AMICUS THERAPEUTICS INC

FORM 10-K
(Annual Report)

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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, 2014
OR

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

For the transition period from to

Commission File Number 001-33497

Amicus Therapeutics, Inc.

(Exact name of registrant as specified in its charter)

Delaware	**71-0869350**
(State or other jurisdiction of incorporation or organization)	*(IRS Employer Identification No.)*

1 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 ☐ No ☒

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

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

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

Indicate by check mark if disclosure of delinquent filers pursuant to Item 405 of Regulation S-K (§22.405) is not contained herein, and will not be contained, to the best of the registrant's knowledge, in definitive proxy or information statements incorporated by reference in Part III of this Form 10-K or any amendment to this Form 10-K. ☐

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

Large accelerated filer ☐	Accelerated filer ☒	Non-accelerated filer ☐ (Do not check if a smaller reporting company)	Smaller Reporting Company ☐

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

The aggregate market value of the 33,307,124 shares of voting common equity held by non-affiliates of the registrant, computed by reference to the closing price as reported on the NASDAQ, as of the last business day of the registrant's most recently completed second fiscal quarter (June 30, 2014) was approximately $111,245,794. Shares of voting and non-voting stock held by executive officers, directors and holders of more than 10% of the outstanding stock have been excluded from this calculation because such persons or institutions may be deemed affiliates. This determination of affiliate status is not a conclusive determination for other purposes.

As of February 24, 2015, there were 95,624,073 shares of common stock outstanding.

DOCUMENTS INCORPORATED BY REFERENCE: Portions of the Proxy Statement for the registrant's 2015 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.

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," "potential," "intend," "may," "plan," "predict," "project," "will," "should," "would" and similar expressions are intended to identify forward-looking statements, although not all forward- looking statements contain these identifying words.

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

- the progress and results of our clinical trials of our drug candidates, including migalastat HCl ("migalastat");
- the cost of manufacturing drug supply for our clinical and preclinical studies, including the significant cost of new enzyme replacement therapy ("ERT") cell line development and manufacturing as well as the cost of manufacturing Pompe ERT;
- the scope, progress, results and costs of preclinical development, laboratory testing and clinical trials for our product candidates including those testing the use of pharmacological chaperones co-formulated and co-administered with ERT and for the treatment of lysosomal storage diseases ("LSDs");
- the costs, timing and outcome of regulatory review of our product candidates;
- the number and development requirements of other product candidates that we pursue;
- the costs of commercialization activities, including product marketing, sales and distribution;
- the emergence of competing technologies and other adverse market developments;
- the costs of preparing, filing and prosecuting patent applications and maintaining, enforcing and defending intellectual property related claims;
- the extent to which we acquire or invest in businesses, products and technologies; and
- our ability to establish collaborations and obtain milestone, royalty or other payments from any such collaborators.

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

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

PART I

Item 1. *BUSINESS.*

Overview

We are a biopharmaceutical company focused on the discovery, development and commercialization of next-generation medicines for a range of rare and orphan diseases, with a focus on improved therapies for lysosomal storage disorders ("LSDs"). Our lead product candidate is a small molecule that can be used as a monotherapy and in combination with enzyme replacement therapy ("ERT") for Fabry disease. Our development programs also include next-generation ERTs for LSDs, including Fabry disease, Pompe disease and Mucopolysaccharidosis Type I ("MPS I"). We believe that our platform technologies and our advanced product pipeline uniquely position us at the forefront of developing therapies for rare and orphan diseases.

In LSDs such as Fabry and Pompe, a mutation in the specific disease-causing gene can lead to the production in the body of a mutant form of the enzyme that is less stable than the normal form, and that may be prematurely degraded before reaching the location in the cell where it is needed. For patients with LSDs who are receiving ERT, the infused (exogenous) protein may also unfold and lose activity at any stage in the process — from the infusion bag to the bloodstream, to the eventual uptake into cells and tissue. The result is a loss of enzyme activity and disruption of proper trafficking of the enzyme to lysosomes. Our novel treatment approach consists of using pharmacological chaperones that are designed to selectively bind and stabilize either the endogenous or exogenous target proteins and facilitate trafficking to the location in cells where these proteins are needed (the lysosome).

Our personalized medicine approach consists of an oral small molecule pharmacological chaperone monotherapy that is designed to bind to and stabilize a patient's own endogenous target protein. Patients with "amenable mutations" may respond based on their genetics.

Our Chaperone-Advanced Replacement Therapy, or CHART™, platform combines chaperones with ERTs independent of a patient's own genetics. In each CHART program, a unique pharmacological chaperone is designed to bind to a specific therapeutic (exogenous) enzyme, stabilizing the enzyme in its properly folded and active form. This may allow for enhanced tissue uptake, greater lysosomal activity, more reduction of substrate, and the potential for lower immunogenicity.

Our Fabry franchise strategy is to develop the pharmacological chaperone migalastat HCl ("migalastat") for all patients with Fabry disease — as a monotherapy for patients with amenable mutations and in combination with ERT for all other patients. We have completed two Phase 3 global registration studies (Study 011 and Study 012) of migalastat monotherapy and plan to submit marketing applications in 2015. We have reported Phase 3 data in both treatment-naïve patients ("Study 011" or "FACETS") and ERT switch patients ("Study 012" or "ATTRACT"). Positive results from these studies have shown that treatment with migalastat results in reductions in disease substrate, stability of kidney function, reductions in cardiac mass, and improvement in gastrointestinal symptoms in patients with amenable mutations in a validated assay ("GLP HEK assay"). Data from the Fabry Registry indicate that the leading cause of death in patients is from cardiovascular disease (source: Mehta 2009).

Following a meeting with the European Medicines Agency ("EMA") held in the fourth quarter of 2014, we are on track to submit a marketing application in Europe in mid-2015. We have also scheduled a meeting with the U.S. Food and Drug Administration ("FDA") in the first quarter of 2015 as we work to make migalastat available for all amenable Fabry patients as quickly as possible.

We expect to initiate a longer-term Phase 2 Fabry co-administration study in 2015 in support of our Fabry franchise strategy to develop migalastat in combination with ERT for Fabry patients with non-amenable mutations. Preliminary results from our previously completed open-label Phase 2 safety and pharmacokinetics study ("Study 013") in Fabry patients showed increased levels of active alpha-Gal

A enzyme levels in plasma and in skin following co-administration with migalastat compared to ERT alone.

We are leveraging our biologics capabilities and CHART™ to develop a next-generation Pompe ERT. This ERT consists of a uniquely engineered recombinant human acid alpha-glucosidase ("rhGAA") enzyme (designated "ATB200") with an optimized carbohydrate structure to enhance uptake, administered in combination with a pharmacological chaperone to improve activity and stability. Acid alpha-glucosidase ("GAA") is the enzyme deficient in Pompe patients. In preclinical studies, ATB200 demonstrated greater tissue enzyme levels and substrate ("glycogen") reduction compared to the current approved ERT for Pompe disease ("alglucosidase alfa"), as well as further improvement when ATB200 was administered in combination with a chaperone. Clinical studies of pharmacological chaperones in combination with currently marketed ERTs recently established initial human proof-of-concept that a chaperone can stabilize enzyme activity and potentially improve ERT tolerability.

Additional preclinical CHART programs include a next-generation ERT for MPS I. In addition, our enzyme targeting technology is applicable to multiple ERTs and complementary to our CHART platform for the development of next-generation therapies for multiple LSDs. We believe that together these platform technologies may provide a unique tool set to address some of the major challenges with currently marketed ERT products — sub optimal enzyme activity and stability; poor targeting and uptake; and tolerability and immunogenicity.

Although LSDs are relatively rare diseases, they represent a substantial commercial opportunity due to the severity of the symptoms and the chronic nature of the diseases. The publicly reported worldwide net product sales for currently approved treatments for three LSDs were approximately $1.9 billion in 2014.

Our Pharmacological Chaperone Technology

We are leveraging our pharmacological chaperone technology to develop next-generation treatments for human genetic diseases by targeting mutated proteins that are unstable, unfolded or misfolded. In the human body, proteins are involved in almost every aspect of cellular function. Proteins are linear strings of amino acids that fold and twist into specific three-dimensional shapes in order to function properly. Certain human diseases result from mutations that cause changes in the amino acid sequence of a protein, and these changes often reduce protein stability and may prevent them from folding properly.

Pharmacological chaperones are small molecules designed to selectively bind to a target protein, increase its stability and help keep it folded in the correct three-dimensional shape. For LSDs, pharmacological chaperones are designed to bind to, and facilitate trafficking of, both endogenous and exogenous enzymes to the location in cells where they are needed (the lysosome). This important feature has allowed us to develop pharmacological chaperones as monotherapy agents (to be used without ERT) and our CHART platform of pharmacological chaperones in combination with ERT.

Pharmacological Chaperone Monotherapy

Many natural (endogenous) proteins are made in the endoplasmic reticulum ("ER") and sent to other parts of the cell. Unstable, unfolded or misfolded proteins are generally eliminated or retained in the ER rather than being transported to the intended destination in the cell. The accumulation of unfolded or misfolded proteins in the ER and the interruption of trafficking of important proteins to their proper cellular locations can cause several types of problems including:

- complete or partial loss of appropriate protein function,
- accumulation of lipids and other substances that should be degraded, and

- disruption of cellular function and eventual cell death.

These defects may lead to various types of human genetic diseases, including LSDs. As monotherapy agents for LSDs, pharmacological chaperones are designed to bind to and stabilize endogenous lysosomal enzymes for proper trafficking to the lysosome, which may also alleviate the build-up of mutant proteins in the ER. Once in the lysosome, the pharmacological chaperone disassociates and the enzyme is free to break down substrate. Based on this mechanism, individuals with genetic mutations that result in some residual biological activity are potentially eligible for pharmacological chaperone monotherapy.

CHART Technology Platform

ERT is the standard of care for several LSDs, based on the intravenous infusion of recombinant or gene-activated human enzyme. The enzyme is delivered into the blood in order to be taken up by cells and then transported to the lysosome. Upon entering the lysosome, this enzyme is intended to perform the function of the absent or deficient endogenous enzyme. However, the pH in the infusion bag and in blood is higher than the enzyme's natural acidic environment in the lysosome. As a result, the infused enzyme may rapidly unfold and lose activity and may be misdirected to non-target tissues or rapidly cleared from the body. Exposure to high concentrations of infused enzymes can impact efficacy or cause adverse effects.

Possible problems related to the in stability of infused enzyme include:

- denaturation and reduced activity;
- poor targeting and uptake into key tissues of disease; and
- poor tolerability and increased immunogenicity.

In our CHART programs, each chaperone is designed to bind to and stabilize a specific therapeutic enzyme. We believe this technology may be able to improve the stability, uptake and activity of the enzyme, and may improve tolerability and lower immunogenicity compared to administration of currently marketed ERTs alone. This combination approach may benefit patients with LSDs, including patients with inactive endogenous proteins who are not amenable to chaperone monotherapy.

Enzyme Targeting Technology

The uptake of ERTs into patient's cells is mediated by a particular carbohydrate called mannose 6-phosphate ("M6P"). M6P enables binding and delivery of therapeutic drug to lysosomes via M6P receptors on cell surfaces. Many currently approved ERTs have limited amounts of M6P thereby limiting the uptake of therapeutic drug into a patient's cells.

We are developing next-generation ERTs with significantly higher amounts of M6P for improved lysosomal targeting compared to existing ERTs. We believe that this technology to enhance drug targeting, together with our CHART platform to improve enzyme stability, may be utilized to develop a pipeline of more effective next-generation ERTs for LSDs.

Migalastat for Fabry Disease

Overview

Our most advanced product candidate, migalastat, is an investigational, small molecule pharmacological chaperone for the treatment of Fabry disease. As an orally administered monotherapy, migalastat is designed to bind to and stabilize, or "chaperone" a endogenous own alpha-Gal A enzyme in those patients with genetic mutations identified as amenable to this chaperone in a cell-based assay.

For all other Fabry patients, migalastat in combination with ERT may improve outcomes by keeping infused alpha-Gal A enzyme in its properly folded and active form.

