AND EXCHANGE COMMISSION PURSUANT TO RULE 24b-2 PROMULGATED UNDER THE SECURITIES EXCHANGE ACT OF 1934, AS AMENDED.

Letter from Douglas A. Branch to Gary Clements dated as of the Effective Date

November 7, 2007

Mr. Gary Clements
Senior Business Development Director
Shire Human Genetic Therapies
700 Main Street
Cambridge, MA 02139

Re: License and Collaboration Agreement (the "Agreement") by and between Amicus Therapeutics, Inc. ("Amicus") and Shire Pharmaceuticals Ireland Ltd. ("Shire")

Dear Mr. Clements:

In connection with Sections 2.2.2(b) and 2.4.4 of the above-referenced Agreement, Appendix A to this letter sets forth the companies to be listed on Appendix 3 of the Agreement.

Very truly yours,

/s/ Douglas Branch

Douglas A. Branch

Vice President and General Counsel

Acknowledged by:

/s/ Gary Clements

Mr. Gary Clements

[***] INDICATES MATERIAL THAT HAS BEEN OMITTED AND FOR WHICH CONFIDENTIAL TREATMENT HAS BEEN REQUESTED. ALL SUCH OMITTED MATERIAL HAS BEEN FILED WITH THE SECURITIES AND EXCHANGE COMMISSION PURSUANT TO RULE 24b-2 PROMULGATED UNDER THE SECURITIES EXCHANGE ACT OF 1934, AS AMENDED.

APPENDIX A

[***]

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[***] INDICATES MATERIAL THAT HAS BEEN OMITTED AND FOR WHICH CONFIDENTIAL TREATMENT HAS BEEN REQUESTED. ALL SUCH OMITTED MATERIAL HAS BEEN FILED WITH THE SECURITIES AND EXCHANGE COMMISSION PURSUANT TO RULE 24b-2 PROMULGATED UNDER THE SECURITIES EXCHANGE ACT OF 1934, AS AMENDED.

November 7, 2007

Mr. Gary Clements
Senior Business Development Director
Shire Human Genetic Therapies
700 Main Street
Cambridge, MA 02139

Re: License and Collaboration Agreement (the "Agreement") by and between Amicus Therapeutics, Inc. ("Amicus") and Shire Pharmaceuticals Ireland Ltd. ("Shire")

Dear Mr. Clements:

As required by Section 6.4.4(a)(ii) of the above-referenced Agreement, Appendix A to this letter contains the list of Back-Up Compounds referenced therein.

Very truly yours,

/s/ Douglas Branch

Douglas A. Branch
Vice President and General Counsel

Acknowledged by:

/s/ Gary Clements

Mr. Gary Clements

[***] INDICATES MATERIAL THAT HAS BEEN OMITTED AND FOR WHICH CONFIDENTIAL TREATMENT HAS BEEN REQUESTED. ALL SUCH OMITTED MATERIAL HAS BEEN FILED WITH THE SECURITIES AND EXCHANGE COMMISSION PURSUANT TO RULE 24b-2 PROMULGATED UNDER THE SECURITIES EXCHANGE ACT OF 1934, AS AMENDED.

APPENDIX A

[***]

[***] INDICATES MATERIAL THAT HAS BEEN OMITTED AND FOR WHICH CONFIDENTIAL TREATMENT HAS BEEN REQUESTED. ALL SUCH OMITTED MATERIAL HAS BEEN FILED WITH THE SECURITIES AND EXCHANGE COMMISSION PURSUANT TO RULE 24b-2 PROMULGATED UNDER THE SECURITIES EXCHANGE ACT OF 1934, AS AMENDED.

EXHIBIT 23.1

CONSENT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

We consent to the incorporation by reference in the Registration Statement (Form S-8 No. 333-141700) 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, and 4) Amicus Therapeutics, Inc. 2007 Employee Stock Purchase Plan of our report dated February 5, 2008, with respect to the consolidated financial statements of Amicus Therapeutics, Inc., included in this Annual Report (Form 10-K) for the year ended December 31, 2007.

/s/ ERNST & YOUNG LLP

MetroPark, New Jersey
February 7, 2008

EXHIBIT 31.1

CERTIFICATIONS PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002
CERTIFICATION BY CHIEF 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)) 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. 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

c. 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: February 8, 2008

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

EXHIBIT 31.2

CERTIFICATIONS PURSUANT TO SECTION 302 OF
THE SARBANES-OXLEY ACT OF 2002
CERTIFICATION BY CHIEF FINANCIAL OFFICER

I, James E. Dentzer, 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)) 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. 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

c. 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: February 8, 2008

/s/ James E. Dentzer
James E. Dentzer
Chief Financial Officer

EXHIBIT 32.1

Certification by the Chief 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, 2007 (the "Report"), as filed with the Securities and Exchange Commission on February 8, 2008, 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
John F. Crowley
Chief Executive Officer
February 8, 2008

EXHIBIT 32.2

Certification by the Chief 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, James E. Dentzer, 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, 2007 (the "Report"), as filed with the Securities and Exchange Commission on February 8, 2008, 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/ James E. Dentzer
James E. Dentzer
Chief Financial Officer
February 8, 2008