Migalastat for Fabry Disease as a Monotherapy: Phase 3 Global Registration Program

Study 011 was a 24-month study of Fabry disease patients naïve to or not receiving ERT, which investigated the safety and efficacy of oral migalastat, (150 mg every other day). The study consisted of a 6-month double-blind, placebo-controlled period, a 6-month open-label period, and a 12-month open-label extension phase. Subjects completing Study 011 were eligible to continue treatment with migalastat in a long-term open-label extension ("Study 041"). 67 subjects (24 male) were enrolled. All subjects enrolled in Study 011 had amenable mutations in the clinical trial human embryonic kidney ("HEK") cell-based in vitro assay that was available at study initiation ("clinical trial assay"). Following the completion of enrollment, a GLP-validated HEK assay was developed with a third party to measure the criteria for amenability with more quality control and rigor ("GLP HEK assay"). Approximately 10% of mutations in the HEK database switched categorization between "amenable" and "non-amenable" when moving from the clinical trial assay to the GLP HEK assay. Therefore, there were changes in categorization from amenable to non-amenable in 17 of the 67 patients enrolled in Study 011.

Study 011 was designed to measure the reduction of the disease substrate (Globotriaosylceramide, or "GL-3") in the interstitial capillaries of the kidney following treatment with oral migalastat. The 24-month study began with a 6-month double-blind, placebo-controlled treatment period, after which all patients were treated with migalastat for a 6-month open-label follow-up period, and a subsequent 12-month open-label extension phase. The study also measured clinical outcomes, including renal function, as secondary endpoints.

As previously reported, patients on migalastat experienced greater reductions in GL-3 as compared to placebo during the initial 6-month period; however, this difference was not statistically significant under the original analysis of the primary endpoint (responder analysis with a 50% reduction threshold at month 6). The variability and low levels of GL-3 at baseline contributed to a higher-than-anticipated placebo response at month 6.

Following the unblinding of the 6-month data, and while still blinded to the 12-month data, we reported the mean change in GL-3 from the baseline to month 6 as a post-hoc analysis in the subgroup of patients with GLP HEK-amenable mutations. This analysis showed a statistically significant reduction in GL-3 in the migalastat group compared to placebo. The mean change in GL-3 was identified as a more appropriate way to control for the variability in GL-3 levels in Study 011 and to measure the biological effect of migalastat.

Results from this subgroup analysis further support use of the GLP HEK assay in predicting responsiveness to migalastat. Following a Type C Meeting with the FDA, we revised the Statistical Analysis Plan to pre-specify the primary analysis at month 12 as the mean change in interstitial capillary GL-3 in patients with GLP HEK amenable mutations.

Throughout 2014 and in early 2015 we announced positive 12- and 24-month data from Study 011 and longer-term data from Study 041 in patients with amenable mutations who were naïve to ERT. Top-line data were announced in April 2014 and data were presented to the scientific community at the American Society of Human Genetics (ASHG) in October 2014 and WORLDSymposium™ in February 2015. Highlights were as follows:

- Subjects who switched from placebo to migalastat after month 6 demonstrated a statistically significant reduction in disease substrate, or kidney interstitial capillary GL-3, at month 12 (p=0.013), and a statistically significant reduction of disease substrate in another important biomarker of disease, plasma lyso-Gb3. Subjects who remained on migalastat demonstrated a

durable reduction in kidney interstitial capillary GL-3, as well as a durable reduction in lyso-Gb3.

- Kidney function, as measured by estimated glomerular filtration rate (eGFR) and iohexol measured GFR (mGFR), remained stable following 18-24 months of treatment with migalastat in Study 011. Kidney function, as measured by eGFR, continued to remain stable in patients receiving migalastat in Study 011 for at least 18 months and continuing migalastat treatment in Study 041 for an average of 32 months. mGFR was not collected in Study 041.

- Reduction in cardiac mass, as measured by left ventricular mass index (LVMi), was statistically significant following treatment with migalastat for up to 36 months (average of 22 months) in patients in Study 011 and 041.

- There was a significant decrease in diarrhea (unadjusted p=0.03) in patients treated with migalastat versus placebo during the 6-month double-blind phase (Stage 1). After 18-24 months of treatment with migalastat, significant improvements in diarrhea and indigestion were observed in addition to favorable trends in reflux and constipation. Gastrointestinal symptoms were assessed using the Gastrointestinal Symptoms Rating Scale (GSRS), a validated instrument

- Migalastat was generally safe and well-tolerated

Study 012, our second Phase 3 registration study, is a randomized, open-label 18-month study investigating the safety and efficacy of oral migalastat (150 mg, every other day) compared to standard-of-care infused ERTs (agalsidase beta and agalsidase beta). The study also includes a 12-month open-label migalastat extension phase. The study enrolled a total of 60 patients (males and females) with Fabry disease and genetic mutations identified as amenable to migalastat monotherapy in a clinical trial assay. Subjects were randomized 1.5:1 to switch to migalastat or remain on ERT. All subjects had been receiving ERT infusions for a minimum of 12 months (at least 3 months at the labeled dose) prior to entering the study. Based on the GLP HEK assay, there were changes in categorization from amenable to non-amenable in 4 of the 60 patients enrolled in Study 012.

Taking into account scientific advice from European regulatory authorities, the pre-specified co-primary outcome measures of efficacy in Study 012 are the descriptive assessments of comparability of the mean annualized change in mGFR and eGFR for migalastat and ERT. Both mGFR and eGFR are considered important measures of renal function. Success on mGFR and eGFR was prescribed to be measured in two ways: 1) a 50% overlap in the confidence intervals between the migalastat and ERT treatment groups; and 2) whether the mean annualized changes for patients receiving migalastat are within 2.2 mL/min/1.73 m2/yr of patients receiving ERT. We pre-specified that these renal function outcomes would be analyzed in patients with GLP HEK amenable mutations.

In August 2014, we announced positive 18-month data from the Study 012. Data from Study 012 were also presented to the scientific community at the American Society of Nephrology ("ASN") in November 2014 and WORLDSymposium in February 2015. Highlights were as follows:

- Migalastat had a comparable effect to ERT on patients' kidney function as measured by the change in eGFR and mGFR from baseline to month 18.

- Levels of plasma lyso-Gb3, an important biomarker of disease, remained low and stable in patients with amenable mutations who switched from ERT to migalastat.

- There was a statistically significant decrease in LVMi from baseline to month 18 in patients who switched from ERT to migalastat

- Measures of pain and quality of life from the Brief Pain Inventory ("BPI") and Short Form 36 ("SF36") remained stable when patients switched from ERT to migalastat.

- Migalastat was generally safe and well-tolerated.

Following a meeting with the European Medicines Agency ("EMA") held in the fourth quarter of 2014, we are on track to submit a marketing application in Europe in mid-2015. We have also scheduled a meeting with the US FDA in the first quarter of 2015 as we work to make migalastat available for all amenable Fabry patients as quickly as possible.

Migalastat Combination Programs for Fabry Disease

In support of our Fabry Franchise strategy to develop migalastat in combination with ERT for Fabry patients with non-amenable mutations, we plan to conduct a longer-term Phase 2 Fabry co-administration study in 2015. In parallel, we are internally developing our own Fabry cell line for co-formulation with migalastat as a next-generation ERT for Fabry disease. We previously completed an open-label Phase 2 safety and pharmacokinetics study ("Study 013") that investigated two oral doses of migalastat (150 mg and 450 mg) co-administered with agalsidase beta or agalsidase alfa in males with Fabry disease. Unlike Study 011 and Study 012, patients in Study 013 were not required to have alpha-Gal mutations amenable to chaperone therapy because, when co-administered with ERT, migalastat is designed to bind to and stabilize the exogenous enzyme in the circulation in any patient receiving ERT. Each patient received their current dose and regimen of ERT at one infusion. A single oral dose of migalastat (150 mg or 450 mg) was co-administered two hours prior to the next infusion of the same ERT at the same dose and regimen. Preliminary results from Study 013 showed increased levels of active alpha-Gal enzyme levels in plasma and skin following co-administration compared to ERT alone.

Causes of Fabry Disease and Rationale for Use of Migalastat

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

Migalastat monotherapy is designed to act as a pharmacological chaperone for alpha-Gal A by selectively binding to the enzyme, which increases its stability and helps the enzyme fold into its correct three-dimensional shape. This stabilization of alpha-Gal A allows the cell's quality control mechanisms to recognize the enzyme as properly folded so that trafficking of the enzyme to the lysosome is increased, enabling it to carry out its intended biological function, the metabolism of GL-3.

Because migalastat increases levels of a patient's naturally produced alpha-Gal A, Fabry disease patients most likely to respond to treatment with migalastat monotherapy are those with a missense mutation or other genetic mutations that result in production of alpha-Gal that is less stable but that maintains some residual catalytic activity. We estimate that approximately thirty to fifty percent of patients with Fabry disease may have alpha-Gal A mutations that are amenable to migalastat as a monotherapy. Patients with genetic mutations leading to a partially made alpha-Gal A enzyme or alpha-Gal A enzyme with an irreversible loss of activity are less likely to respond to treatment with

migalastat as a monotherapy. However, we believe that all Fabry patients are potentially treatable with migalastat in combination with ERT.

The combination of migalastat and ERT allows binding and stabilization of infused enzyme in the circulation as patients receive ERT. We believe migalastat in combination with ERT may be able to improve the stability, activity, uptake and tolerability of the therapeutic enzyme. This combination approach may benefit patients with inactive endogenous proteins who are not amenable to chaperone monotherapy.

Fabry Disease Background

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

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

Classic Fabry Disease

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

Later-Onset Fabry Disease

Individuals with later-onset Fabry disease can be male or female. They typically first experience disease symptoms in adulthood, and often have disease symptoms focused on a single organ. For example, many males and females with later-onset Fabry disease have enlargement of the left ventricle of the heart. As the patients advance in age, the cardiac complications of the disease progress and can lead to death. Studies reported in Circulation and Journal of the American Heart Association (March 2002 and August 2004, respectively), 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 study reported in The Lancet (November 2005) found that approximately 4% of 721 male and female patients in Germany between the ages of 18 to 55 with stroke of unknown cause have Fabry disease.

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

Newborn screening studies in Italy, Taiwan and Austria, published in the American Journal of Human Genetics (2006), Human Mutation (2009) and the Lancet (2011) respectively, report that the incidence of Fabry mutations in newborns is over ten times higher than previous estimates for classic patients, Combined these studies screened over 263 thousand newborns, and found the incidence of Fabry mutations to be between 1:2,400 to 1: 3,859. This high incidence was attributed to a large number of newborn males with alpha-Gal A mutations often associated with later-onset Fabry disease, which may not have been identified in previous screening studies that relied on diagnosis based on development of symptoms of classic Fabry disease.

Fabry Disease Market Opportunity

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

We expect that as awareness of later-onset Fabry disease grows, the number of patients diagnosed with the disease will increase. Increased awareness of all forms of Fabry disease, particularly for specialists not accustomed to treating Fabry disease patients, may lead to increased testing and diagnosis of patients with the disease

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 from recent newborn screening studies published in the American Journal of Human Genetics (2006), Human Mutation (2009) and the Lancet (2011) suggest that the vast majority of newly diagnosed patients with later-onset Fabry disease also have missense mutations. Because missense mutations often result in less stable, misfolded alpha-Gal A with some residual enzyme activity, we believe patients with these mutations may benefit from treatment with monotherapy migalastat. We also believe that many other types of genetic mutations may result in misfolded alpha-Gal A and therefore may also respond to treatment with monotherapy migalastat. Based on this, we believe that approximately thirty to fifty percent of the Fabry disease patient population may benefit from treatment with migalastat as a

monotherapy. However, the entire Fabry disease patient population has the potential to benefit from migalastat in combination with ERT.

Existing Products for the Treatment of Fabry Disease and Potential Advantages of Migalastat

Currently, two ERT products are approved for the treatment of Fabry disease: agalsidase beta and agalsidase alfa. Agalsidase beta is approved globally (conditionally in the U.S.) and commercialized by sanofi aventis through Genzyme Corporation, while agalsidase alfa commercialized by Shire and approved in the EU and other countries but not in the U.S. Orphan drug exclusivity for both agalsidase beta and agalsidase alfa has expired in the EU and for agalsidase beta , in the U.S. as well. The net product sales of agalsidase beta and agalsidase alfa for 2014 were approximately $610 million as publicly reported by sanofi aventis and $500 million as publicly reported by Shire, respectively.

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

For Fabry disease patients who respond to migalastat, we believe that the use of migalastat may have advantages relative to the use of agalsidase beta and agalsidase alfa. Published data for patients treated with agalsidase beta and agalsidase alfa 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, agalsidase beta and agalsidase alfa are believed to have difficulty penetrating some tissues and cell types. In particular, it is widely believed that agalsidase beta and agalsidase alfa 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, migalastat has the potential to reach cells of all the target tissues of Fabry disease. Furthermore, treatment with agalsidase beta and agalsidase alfa requires intravenous infusions every other week, frequently on-site at health care facilities, presenting an inconvenience to Fabry patients. Hence, oral treatment with migalastat may be much more convenient for patients. Lastly, agalsidase beta and agalsidase alfa are protein therapeutics, and have been shown to lead to the generation of anti-drug antibodies in some patients, which can affect efficacy. Some patients also experience infusion-associated reactions that can last for hours or days. In contrast, migalastat is not expected to have immunogenic effects, and may not have the safety risks associated with intravenous infusion.

In addition, as discussed above, we believe that migalastat in combination with ERT may improve key characteristics of the infused enzymes used in ERT by allowing for increased transport of active enzymes to the lysosomes and degradation of substrate, thereby potentially increasing ERT's efficacy. Importantly, patients who may not have alpha-Gal A mutations amenable to migalastat monotherapy treatment may benefit from migalastat in combination with ERT, making migalastat potentially available to all Fabry patients.

Next-Generation ERT for Pompe Disease

We are leveraging our biologics capabilities and CHART platform to develop a next-generation Pompe ERT. This ERT consists of a uniquely engineered rhGAA enzyme (ATB200) with an optimized carbohydrate structure to enhance uptake, administered in combination with a pharmacological chaperone to improve activity and stability. We acquired ATB200 as well as our enzyme targeting technology through our purchase of Callidus Biopharma.

In preclinical studies, ATB200 demonstrated greater tissue enzyme levels and further substrate reduction compared to the current approved ERT for Pompe disease (alglucosidase alfa), which were

further improved with the addition of a chaperone. Clinical studies of pharmacological chaperones in combination with currently marketed ERTs have established initial human proof-of-concept that a chaperone can stabilize enzyme activity and potentially improve ERT tolerability. In 2013, we completed a Phase 2 safety and pharmacokinetics study ("Study 010") that investigated single ascending oral doses of a pharmacological chaperone co-administered with alglucosidase alfa or recombinant human GAA enzyme, rhGAA marketed by Genzyme, in patients with Pompe disease. Each patient received one infusion of ERT alone, and then a single oral dose of the pharmacological chaperone just prior to the next ERT infusion. Results from this study showed an increase in GAA enzyme activity in plasma and muscle when co-administered compared to ERT alone.

Taken together, these preclinical and clinical results support further development of ATB200 in combination with a pharmacological chaperone as a next-generation Pompe ERT. The initiation of a Phase 1/2 clinical study is expected in the second half of 2015.

Pompe Disease Background

Like Fabry disease, Pompe disease is an LSD that results from a deficiency in an enzyme, GAA. Signs and symptoms of Pompe can be severe and debilitating and include progressive muscle weakness throughout the body, particularly the heart and skeletal muscles. The enzyme deficiencies in Pompe patients are caused by inherited genetic mutations. Certain of these mutations cause changes in the amino acid sequence of the enzyme that may result in the production of an enzyme with reduced stability that does not fold into its correct three-dimensional shape. Although the enzymes produced in patient cells often retain the potential for some level of biological activity, the cell's quality control mechanisms recognize and retain the misfolded enzyme in the ER until it is ultimately moved to another part of the cell for degradation and elimination. Consequently, little or no GAA in Pompe patients traffics to the lysosome, where it normally breaks down its substrate, a complex sugar called glycogen. This leads to accumulation of glycogen in cells, which is believed to result in the clinical manifestations of Pompe disease. 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 early 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. 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.

Acquisition of Callidus

In November 2013, we entered into a merger agreement with Callidus, a privately held biotechnology company which was engaged in developing a next-generation Pompe ERT and complementary enzyme targeting technologies.

In connection with our acquisition of Callidus, we agreed to issue an aggregate of 7.2 million shares of our common stock to the former stockholders of Callidus. In addition, we will be obligated to make additional payments to the former stockholders of Callidus upon the achievement of certain clinical milestones of up to $35 million and regulatory milestones of up to $105 million set forth in the merger agreement, provided that the aggregate merger consideration shall not exceed $130 million. We may, at our election, satisfy certain milestone payments identified in the merger agreement aggregating $40 million in shares of our common stock. The milestone payments not permitted to be satisfied in common stock (as well as any payments that we are permitted to, but chooses not to, satisfy in common stock), as a result of the terms of the merger agreement, will be paid in cash.

Strategic Alliances and Arrangements

In November 2013, we entered into the Revised Agreement (the Revised Agreement) with GSK, pursuant to which we obtained global rights to develop and commercialize migalastat as a monotherapy and in combination with ERT for Fabry disease. The Revised Agreement amends and replaces in its entirety the Expanded Agreement entered into between us and GSK in July 2012. Under the terms of the Revised Agreement, for migalastat monotherapy, GSK is eligible to receive post-approval and sales-based milestones, as well as tiered royalties in the mid-teens in eight major markets outside the U.S. There was no other consideration paid to GSK as part of the Revised Agreement.

In September 2013, we entered into a collaboration agreement with Biogen to discover, develop and commercialize novel small molecules that target the GCase enzyme for the treatment of Parkinson's disease. In September 2014, we concluded our research collaboration with Biogen. Our most advanced Parkinson's candidate is AT3375, which was developed outside the collaboration and is wholly-owned by us.

We will continue to evaluate other business development opportunities as appropriate that build stockholder value and provide us with access to the financial, technical, clinical and commercial resources necessary to develop and market pharmacological chaperone therapeutics and other technologies or products. We are exploring potential collaborations, alliances and other business development opportunities on a regular basis. These opportunities may include the acquisition of preclinical-stage, clinical-stage or marketed products so long as such transactions are consistent with our strategic plan to develop and provide therapies to patients living with rare and orphan diseases, and support our continued transformation from a development stage company into a commercial biotechnology company.

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 replacement enzymes and small molecules for 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 migalastat, pharmacological chaperone and ERT combination therapy are described below and include both patents and patent applications we own or exclusively license:

- We have an exclusive license to six issued U.S. patents that cover use of migalastat to treat Fabry disease, as well as corresponding European, Japanese and Canadian patents. These exclusively licensed U.S. patents relating to migalastat expire in 2018 (not including the Hatch-Waxman statutory extension, which is described below), while the European, Japanese and Canadian patents will expire in 2019 (not including the Supplemental Protection Certificates or SPC extensions, which are described below). The patents 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 migalastat . In addition, we own pending U.S. applications directed to dosing regimens with migalastat , which, if granted, may result in patents that expire in 2027. Further, we own an issued U.S. patent directed to synthetic steps related to the commercial process for preparing migalastat, which expires in 2026, as well as issued patents in China, Hong Kong and Japan. Foreign counterpart applications are pending in Brazil, Europe, Israel and India. We jointly own one issued U.S. patent and one issued Mexican patent covering a method of determining whether male Fabry patients are likely to respond to treatment with migalastat which expires in 2027. Foreign counterpart applications are pending in Australia, Canada, Europe and Hong Kong. We have one issued U.S. patent covering a method of treating a patient diagnosed with Fabry disease with migalastat wherein the Fabry patient has one of several α -galactosidase A mutations. This patent will expire in 2029. We also have a pending U.S. application covering a method of determining which α -galactosidase A mutations are likely to be amendable to therapy with migalastat which, if granted, will expire in 2029. Foreign counterpart applications are also pending in Europe, Japan, Canada, Mexico and Australia, which if granted, will also expire in 2029.

- We have an exclusive license to pending patent applications covering the co-administration of migalastat with ERT (recombinant α -galactosidase A). Patents covering specific combinations have issued in Europe, China, India and Mexico. These issued patents will expire in 2024. Other applications from this family are pending in the U.S., Europe, Canada, Brazil, China, Hong Kong, India, Israel, Japan and Mexico. If patents issue from these applications, expiration will be in 2024. We also own a U.S. provisional patent application covering specific doses and dosing regimens of migalastat to treat Fabry disease in combination with ERT (recombinant α -galactosidase A) in the U.S., Brazil, Canada, Chile, China, Europe, Hong Kong, Israel, India, Japan, South Korea, Mexico, New Zealand, Singapore, Taiwan and South Africa. If patents issue from these applications, expiration will be in 2032 to 2033.

- We own a pending patent application covering a high concentration co-formulation of recombinant acid α -glucosidase and pharmacological chaperone in the U.S., Canada, Europe, Japan, Mexico and South Korea. If patents issue from this international application, expiration will be in 2033.

- We own a U.S. patent application covering a co-formulation of recombinant α -galactosidase A and migalastat. If a patent issues from this application, expiration will be in 2033.

- As part of the Callidus acquisition, we acquired certain patent applications including an application series covering methods for coupling targeting peptides to recombinant lysosomal enzymes, including recombinant α -galactosidase A. These applications are pending in the U.S., Europe, Japan, Brazil, Canada, China and the Republic of Korea. If patents issue from these applications, expiration will be in 2032. Another patent application series covers a variant recombinant β -glucocerebrosidase which was filed in the U.S., Europe, Japan, Brazil, Canada, China and the Republic of Korea. If patents issue from these applications, expiration will be in

2031. Yet another patent application series covers novel signal sequences to improve protein expression and secretion of proteins. These applications were filed in the U.S., Europe, Japan, Brazil, Canada, China and the Republic of Korea. If patents issue from these applications, expiration will be in 2031.

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, and amendments thereto, 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. For information regarding our migalastat collaboration with GSK, please see "Strategic Alliances and Arrangements" above. For our other license agreements, 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 migalastat and other pharmacological chaperones for the prevention or treatment of human diseases or clinical conditions by increasing the activity of wild-type and mutant enzymes pursuant to a license agreement with Mt. Sinai School of Medicine ("MSSM") of New York University. In connection with this agreement, we issued 232,266 shares of our common stock to MSSM in April 2002. In October 2006, we issued MSSM an additional 133,333 shares of common stock and made a payment of $1.0 million in consideration of an expanded field of use under that license. Under this agreement, to date we have paid no upfront or annual license fees and we have no milestone or future payments other than royalties on net sales. However, in October 2008, we amended and restated this license agreement to, among other items, provide us with the sole right to control the prosecution of patent rights under such agreement and to clarify the portion of royalties and milestone payments we received from Shire that were payable to MSSM. In connection therewith, we agreed to pay MSSM $2.6 million in connection with the $50 million upfront payment that we received in November 2007 from Shire, our former collaborator, which was already accrued for at year-end 2007, and an additional $2.6 million for the sole right to and control over the prosecution of patent rights. In addition, we paid MSSM $3 million of the $30 million upfront payment received from GSK in the fourth quarter of 2010. This agreement expires upon expiration of the last of the licensed patent rights, which will be in 2019, subject to any patent term extension that may be granted, or 2024 if we develop a product for combination therapy (pharmacological chaperone plus ERT) and a patent issues from the pending application covering the combination therapy, subject to any patent term extension that may be granted.

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 migalastat, we will owe royalties only to MSSM and will owe no milestone payments.

Our rights with respect to these agreements to develop and commercialize migalastat 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 own certain trademarks in the U.S. and/or abroad, including AMICUS THERAPEUTICS® & design, AMICUS THERAPEUTICS® and CHART®. At present, all of the U.S. trademark applications for these marks have been either filed or registered by the U.S. Patent and Trademark Office.

Manufacturing

We continue to rely on contract manufacturers to supply the active pharmaceutical ingredients and final drug product for migalastat, other pharmacological chaperones and our next-generation ERT product candidates. The active pharmaceutical ingredients and final formulations for these products are manufactured under current good manufacturing practices ("cGMP"). The components in the final formulation for each product are commonly used in other pharmaceutical products and are well characterized ingredients. We have implemented appropriate controls for assuring the quality of both active pharmaceutical ingredients and final drug products. Product specifications will be established in concurrence with regulatory bodies at the time of product registration.

Competition

Overview

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

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

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

Major Competitors

Our major competitors include pharmaceutical and biotechnology companies in the U.S. and abroad that have approved therapies or therapies in development for lysosomal storage disorders within our core programs. Other competitors are pharmaceutical and biotechnology companies that have

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

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	2014 Sales (in millions USD)
sanofi aventis	Fabry disease	Fabrazyme®	ERT	Marketed	$ 610
	Pompe disease	Myozyme®/ Lumizyme®	ERT	Marketed	$ 720
	Fabry disease	GZ402671	Oral GCS Inhibitor	Phase 1	N/A
	Pompe disease	GZ402666 ("neo GAA")	ERT	Phase 1	N/A
Shire	Fabry disease	Replagal®	ERT	Marketed	$ 500
Biomarin Pharmaceutical, Inc.	Pompe disease	BMN-701	ERT	Phase 2/3	N/A
	MPS I	Aldurazyme®	ERT	Marketed	$ 106
Protalix Biotherapeutics	Fabry disease	PRX-102	ERT	Phase 1/2	N/A

Government Regulation

FDA Approval Process

In the U.S., pharmaceutical products are subject to extensive regulation by the FDA. The Federal Food, Drug, and Cosmetic Act, Public Health Services 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 file a marketing application, to issue Complete Response letters or to not approve pending NDAs or biologic product license applications (BLAs), warning letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, civil penalties, litigation, government investigation and criminal prosecution.

Pharmaceutical product development in the U.S. typically involves nonclinical laboratory and animal tests, the submission to the FDA of an investigational new drug application ("IND"), which must become effective before clinical testing may commence, and adequate and well-controlled clinical trials to establish the safety and effectiveness of the drug for each indication for which FDA approval is sought. Satisfaction of FDA pre-market approval requirements typically takes many years and the actual time required varies 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 studies to assess the characteristics, 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 at least one proposed clinical trial protocol. Long-term preclinical safety evaluations, such as animal tests of reproductive toxicity and carcinogenicity, continue during the IND phase of development. Reproductive toxicity studies are required to allow inclusion of women of child bearing potential in clinical trials, whereas carcinogenicity studies are required for registration. The results of these long term studies would eventually be described in product labeling.

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

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

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

Clinical trials to support a NDA or BLA for marketing approval are typically conducted in three sequential phases, but the phases may overlap. In Phase 1, the initial introduction of the drug into healthy human subjects or patients, the drug is tested to assess metabolism, pharmacokinetics, pharmacological actions, side effects associated with increasing doses and, if possible, early evidence on effectiveness. Phase 2 usually involves trials in a limited patient population, to determine the effectiveness of the drug for a particular indication or indications, dosage tolerance and optimum dosage, and identify common adverse effects and safety risks. If a compound demonstrates evidence of effectiveness and an acceptable safety profile in Phase 2 evaluations, Phase 3 trials are undertaken to obtain the additional information about clinical efficacy and safety in a larger number of patients over longer treatment periods, 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 or BLA is prepared and submitted to the FDA. FDA approval of the NDA or BLA is required before marketing of the product may begin in the U.S. The NDA or BLA 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 or BLA is substantial. Under federal law, the submission of most NDAs and BLAs is additionally subject to a substantial application user fee; although for Orphan Drugs these fees are waived, and the holder of an approved NDA or BLA may also be subject to annual product and establishment user fees. These fees are typically increased annually.

The FDA has 60 days from its receipt of an NDA or BLA 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 NDAs. Marketing applications are assigned review status during the filing period. Review status could be either standard or priority. Most such applications for standard review are reviewed within 12 months under PDUFA V (2 months for filing plus ten months for review. The FDA attempts to review a drug candidate that is eligible for priority review within six months, as discussed below. The review process may be extended by FDA for three additional months to evaluate major amendments submitted during the pre-specified PDUFA V review clock 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 for public review,

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 or BLA, 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 or BLA contains data that provide substantial evidence that the drug is safe and effective in the indication studied and to be marketed.

After FDA evaluates the NDA or BLA and the manufacturing facilities, it issues an approval letter or a complete response letter. Complete response letters outline the deficiencies in the submission that prevent approval and may require substantial additional testing or information for the FDA to reconsider the application. If and when those deficiencies have been addressed to the FDA's satisfaction in an amendment submitted to the NDA or BLA, the FDA will then issue an approval letter. FDA has committed to reviewing such resubmissions in 2 or 6 months depending on the type and extent 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 commitments to conduct additional testing and/ or surveillance to monitor the drug's safety or efficacy and may impose other conditions, including distribution and 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, problems are identified following initial marketing or post-marketing commitments are not met.

The Hatch-Waxman Act

In seeking approval for a drug through an NDA, applicants are required to list with the FDA certain patent(s) with claims that cover the applicant's product or approved method of use. 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 route of administration, active ingredients strength and dosage form as the listed drug and has been shown through bioequivalence testing to be, in most cases, therapeutically equivalent to the listed drug. ANDA applicants are not required to conduct or submit results of preclinical or clinical tests to prove the safety or effectiveness of their drug product, other than the requirement for bioequivalence testing. Drugs approved in this way are commonly referred to as "generic equivalents" to the listed drug and can often be substituted by pharmacists under prescriptions written for the original listed, "innovator" 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 submits 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.

Patent term and data exclusivity run in parallel. An 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 certification that challenges 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 or BLA 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, communications regarding unindicated uses, industry-sponsored scientific and educational activities and promotional activities involving the internet.

Drugs may be promoted only for 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, new safety information, labeling, or manufacturing processes or facilities, require submission and FDA approval of a new NDA, NDA supplement, BLA or BLA supplement before the change can be implemented. New efficacy claims require submission and approval of an NDA supplement and BLA supplement for each new indication. The efficacy claims typically require new clinical data similar to that included in the original application. The FDA uses the same procedures and actions in reviewing NDA and BLA supplements as it does in reviewing NDAs and BLAs. Additional exclusivity may be granted for new efficacy claims. Generic ANDA cannot be labeled for these types of claims until the new exclusivity period expires.

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

Orphan Drugs

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

Pediatric Information

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

Fast Track Designation

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

In addition to other benefits such as the ability to use surrogate endpoints and have greater interactions with FDA, FDA may initiate review of sections of a Fast Track drug's NDA or BLA 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 review period as specified under PDUFA V for filing and reviewing an application does not begin until the last section of the NDA or BLA has been 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 six-months from filing for a New Molecular Entity ("NME") or six months from submission for a non-NME 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. The FDA makes its determination of priority or standard review during the 60-day filing period after an initial NDA or BLA submission.

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. This approval mechanism is provided for under 21CRF314 Subpart H. In this case, clinical trials are conducted in which a biomarker is used as the primary outcome for approval. This biomarker substitutes for a direct measurement of how a patient feels, functions, or survives. Such biomarkers 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. When the Phase 4 commitment is successfully completed, the biomarker is deemed to be a surrogate endpoint. Failure to conduct required post-approval studies, or confirm a clinical benefit during post-marketing studies, could lead 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, an ANDA or BLA. A fourth alternative is a special type of NDA, commonly referred to as a Section 505(b) (2) NDA, which enables the applicant to rely, in part, on the safety and efficacy data of an existing product, or published literature, in support of its application.

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

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

Patient Protection and Affordable Care Act of 2010

The Biologics Price Competition and Innovation Act of 2009 ("BPCIA"), which was enacted as part of the Patient Protection and Affordable Care Act of 2010, as amended by the Health Care and Education Reconciliation Act of 2010 ("PPACA") created an abbreviated approval pathway for biological products that are demonstrated to be "biosimilar" or "interchangeable" with an FDA-licensed reference biological product via an approved BLA. Biosimilarity to an approved reference product requires that there be no differences in conditions of use, route of administration, dosage form, and strength, and no clinically meaningful differences between the biological product and

the reference product in terms of safety, purity, and potency. Biosimilarity is demonstrated in steps beginning with rigorous analytical studies or "fingerprinting", *in vitro* studies, *in vivo* , animal studies, and generally at least one clinical study, absent a waiver from the Secretary of Health and Human Services. The biosimilarity exercise tests the hypothesis that the investigational product and the reference product are the same. If at any point in the stepwise biosimilarity process a significant difference is observed, then the products are not biosimilar, and the development of a stand-alone NDA or BLA is necessary. In order to meet the higher hurdle of interchangeability, a sponsor must demonstrate that the biosimilar product can be expected to produce the same clinical result as the reference product, and for a product that is administered more than once, that the risk of switching between the reference product and biosimilar product is not greater than the risk of maintaining the patient on the reference product. Complexities associated with the larger, and often more complex, structures of biological products, as well as the process by which such products are manufactured, pose significant hurdles to implementation that are still being evaluated by the FDA. Under the BPCIA, a reference biologic is granted twelve years of exclusivity from the time of first licensure of the reference product.

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

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

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

Physician Drug Samples

As part of the sales and marketing process, pharmaceutical companies frequently provide samples of approved drugs to physicians. The Prescription Drug Marketing Act (the "PDMA") imposes requirements and limitations upon the provision of drug samples to physicians, as well as prohibits states from licensing distributors of prescription drugs unless the state licensing program meets certain

federal guidelines that include minimum standards for storage, handling and record keeping. In addition, the PDMA sets forth civil and criminal penalties for violations.

Regulation Outside the U.S.

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

To obtain regulatory approval of an orphan drug under EU regulatory systems, we are mandated to submit marketing authorization applications in a Centralized 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 EMA for migalastat for the treatment of Fabry disease. The EMA 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 fee reductions for protocol assistance and 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 EMA marketing approval for the indication for which it has such designation, the product is entitled to orphan drug exclusivity, which means the EMA 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.

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, 2014, we had 97 full-time employees, 68 of whom were primarily engaged in research and development activities and 29 of whom provide administrative services. A total of 25 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 1 Cedar Brook Drive, Cranbury, NJ 08512 and our telephone number is (609) 662-2000. Our website address is *www.amicusrx.com* . We make available free of charge on our website our annual, quarterly and current reports, including amendments to such reports, as soon as reasonably practicable after we electronically file such material with, or furnish such material to, the U.S. Securities and Exchange Commission.

Information relating to our corporate governance, 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.amicusrx.com* under the "Investors — Corporate Governance" caption and in

print to any stockholder upon request. Any waivers or material amendments to the Code will be posted promptly on our website.

We have filed applications to register certain trademarks in the U.S. and abroad, including AMICUS THERAPEUTICS® and design and AMICUS THERAPETUICS®. Fabrazyme®, Myozyme®, Lumizyme®, and Replagal® are the property of their respective owners.

ITEM 1A. RISK FACTORS

The occurrence of any of the following risks could harm our business, financial condition, results of operations and/or growth prospects. In that case, the trading price of our common stock could decline, and you may lose all or part of your investment. You should understand that it is not possible to predict or identify all such risks. Consequently, you should not consider the following to be a complete discussion of all potential risks or uncertainties.

Risks Related to Our Business, 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 accumulated deficit was $447.4 million as of December 31, 2014. To date, we have financed our operations primarily through private placements of our redeemable convertible preferred stock, proceeds from our initial public and secondary stock offerings, debt financings and from our collaboration agreements. We have devoted substantially all of our efforts to research and development, including our preclinical development activities and clinical trials. We have not completed development of any drugs. We expect to continue to incur significant and increasing operating losses for at least the next several years and we are unable to predict the extent of any future losses as we:

- continue our ongoing Phase 3 clinical trials of migalastat for the treatment of Fabry disease to support regulatory approval in the United States and worldwide;
- begin Phase 1/2 clinical studies of migalastat co-administered or co-formulated with ERT for Fabry disease;
- continue our preclinical studies on the use of pharmacological chaperones co-formulated and co-administered with ERT for Fabry, Pompe and other lysosomal storage diseases;
- continue the research and development of additional product candidates;
- seek regulatory approvals for our product candidates that successfully complete clinical trials; and
- establish a sales and marketing infrastructure to commercialize products for which we may obtain regulatory approval.

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

impair our ability to raise capital, expand our business, diversify our product offerings or continue our operations.

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

We expect to continue to incur substantial research and development expenses in connection with our ongoing activities, particularly as we continue our Phase 3 development of migalastat. Further, subject to obtaining regulatory approval of any of our product candidates including migalastat, we expect to incur significant commercialization expenses for product sales and marketing, securing commercial quantities of product from our manufacturers and product distribution. Under the Revised Agreement entered into with GSK in November 2013, GSK will no longer share in the research and development costs related to migalastat as of January 1, 2014. We are responsible for 100% of all research and development costs for all of our programs.

In order to complete clinical trials related to migalastat, seek regulatory approvals of migalastat, commercially launch the product candidate and continue our other clinical and preclinical programs, we will need to seek additional funding. 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 our drug candidates, including migalastat;
- the cost of manufacturing drug supply for our clinical and preclinical studies, including the significant cost of new ERT cell line development and manufacturing as well as the cost of manufacturing Pompe ERT;
- the scope, progress, results and costs of preclinical development, laboratory testing and clinical trials for our product candidates including those testing the use of pharmacological chaperones co-formulated and co-administered with ERT and for the treatment of LSDs;
- 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 are able to raise capital by issuing equity securities, our stockholders will experience dilution. In addition, stockholders may experience dilution if the holders of the warrants issued in connection with our private placement in November 2013 exercise their warrants. Debt

financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends and may include rights that are senior to the holders of our common stock. Our current loan and security agreement with Silicon Valley Bank includes a covenant whereby we must maintain a minimum amount of liquidity measured at the end of each month where unrestricted cash, cash equivalents and marketable securities is greater than $20 million plus outstanding debt due to Silicon Valley Bank.

Our credit and security agreement with MidCap Funding III, LLC contains restrictions that limit our flexibility in operating our business. We may be required to make a prepayment or repay the outstanding indebtedness earlier than we expect under our credit and security agreement if a mandatory prepayment event or an event of default occurs, including a material adverse change with respect to us, which could have a materially adverse effect on our business.

Our credit and security agreement with MidCap Funding III, LLC ("MidCap") as administrative agent for the other lenders named therein, pursuant to which we have drawn-down $15.0 million, contains various covenants that limit our ability to engage in specified types of transactions. These covenants limit our ability to, among other things:

- incur or assume certain debt;
- merge or consolidate;
- change the nature of our business;
- change our organizational structure or type;
- dispose of certain assets;
- grant liens on our assets
- make certain investments;
- pay dividends; and
- enter into material transactions with affiliates or third parties.

The restrictive covenants of the agreement could cause us to be unable to pursue business opportunities that we or our stockholders may consider beneficial. A breach of any of these covenants could result in an event of default under the agreement. An event of default will also occur if, among other things, a material adverse change in our business, operations or condition (financial or otherwise) or prospects occurs, or a material impairment of the prospect of our repayment of any portion of the amounts we owe under the agreement occurs. In the case of a continuing event of default under the agreement, MidCap could elect to declare all amounts outstanding to be immediately due and payable and terminate all commitments to extend further credit (and in the case of an event of default related to bankruptcy or insolvency, all amounts outstanding would be immediately due and payable and commitments terminated), proceed against the collateral in which we granted MidCap a security interest under the agreement, or otherwise exercise the rights of a secured creditor. Amounts outstanding under the agreement are secured by all of our existing and future assets (excluding intellectual property we own, which is subject to a negative pledge arrangement).

We may not have enough available cash or be able to raise additional funds on satisfactory terms, if at all, through equity or debt financings to make any required mandatory prepayment or repay such indebtedness at the time any such prepayment event or event of default occurs. In such an event, we may be required to delay, limit, reduce or terminate our product development or commercialization efforts or grant to others rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves. Our business, financial condition and results of operations could be materially adversely affected as a result.

We may acquire other assets or businesses, or form collaborations or make investments in other companies or technologies that could harm our operating results, dilute our stockholders' ownership, increase our debt or cause us to incur significant expense.

As part of our business strategy, we may pursue acquisitions of assets or businesses, or strategic alliances and collaborations, to expand our existing technologies and operations. We may not identify or complete these transactions in a timely manner, on a cost-effective basis, or at all, and we may not realize the anticipated benefits of any such transaction, any of which could have a detrimental effect on our financial condition, results of operations and cash flows. We may not be able to find suitable acquisition candidates, and if we make any acquisitions, we may not be able to integrate these acquisitions successfully into our existing business and we may incur additional debt or assume unknown or contingent liabilities in connection therewith. Integration of an acquired company or assets may also disrupt ongoing operations, require the hiring of additional personnel and the implementation of additional internal systems and infrastructure, especially the acquisition of commercial assets, and require management resources that would otherwise focus on developing our existing business. We may not be able to find suitable collaboration partners or identify other investment opportunities, and we may experience losses related to any such investments.

To finance any acquisitions or collaborations, we may choose to issue debt or shares of our common stock as consideration. Any such issuance of shares would dilute the ownership of our stockholders. If the price of our common stock is low or volatile, we may not be able to acquire other assets or companies or fund a transaction using our stock as consideration. Alternatively, it may be necessary for us to raise additional funds for acquisitions through public or private financings. Additional funds may not be available on terms that are favorable to us, or at all.

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

We commenced operations in February 2002. Our operations to date have been limited to organizing and staffing our company, acquiring and developing our technology and undertaking preclinical studies and clinical trials of our most advanced product candidates. We have not yet generated any commercial sales for any of our product candidates. We have not yet demonstrated our ability to 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, if we are successful in obtaining marketing approval for any of our lead product candidates or if we acquire commercial assets, 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.

Our business and operations would suffer in the event of computer system failures.

Despite the implementation of security measures, our internal computer systems, and those of our contract research organizations, contract manufacturing organizations, or CMO, and other third parties on which we rely, we are vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. System failures, accidents or security breaches could cause interruptions in our operations, and could result in a material disruption of our clinical activities and business operations, in addition to possibly requiring substantial expenditures of resources to remedy. If such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our drug development programs. For example, the loss of clinical trial data from completed or ongoing clinical trials could result in delays in our

regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruptions or security breach was to result in a loss or damage to our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability and the further development of our product candidates could be delayed.

Risks Related to the Development and Commercialization of Our Product Candidates

We depend heavily on the success of our most advanced product candidates. 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 our most advanced product candidates, including migalastat, or experience significant delays in doing so, our business will be materially harmed.

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

- successful enrollment of patients in our clinical trials on a timely basis;
- obtaining supplies of our product candidates and, where required, third party marketed products including ERTs, for completion of our clinical trials on a timely basis;
- successful completion of preclinical studies and clinical trials;
- obtaining regulatory agreement in the structure and design of our clinical programs;
- obtaining marketing approvals from the 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 cGMP regulations;
- launching commercial sales of the product, whether alone or in collaboration with others;
- acceptance of the product by patients, the medical community and third party payors;
- competition from other companies and their therapies;
- successful protection of our intellectual property rights from competing products in the U.S. and abroad; and
- a continued acceptable safety and efficacy profile of our product candidates following approval.

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

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

Currently, most reported estimates of the prevalence of these diseases are based on studies of small subsets of the population of specific geographic areas, which are then extrapolated to estimate the prevalence of the diseases in the broader world population. In addition, as new studies are performed the estimated prevalence of these diseases may change. There can be no assurance that the prevalence of Fabry disease or Pompe disease in the study populations, particularly in these newer studies, accurately reflects the prevalence of these diseases in the broader world population. If our

estimates of the prevalence of Fabry disease or of the number of patients who may benefit from treatment with our product candidates prove to be incorrect, the market opportunities for our product candidates may be smaller than we believe they are, our prospects for generating revenue may be adversely affected and our business may suffer.

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

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

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

In addition, while the clinical trials of our drug candidates are designed based on the available relevant information, in view of the uncertainties inherent in drug development, such clinical trials may not be designed with focus on indications, patient populations, dosing regimens, safety or efficacy parameters or other variables that will provide the necessary safety or efficacy data to support regulatory approval to commercialize the resulting drugs. In addition, individual patient responses to the dose administered of a drug may vary in a manner that is difficult to predict. Also, the methods we select to assess particular safety or efficacy parameters may not yield statistical precision in estimating our drug candidates' effects on study participants. Even if we believe the data collected from clinical trials of our drug candidates are promising, these data may not be sufficient to support approval by the FDA or foreign regulatory authorities. Preclinical and clinical data can be interpreted in different ways. Accordingly, the FDA or foreign regulatory authorities could interpret these data in different ways from us or our partners, which could delay, limit or prevent regulatory approval.

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, if the FDA requires different endpoints than the endpoints we anticipate using or a different analysis of those endpoints, it may be more difficult for us to obtain, or we may be delayed in obtaining, FDA approval of our product candidates. If we are not successful in commercializing any of our lead product candidates, or are significantly delayed in doing so, our business will be materially harmed.

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

We have limited experience in conducting and managing the preclinical development activities and clinical trials necessary to obtain regulatory approvals, including approval by the FDA. We have not obtained regulatory approval nor commercialized any of our product candidates. 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 rare and we expect only a subset of the patients with these diseases to be eligible for our clinical trials. 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. For example, the entry criteria for our Phase 3 study in migalastat for Fabry disease to support approval in the United States (Study 011) required that patients must have a genetic mutation that we believe is responsive to migalastat, and may not have received ERT in the past or must have stopped treatment for at least six months prior to enrolling in the study. As a result, enrollment of the study lasted for over two years.

In addition, 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. Our inability to enroll a sufficient number of patients for any of our current or future clinical trials would result in significant delays or may require us to abandon one or more clinical trials altogether.

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

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

- our preclinical tests or clinical trials may produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional preclinical testing or clinical trials or we may abandon projects that we expect to be promising;
- we may decide to amend existing protocols for on-going clinical trials;
- regulators or institutional review boards may not authorize us to commence a clinical trial or conduct a clinical trial at a prospective trial site;
- conditions imposed on us by the FDA or any non-U.S. regulatory authority regarding the scope or design of our clinical trials may require us to resubmit our clinical trial protocols to institutional review boards for re-inspection due to changes in the regulatory environment;
- the number of patients required for our clinical trials may be larger than we anticipate or participants may drop out of our clinical trials at a higher rate than we anticipate;
- our third party contractors or clinical investigators may fail to comply with regulatory requirements or fail to meet their contractual obligations to us in a timely manner;

- we might have to suspend or terminate one or more of our clinical trials if we, the regulators or the institutional review boards determine that the participants are being exposed to unacceptable health risks;
- regulators or institutional review boards may require that we hold, suspend or terminate clinical research for various reasons, including noncompliance with regulatory requirements;
- the cost of our clinical trials may be greater than we anticipate;
- the supply or quality of our product candidates or other materials necessary to conduct our clinical trials, such as existing treatments like ERT, may be insufficient or inadequate or we may not be able to reach agreements on acceptable terms with prospective clinical research organizations; and
- the effects of our product candidates may not be the desired effects or may include undesirable side effects or the product candidates may have other unexpected characteristics.

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

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

Our product development costs will also increase if we experience delays in testing or approvals. We do not know whether any preclinical tests or clinical trials will be initiated as planned, will need to be restructured or will be completed on schedule, if at all. Significant preclinical or clinical trial delays also could shorten the patent protection period during which we may have the exclusive right to commercialize our product candidates. Such delays could allow our competitors to bring products to market before we do and impair our ability to commercialize our products or product candidates.

Even if migalastat or any other product candidate that we develop receives marketing approval, we will continue to face extensive regulatory requirements and the product may still face future development and regulatory difficulties.

Even if marketing approval is obtained, a regulatory authority may still impose significant restrictions on a product's indications, conditions for use, distribution or marketing or impose ongoing requirements for potentially costly post-market surveillance, post-approval studies or clinical trials. For example, any labeling ultimately approved by the FDA for migalastat, if it is approved for marketing, may include restrictions on use, such as limitations on how Fabry disease is defined and diagnosed. In addition, the labeling may include restrictions based upon evidence of specific genetic mutations or symptoms found in patients. Migalastat will also be subject to ongoing FDA requirements governing the labeling, packaging, storage, advertising, distribution, promotion, recordkeeping and submission of safety and other post-market information, including adverse events, and any changes to the approved product, product labeling, or manufacturing process. The FDA has significant post-market authority, including, for example, the authority to require labeling changes based on new safety information, and to require post-market studies or clinical trials to evaluate serious safety risks related to the use of a drug. For products approved under the Accelerated Approval regulations, the FDA has the authority to require clinical studies to confirm the clinical benefit associated with the surrogate endpoint. In addition, manufacturers of drug products and their facilities are subject to continual review and

periodic inspections by the FDA and other regulatory authorities for compliance with cGMP and other regulations.

If we, our drug products or the manufacturing facilities for our drug products fail to comply with applicable regulatory requirements, a regulatory agency may:

- issue warning letters or untitled letters;
- seek an injunction or impose civil or criminal penalties or monetary fines;
- suspend or withdraw marketing approval;
- suspend any ongoing clinical trials;
- refuse to approve pending applications or supplements to applications submitted by us;
- suspend or impose restrictions on operations, including costly new manufacturing requirements;
- seize or detain products, refuse to permit the import or export of products or request that we initiate a product recall; or
- refuse to allow us to enter into supply contracts, including government contracts.

The FDA and other regulatory agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses. If we are found to have promoted off-label uses, we may become subject to significant liability.

The FDA and other regulatory agencies strictly regulate the promotional claims that may be made about prescription products. In particular, a product may not be promoted for uses that are not approved by the FDA or such other regulatory agencies as reflected in the product's approved labeling. In particular, any labeling approved by the FDA for migalastat or any of our other product candidates may include restrictions on use. The FDA may impose further requirements or restrictions on the distribution or use of migalastat or any of our other product candidates as part of a REMS plan. If we receive marketing approval for migalastat or any other product candidates, physicians may nevertheless prescribe such products to their patients in a manner that is inconsistent with the approved label. If we are found to have promoted such off-label uses, we may become subject to significant liability. The federal government has levied large civil and criminal fines and / or other penalties against companies for alleged improper promotion and has investigated and / or prosecuted several companies in relation to off-label promotion (which is a violation of Federal regulations). The FDA has also requested that certain companies enter into consent decrees or permanent injunctions under which specified promotional conduct is changed, curtailed or prohibited.

The commercial success of any product candidates that we may develop, including migalastat, 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 migalastat , 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:

- prevalence and severity of any side effects, including any limitations or warnings contained in a product's approved labeling;
- efficacy and potential advantages over alternative treatments;
- pricing;

- relative convenience and ease of administration;
- willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- 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 product candidate 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.

In addition, the Patient Protection and Affordable Care Act of 2010 and the Health Care and Education Reconciliation Act of 2010 (collectively referred to as the "Health Care Reform Law") are designed to overhaul the United States health care system and regulate many aspects of health care delivery and financing. The Health Care Reform Law is intended to broaden access to health insurance, primarily through the imposition of health insurance mandates on employers and individuals and expansion of the Medicaid program, reduce or constrain the growth of health care spending, enhance remedies against fraud and abuse, add new transparency requirements for health care and health insurance industries, impose new taxes and fees on the health industry and impose additional health policy reforms. The Health Care Reform Law will require the promulgation of substantial regulations with significant effects on the health care industry.

A number of provisions contained in the Health Care Reform Law may affect us and will likely increase certain of our costs. For example, the new law revised the definition of "average manufacturer price" for reporting purposes and the volume of rebated drugs has been expanded to include beneficiaries in Medicaid managed care organizations, which could increase the amount of Medicaid drug rebates to states. Also, beginning in 2013, drug manufacturers will be required to report information on payments or transfers of value to physicians and teaching hospitals, as well as investment interests held by physicians and their immediate family members during the preceding calendar year. Under a final rule issued by the Centers for Medicare & Medicaid Services ("CMS"), drug manufacturers must begin to collect the required data on August 1, 2013 and report the data to CMS by March 31, 2014. Failure to submit required information may result in civil monetary penalties. Additionally, the Health Care Reform Law includes a 50% discount on brand name drugs for Medicare Part D participants in the coverage gap, or "donut hole." We do not know the full effect that the Health Care Reform Law will have on our commercialization efforts if migalastat, or any other of our drugs, is approved. Although it is too early to determine the effect of the Health Care Reform Law, the law appears likely to continue the pressure on pharmaceutical pricing, especially under the Medicare program, and may also increase our regulatory burdens and operating costs.

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 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, including migalastat, 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 for other product candidates on acceptable terms or at all, and if we do enter into a distribution arrangement, our success will be dependent upon the performance of our partner.

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

- our inability to recruit and retain adequate numbers of effective sales and marketing personnel;
- the inability of sales personnel to obtain access to or successfully market to adequate numbers of physicians to prescribe our products;
- the lack of additional products to be marketed 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 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;
- damage to our reputation;
- regulatory investigations, prosecutions or enforcement actions 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 such product candidates or products.

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, acquire or commercialize in the future from major pharmaceutical companies, specialty pharmaceutical companies and biotechnology companies worldwide. For example, several large pharmaceutical and biotechnology companies currently market and sell products for the treatment of lysosomal storage diseases, including Fabry disease. These products include Sanofi Aventis' Fabrazyme® and Shire plc's Replagal®. In addition, Sanofi markets and sells Myozyme® and Lumizyme® for the treatment of Pompe disease. For MPS I, Biomarin Pharmaceutical, Inc. manufactures and supplies Aldurazyme® to Sanofi for global sales and marketing. We are also aware of other enzyme replacement and substrate reduction therapies in development by third parties, including

Biomarin Pharmaceutical's BMN-701, an enzyme replacement therapy in Phase 2/3 development for Pompe disease.

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 will not render our product candidates or any acquired products obsolete or noncompetitive either during the research phase or once the products reaches 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 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 to third parties. The manufacture of pharmaceutical products requires significant expertise and capital investment, including the development of advanced manufacturing techniques and process controls. Manufacturers of pharmaceutical products often encounter difficulties in production, particularly in scaling up initial production. These problems include difficulties with production costs and yields and quality control, including stability of the product candidate. The occurrence of any of these problems could significantly delay our clinical trials or the commercial availability of our products.

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

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

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

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

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

Our product candidates and any products that we may develop or acquire 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.

Transitioning our business to focus on the commercialization of our products, specifically migalastat, may require increased reliance on third-party relationships to enable this transition, which may have an adverse effect on our business.

We acquired significant commercial rights to all formulations of migalastat under the Revised Agreement with GSK. If we were to obtain marketing approval for migalastat from the FDA, we will need to continue to transition from a company with a development focus to a company capable of supporting commercial activities. We may not be successful in such a transition. We have not yet demonstrated an ability to obtain marketing approval for or commercialize a product candidate. As a result, we may not be as successful as companies that have previously obtained marketing approval for drug candidates and commercially launched drugs.

There are risks involved with both establishing our own sales and marketing capabilities and entering into arrangements with third parties to perform these services. For example, recruiting and training a sales force is expensive and time consuming and could delay any product launch. If the commercial launch of a product candidate for which we recruit a sales force and establish marketing capabilities is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses. This may be costly, and our investment would be lost if we cannot retain or reposition our sales and marketing personnel. Factors that may inhibit our efforts to commercialize our products 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 any future products;
- the lack of complementary products to be offered by sales personnel, which may put us at a competitive disadvantage relative to companies with more extensive product lines; and
- unforeseen costs and expenses associated with creating an independent sales and marketing organization.

If we enter into arrangements with third parties to perform sales, marketing and distribution services, our product revenues or the profitability of these product revenues to us are likely to be lower than if we were to market and sell any products that we develop ourselves. In addition, we may not be successful in entering into arrangements with third parties to sell and market our product candidates or doing so on terms that are favorable to us. We likely will have little control over such third parties, and any of them may fail to devote the necessary resources and attention to sell and market our products

effectively. If we do not establish sales and marketing capabilities successfully, either on our own or in collaboration with third parties, we will not be successful in commercializing our product candidates.

Changes to our collaboration with GSK will require us to develop a new source of ERT for a key component of migalastat co-formulated with human recombinant alpha-Gal enzyme.

Our initial co-formulated product candidate for Fabry Disease that we developed as part of our collaboration with GSK utilized migalastat co-formulated with a proprietary human recombinant alpha-Gal enzyme. We plan to continue development of a co-formulated ERT with migalastat with an internally developed Fabry cell line as a next-generation ERT for Fabry disease.

The risks involved with developing our own internal cell line are in addition to the risks described above with respect to securing and using third party manufacturers and it could significantly and adversely affect the overall cost of developing the co-formulated product candidate and significantly increase the timelines for development.

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

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

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

We do not independently conduct clinical trials for our product candidates or certain preclinical development activities of our product candidates, such as long-term safety studies in animals. We rely on, or work in conjunction with, third parties, such as contract research organizations, medical institutions and clinical investigators, to perform these functions. For example, we rely heavily on a contract research organization to help us conduct our ongoing Phase 3 clinical trials in migalastat for the treatment of Fabry disease. Our reliance on these third parties for preclinical and clinical development activities reduces our control over these activities. We are responsible for ensuring that each of our preclinical development activities and our clinical trials is conducted in accordance with the applicable general investigational plan and protocols, however, we have no direct control over these researchers or contractors (except by contract), as they are not our employees. Moreover, the FDA requires us to comply with standards, commonly referred to as Good Clinical Practices for conducting, recording and reporting the results of our preclinical development activities and our clinical trials to assure that data and reported results are credible and accurate and that the rights, safety and

confidentiality of trial participants are protected. Our reliance on third parties that we do not control does not relieve us of these responsibilities and requirements. Furthermore, these third parties may also have relationships with other entities, some of which may be our competitors. If these third parties do not successfully carry out their contractual duties, meet expected deadlines or conduct our preclinical development activities or our clinical trials in accordance with regulatory requirements or our stated protocols, we will not be able to obtain, or may be delayed in obtaining, regulatory approvals for our product candidates and will not be able to, or may be delayed in our efforts to, successfully commercialize our product candidates. Moreover, these third parties may be bought by other entities or they may go out of business, thereby preventing them from meeting their contractual obligations.

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

Extensions, delays, suspensions or terminations of our preclinical development activities or 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. If we elect to seek collaborators in the future but are unable to reach agreements with suitable collaborators, we may fail to meet our business objectives for the affected product or program. We face, and will continue to face, significant competition in seeking appropriate collaborators. Moreover, collaboration arrangements are complex and time consuming to negotiate, document and implement. We may not be successful in our efforts, if any, to establish and implement collaborations or other alternative arrangements. The terms of any collaboration or other arrangements that we establish, if any, may not be favorable to us.

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

- our collaboration agreements are likely to be for fixed terms and subject to termination by our collaborators;
- our collaborators may 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.

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 product candidates 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 product candidates. 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 product candidates. 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 product candidates. 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 United States, the European Patent Office and other countries outside the U.S. that have not been issued as patents. These pending applications include, among others, some of 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 that we have licensed from Mt. Sinai School of Medicine relating to use of migalastat to treat Fabry disease expire in 2018 in the U.S. and 2019 in Europe, Japan, and Canada. In addition to 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. 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 migalastat. 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 license agreement with the Mount Sinai School of Medicine of New York University, 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 license, we have the right to enforce the licensed patent rights. Our existing license imposes, and we expect that future licenses will impose, various diligences, 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 product candidates, 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 product candidates, technology or methods.

We are aware, for example, of U.S. patents, and corresponding international counterparts, owned by third parties that contain claims related to treating protein misfolding. If any of these patents were to be asserted against us, while we do not believe that our product candidates would be found to infringe any valid claim of such 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 with respect to such patents, such license would be available to us on acceptable terms or at all. If we were to challenge the validity of any issued U.S. patent in court, we would need to overcome a presumption of validity that attaches to every patent. This burden is high and would require us to present clear and convincing evidence as to the invalidity of the patent's claims. There is no assurance that a court would find in our favor on infringement or validity.

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

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

requests, depositions or trial testimony. Disclosure of our confidential information and our involvement in intellectual property litigation could materially adversely affect our business.

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

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

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

Risks Related to Regulatory Approval of Our Product Candidates

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

Our product candidates, including migalastat , 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 preparing, submitting and maintaining 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. 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 migalastat for the treatment of Fabry disease in February 2004. We also obtained orphan medicinal product designation in the EU for migalastat in May 2006. 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 migalastat may be important to each of the product candidate's success. Even if we obtain orphan drug exclusivity for our products, we may not be able to maintain it. For example, if a competitive product that is the same drug as our product candidate is shown to be clinically superior to our product candidate or if we are unable to supply the market, 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.

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

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

We are highly dependent on John F. Crowley, our Chairman and Chief Executive Officer, Bradley L. Campbell, our President and Chief Operating Officer, William D. Baird, III, our Chief Financial Officer and Jay Barth, M.D., our Chief Medical Officer. These executives each have significant pharmaceutical industry experience. Mr. Crowley is a commissioned officer in the U.S. Navy (Reserve), and he may be called to active duty service at any time. The loss of Mr. Crowley for protracted military duty could materially adversely affect our business. The loss of the services of any of these executives might impede the achievement of our research, development and commercialization objectives and materially adversely affect our business. We do not maintain "key person" insurance on Mr. Crowley or on any of our other executive officers.

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

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

Risks Related to Our Common Stock

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

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

- establish a classified board of directors, and, as a result, not all directors are elected at one time;
- allow the authorized number of our directors to be changed only by resolution of our board of directors;
- limit the manner in which stockholders can remove directors from our board of directors;
- establish advance notice requirements for stockholder proposals that can be acted on at stockholder meetings and nominations to our board of directors;
- require that stockholder actions must be effected at a duly called stockholder meeting and prohibit actions by our stockholders by written consent;
- limit who may call stockholder meetings;
- authorize our board of directors to issue preferred stock, without stockholder approval, which could be used to institute a "poison pill" that would work to dilute the stock ownership of a potential hostile acquirer, effectively preventing acquisitions that have not been approved by our board of directors; and
- require the approval of the holders of at least 67% of the outstanding voting stock 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.

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;
- acquisitions of business or assets;
- 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 market value of their investment.

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

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

Our executive officers, directors and principal stockholders maintain the ability to exert significant influence and control over matters submitted to our stockholders for approval.

Our executive officers, directors and affiliated stockholders beneficially own shares representing approximately 24% of our common stock as of December 31, 2014. As a result, if these stockholders were to choose to act together, they would be able to exert significant influence and control over matters submitted to our stockholders for approval, as well as our management and affairs. For example, these persons, if they choose to act together, could influence 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.

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

None.

Item 2. ***PROPERTIES.***

We currently lease approximately 73,646 square feet of office and laboratory space in Cranbury, New Jersey and 7,700 square feet of office and laboratory space in San Diego, California under certain lease agreements. The initial term of the Cranbury, New Jersey lease expires February 2019 and may be extended by us for two additional five-year periods. The facility at San Diego, California, was closed as part of the restructuring process in December 2013. The lease for the San Diego, California location expires in September 2016. In May 2014, we entered into a sublease agreement with a tenant for the remainder of our original lease term for the San Diego, California facility. 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. ***MINE SAFETY DISCLOSURES.***

None.

PART II

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

Market For Our Common Stock

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

	High	Low
2014		
First Quarter	$ 3.08	$ 2.04
Second Quarter	$ 3.34	$ 1.82
Third Quarter	$ 7.47	$ 3.60
Fourth Quarter	$ 8.61	$ 5.39

	High	Low
2013		
First Quarter	$ 4.22	$ 2.64
Second Quarter	3.47	2.07
Third Quarter	2.83	2.18
Fourth Quarter	2.45	1.97

The closing price for our common stock as reported by the NASDAQ Global Market on February 24, 2015 was $8.48 per share. As of February 24, 2015, there were 31 holders of record of our common stock.

Dividends

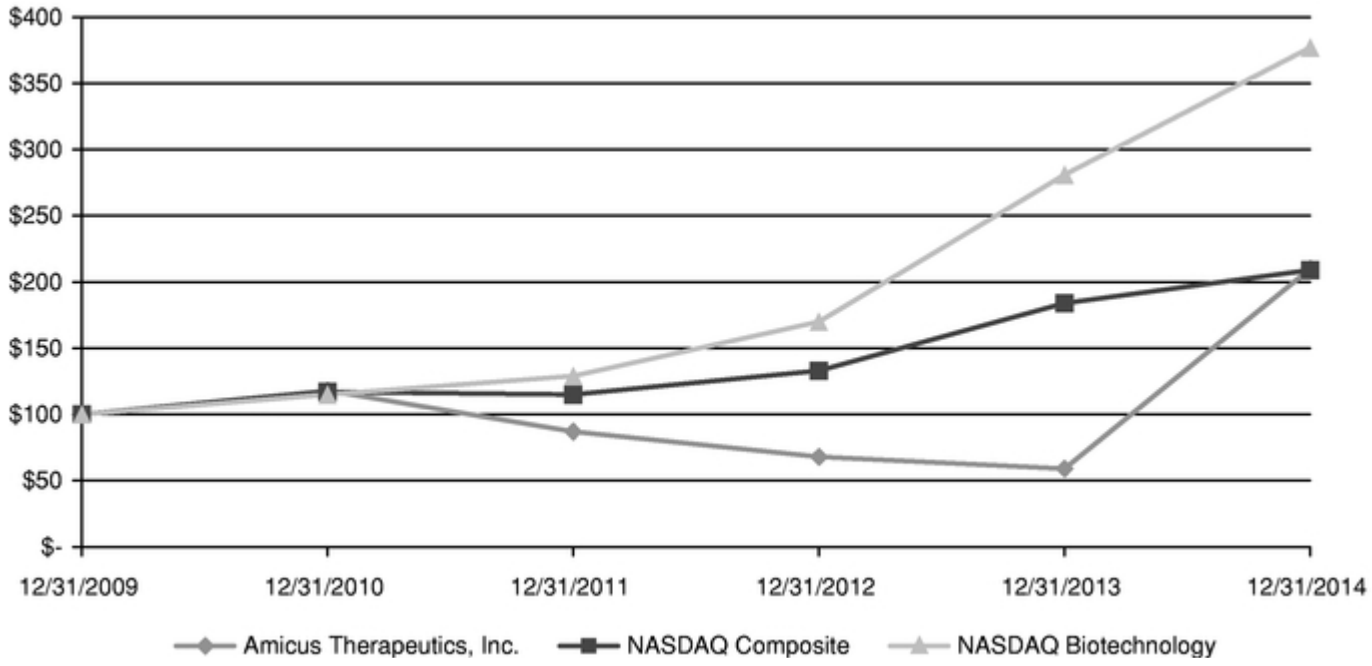
We have never declared or paid any dividends on our capital stock. We currently intend to retain any future earnings to finance our research and development efforts, the further development of our pharmacological chaperone technology and the expansion of our business. We do not intend to declare or pay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

Recent Sales of Unregistered Securities

None

Performance Graph

The following performance graph compares the cumulative total return on our common stock during the last five fiscal years with the NASDAQ Composite Index (U.S.) and the NASDAQ Biotechnology Index during the same period. The graph shows the value at the end of each of the last five fiscal years, of $100 invested in our common stock. 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.



	12/31/2009	12/31/2010	12/31/2011	12/31/2012	12/31/2013	12/31/2014
Amicus Therapeutics, Inc .	$ 100	$ 118	$ 87	$ 68	$ 59	$ 210
NASDAQ Composite	$ 100	$ 117	$ 115	$ 133	$ 184	$ 209
NASDAQ Biotechnology	$ 100	$ 115	$ 129	$ 170	$ 281	$ 377

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

Issuer Purchases of Equity Securities

The Company did not purchase any shares of its common stock for the three months ended December 31, 2014.

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

	Year Ended December 31,				
	2014	2013	2012	2011	2010
Statement of Operations Data:					
Revenue:					
Research revenue	$ 1,224	$ 363	$ 11,591	$ 14,794	$ —
Collaboration and milestone revenue	—	—	6,820	6,640	922
Total revenue	1,224	363	18,411	21,434	922
Operating expenses:					
Research and development	47,624	41,944	50,273	50,856	39,042
General and administrative	20,717	18,893	19,364	19,880	15,660
Changes in fair value of contingent consideration payable	100	—	—	—	—
Restructuring charges	(63)	1,988	—	—	—
Depreciation and amortization	1,547	1,719	1,705	1,585	2,058
Total operating expenses	69,925	64,544	71,342	72,321	56,760
Loss from operations	(68,701)	(64,181)	(52,931)	(50,887)	(55,838)
Other income (expenses):					
Interest income	223	174	316	160	156
Interest expense	(1,484)	(46)	(89)	(148)	(260)
Change in fair value of warrant liability	—	908	653	2,764	(1,410)
Other (expense) income	(77)	—	21	70	1,277
Loss before tax benefit	(70,039)	(63,145)	(52,030)	(48,041)	(56,075)
Income tax benefit	1,113	3,512	3,245	3,629	1,139
Net loss	$ (68,926)	$ (59,633)	$ (48,785)	$ (44,412)	$ (54,936)
Net loss attributable to common stockholders per common share — basic and diluted	$ (0.93)	$ (1.16)	$ (1.07)	$ (1.28)	$ (1.98)
Weighted-average common shares outstanding — basic and diluted	74,444,157	51,286,059	45,565,217	34,569,642	27,734,797

	As of December 31,				
	2014	2013	2012	2011	2010
Balance Sheet Data:					
Cash and cash equivalents and marketable securities	$ 169,139	$ 82,000	$ 99,122	$ 55,702	$ 107,445
Working capital	134,392	77,817	95,374	47,392	93,458
Total assets	209,967	127,563	110,088	69,795	112,552
Total liabilities	87,789	81,812	40,868	40,203	47,618
Accumulated deficit	(447,448)	(378,522)	(318,889)	(270,104)	(225,692)
Total stockholders' equity	$ 122,178	$ 45,751	$ 69,220	$ 29,592	$ 64,934

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

Overview

We are a biopharmaceutical company focused on the discovery, development and commercialization of next-generation medicines for a range of rare and orphan diseases, with a focus on improved therapies for lysosomal storage disorders ("LSDs"). Our lead product candidate is a small molecule that can be used as a monotherapy and in combination with enzyme replacement therapy ("ERT") for Fabry disease. Our development programs also include next-generation ERTs for LSDs, including Fabry disease, Pompe disease and Mucopolysaccharidosis Type I ("MPS I"). We believe that our platform technologies and our advanced product pipeline uniquely position us at the forefront of developing therapies for rare and orphan diseases.

Program Status

Our personalized medicine approach consists of an oral small molecule pharmacological chaperone monotherapy that is designed to bind to and stabilize a patient's own endogenous target protein. Patients with "amenable mutations" may respond based on their genetics. Our Chaperone-Advanced Replacement Therapy, or CHART™, platform combines chaperones with ERTs independent of a patient's own genetics. In each CHART program, a unique pharmacological chaperone is designed to bind to a specific therapeutic (exogenous) enzyme, stabilizing the enzyme in its properly folded and active form. This may allow for enhanced tissue uptake, greater lysosomal activity, more reduction of substrate, and the potential for lower immunogenicity.

Our Fabry franchise strategy is to develop the pharmacological chaperone migalastat HCl ("migalastat") for all patients with Fabry disease — as a monotherapy for patients with amenable mutations and in combination with ERT for all other patients.

Migalastat for Fabry Disease as a Monotherapy

We have completed two Phase 3 global registration studies (Study 011 and Study 012) of migalastat monotherapy and plan to submit marketing applications in 2015. We have reported Phase 3 data in both treatment naïve patients ("Study 011" or "FACETS") and enzyme replacement therapy ("ERT") switch patients ("Study 012" or "ATTRACT"). Positive results from these studies have shown that treatment with migalastat has resulted in reductions in disease substrate, stability of kidney function, reductions in cardiac mass, and improvement in gastrointestinal symptoms in patients with amenable mutations in a validated assay ("GLP HEK assay").

Study 011 was a 24-month study of Fabry disease patients naïve to or not receiving ERT, which investigated the safety and efficacy of oral migalastat. The study consisted of a 6-month double-blind, placebo-controlled period, a 6-month open-label period, and a 12-month open-label extension phase. Subjects completing Study 011 were eligible to continue treatment with migalastat in a long-term open-label extension ("Study 041"). 67 subjects (24 male) were enrolled. All subjects enrolled in Study 011 had amenable mutations in the clinical trial human embryonic kidney ("HEK") cell-based in vitro assay that was available at study initiation ("clinical trial assay"). Following the completion of enrollment, a GLP-validated HEK assay was developed with a third party to measure the criteria for amenability with more quality control and rigor ("GLP HEK assay"). Approximately 10% of mutations in the HEK database switched categorization between "amenable" and "non-amenable" when moving from the clinical trial assay to the GLP HEK assay. Therefore, there were changes in categorization from amenable to non-amenable in 17 of the 67 patients enrolled in Study 011.

Study 011 was designed to measure the reduction of the disease substrate (Globotriaosylceramide, or "GL-3") in the interstitial capillaries of the kidney following treatment with oral migalastat (150 mg

every other day). The 24-month study began with a 6-month double-blind, placebo-controlled treatment period, after which all patients were treated with migalastat for a 6-month open-label follow-up period, and a subsequent 12-month open-label extension phase. The study also measured clinical outcomes, including renal function, as secondary endpoints.

As previously reported, patients on migalastat experienced greater reductions in GL-3 as compared to placebo during the initial 6-month period; however, this difference was not statistically significant under the original analysis of the primary endpoint (responder analysis with a 50% reduction threshold at month 6). The variability and low levels of GL-3 at baseline contributed to a higher-than-anticipated placebo response at month 6.

Following the unblinding of the 6-month data, and while still blinded to the 12-month data, we reported the mean change in GL-3 from the baseline to month 6 as a post-hoc analysis in the subgroup of patients with GLP HEK-amenable mutations. This analysis showed a statistically significant reduction in GL-3 in the migalastat group compared to placebo. The mean change in GL-3 was identified as a more appropriate way to control for the variability in GL-3 levels in Study 011 and to measure the biological effect of migalastat.

Results from this subgroup analysis further support use of the GLP HEK assay in predicting responsiveness to migalastat. Following a Type C Meeting with the U.S. Food and Drug Administration ("FDA"), we revised the Statistical Analysis Plan to pre-specify the primary analysis at month 12 as the mean change in interstitial capillaries GL-3 in patients with GLP HEK amenable mutations.

Throughout 2014 and in early 2015 we announced positive 12- and 24-month data from Study 011 and longer-term data from Study 041 in patients with amenable mutations who were naïve to ERT. Top-line data were announced in April 2014 and data was presented to the scientific community at the American Society of Human Genetics (ASHG) in October 2014 and WORLDSymposium™ in February 2015. Highlights were as follows:

- Subjects who switched from placebo to migalastat after month 6 demonstrated a statistically significant reduction in disease substrate, or kidney interstitial capillary GL-3, at month 12 (p=0.013), and a statistically significant reduction of disease substrate in another important biomarker of disease, plasma lyso-Gb3. Subjects who remained on migalastat demonstrated a durable reduction in kidney interstitial capillary GL-3, as well as a durable reduction in lyso-Gb3.

- Kidney function, as measured by estimated glomerular filtration rate (eGFR) and iohexol measured GFR (mGFR), remained stable following 18-24 months of treatment with migalastat in Study 011. Kidney function, as measured by eGFR, continued to remain stable in patients receiving migalastat in Study 011 for at least 18 months and continuing migalastat treatment in Study 041 for an average of 32 months. mGFR was not collected in Study 041.

- Reduction in cardiac mass, as measured by left ventricular mass index (LVMi), was statistically significant following treatment with migalastat for up to 36 months (average of 22 months) in patients in Study 011 and 041.

- There was a significant decrease in diarrhea (unadjusted p=0.03) in patients treated with migalastat versus placebo during the 6-month double-blind phase (Stage 1). After 18-24 months of treatment with migalastat, significant improvements in diarrhea and indigestion were observed in addition to favorable trends in reflux and constipation. Gastrointestinal symptoms were assessed using the Gastrointestinal Symptoms Rating Scale (GSRS), a validated instrument

- Migalastat was generally safe and well-tolerated

Study 012, our second Phase 3 registration study, is a randomized, open-label 18-month study investigating the safety and efficacy of oral migalastat (150 mg, every other day) compared to

standard-of-care infused ERTs (agalsidase beta and agalsidase alfa). The study also includes a 12 month open-label migalastat extension phase. The study enrolled a total of 60 patients (males and females) with Fabry disease and genetic mutations identified as amenable to migalastat monotherapy in the clinical trial assay. Subjects were randomized 1.5:1 to switch to migalastat or remain on ERT. All subjects had been receiving ERT infusions for a minimum of 12 months (at least 3 months at the labeled dose) prior to entering the study. Based on the GLP HEK assay, there were changes in categorization from amenable to non-amenable in 4 of the 60 patients enrolled in Study 012.

Taking into account scientific advice from European regulatory authorities, the pre-specified co-primary outcome measures of efficacy in Study 012 are the descriptive assessments of comparability of the mean annualized change in mGFR and eGFR for migalastat and ERT. Both mGFR and eGFR are considered important measures of renal function. Success on mGFR and eGFR was prescribed to be measured in two ways: 1) a 50% overlap in the confidence intervals between the migalastat and ERT treatment groups; and 2) whether the mean annualized changes for patients receiving migalastat are within 2.2 mL/min/1.73 m2/yr of patients receiving ERT. We pre-specified that these renal function outcomes would be analyzed in patients with GLP HEK amenable mutations.

In August 2014, we announced positive 18-month data from the Study 012. Data from Study 012 were also presented to the scientific community at the American Society of Nephrology ("ASN") in November 2014 and WORLDSymposium in February 2015. Highlights were as follows:

- Migalastat had a comparable effect to ERT on patients' kidney function as measured by the change in eGFR and mGFR from baseline to month 18.
- Levels of plasma lyso-Gb3, an important biomarker of disease, remained low and stable in patients with amenable mutations who switched from ERT to migalastat.
- There was a statistically significant decrease in LVMi from baseline to month 18 in patients who switched from ERT to migalastat
- Measures of pain and quality of life from the Brief Pain Inventory ("BPI") and Short Form 36 ("SF36") remained stable when patients switched from ERT to migalastat.
- Migalastat was generally safe and well-tolerated.

Following a meeting with the European Medicines Agency ("EMA") held in the fourth quarter of 2014, we are on track to submit a marketing application in Europe in mid-2015. We have also scheduled a meeting with the US FDA in the first quarter of 2015 as we work to make migalastat available for all amenable Fabry patients as quickly as possible.

Migalastat Combination Programs for Fabry Disease

In support of our Fabry Franchise strategy to develop migalastat in combination with ERT for Fabry patients with non-amenable mutations, we plan to conduct a longer-term Phase 2 Fabry co-administration study in 2015. In parallel, we are internally developing our own Fabry cell line for co-formulation with migalastat as a next-generation ERT for Fabry disease. We previously completed an open-label Phase 2 safety and pharmacokinetics study ("Study 013") that investigated two oral doses of migalastat (150 mg and 450 mg) co-administered with agalsidase beta or agalsidase alfa in males with Fabry disease. Unlike Study 011 and Study 012, patients in Study 013 were not required to have alpha-Gal mutations amenable to chaperone therapy because, when co-administered with ERT, migalastat is designed to bind to and stabilize the exogenous enzyme in the circulation in any patient receiving ERT. Each patient received their current dose and regimen of ERT at one infusion. A single oral dose of migalastat (150 mg or 450 mg) was co-administered two hours prior to the next infusion of the same ERT at the same dose and regimen. Preliminary results from Study 013 showed increased

levels of active alpha-Gal A enzyme levels in plasma and skin following co-administration compared to ERT alone.

Next-Generation ERT for Pompe Disease

We are leveraging our biologics capabilities and CHART platform to develop a next-generation Pompe ERT. This ERT consists of a uniquely engineered rhGAA enzyme (ATB200) with an optimized carbohydrate structure to enhance uptake, administered in combination with a pharmacological chaperone to improve activity and stability. We acquired ATB200 as well as our enzyme targeting technology through our purchase of Callidus Biopharma.

In preclinical studies, ATB200 demonstrated greater tissue enzyme levels and further substrate reduction compared to the current approved ERT for Pompe disease (alglucosidase alfa), which were further improved with the addition of a chaperone. Clinical studies of pharmacological chaperones in combination with currently marketed ERTs have established initial human proof-of-concept that a chaperone can stabilize enzyme activity and potentially improve ERT tolerability. In 2013, we completed a Phase 2 safety and pharmacokinetics study ("Study 010") that investigated single ascending oral doses of a pharmacological chaperone co-administered with alglucosidase alfa marketed by Genzyme, in patients with Pompe disease. Each patient received one infusion of ERT alone, and then a single oral dose of the pharmacological chaperone just prior to the next ERT infusion. Results from this study showed an increase in GAA enzyme activity in plasma and muscle when co-administered compared to ERT alone.

Taken together, these preclinical and clinical results support further development of ATB200 in combination with a pharmacological chaperone as a next-generation Pompe ERT. The initiation of a Phase 1/2 clinical study is expected in the second half of 2015.

Collaborations

GSK

In November 2013, we entered into the Revised Agreement (the "Revised Agreement") with GSK, pursuant to which we obtained global rights to develop and commercialize migalastat as a monotherapy and in combination with ERT for Fabry disease. The Revised Agreement amends and replaces in its entirety the Expanded Agreement entered into between us and GSK in July 2012 (the" Expanded Collaboration Agreement"). Under the terms of the Revised Agreement, we obtained global commercial rights to migalastat, both as a monotherapy and co-formulated with ERT. For migalastat monotherapy, GSK is eligible to receive post-approval and sales-based milestones, as well as tiered royalties in the mid-teens in eight major markets outside the U.S. There was no other consideration paid to GSK as part of the Revised Agreement.

Biogen

In September 2013, we entered into a collaboration agreement with Biogen Idec ("Biogen") to discover, develop and commercialize novel small molecules that target the glucocerobrosidase ("GCase") enzyme for the treatment of Parkinson's disease. In September 2014, we concluded our research collaboration with Biogen. Our most advanced Parkinson's candidate is AT3375, which was developed outside the collaboration and is wholly-owned by us.

Other Potential Alliances and Collaborations

We continually evaluate other potential collaborations and business development opportunities that would bolster our ability to develop therapies for rare and orphan diseases including licensing agreements and acquisitions of businesses and assets. We believe such opportunities may be important

to the advancement of our current product candidate pipeline, the expansion of the development of our current technology, gaining access to new technologies and in our transformation to a commercial biotechnology company.

Acquisition of Callidus Biopharma, Inc.

In November 2013, we entered into a merger agreement (the "Merger Agreement") with Callidus Biopharma, Inc. ("Callidus"), a privately held biotechnology company. Callidus was engaged in developing a next-generation Pompe ERT and complementary enzyme targeting technologies.

In connection with our acquisition of Callidus, we agreed to issue an aggregate of 7.2 million shares of our common stock to the former stockholders of Callidus. In addition, we will be obligated to make additional payments to the former stockholders of Callidus upon the achievement of certain clinical milestones of up to $35 million and regulatory approval milestones of up to $105 million set forth in the merger agreement, provided that the aggregate merger consideration shall not exceed $130 million. We may, at our election, satisfy certain milestone payments identified in the merger agreement aggregating $40 million in shares of our common stock. The milestone payments not permitted to be satisfied in common stock (as well as any payments that we are permitted to, but chooses not to, satisfy in common stock), as a result of the terms of the merger agreement, will be paid in cash.

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

Revenue Recognition

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

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

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

We also consider the impact of potential future payments we make in our role as a vendor to our customers and evaluate if these potential future payments could be a reduction of revenue from that customer. If the potential future payments to the customer are:

- a payment for an identifiable benefit, and
- the identifiable benefit is separable from the existing relationship between us and our customer, and
- the identifiable benefit can be obtained from a party other than the customer, and
- the fair value of the identifiable benefit can be reasonably estimated,

then the payments are accounted for separately from the revenue received from that customer. If, however, all these criteria are not satisfied, then the payments are treated as a reduction of revenue from that customer.

If we determine that any potential future payments to our customers are to be considered as a reduction of revenue, we must evaluate if the total amount of revenue to be received under the arrangement is fixed and determinable. If the total amount of revenue is not fixed and determinable due to the uncertain nature of the potential future payments to the customer, then any customer payments cannot be recognized as revenue until the total arrangement consideration becomes fixed and determinable.

The reimbursements for research and development costs under collaboration agreements that meet the criteria for revenue recognition are included in Research Revenue and the costs associated with these reimbursable amounts are included in research and development expenses.

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

Research and Development Expenses

We expect to continue to incur substantial research and development expenses as we continue to develop our product candidates and explore new uses for our pharmacological chaperone technology. Research and development expense consists of:

- internal costs associated with our research and clinical development activities;

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

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

We expense research and development costs as incurred, including payments made to date under our license agreements. We believe that significant investment in product development is a competitive necessity and plan to continue these investments in order to realize the potential of our product candidates.

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,		
	2014	2013	2012
Projects			
Third party direct project expenses			
Monotherapy Studies			
Migalastat (Fabry Disease — Phase 3)	$ 13,567	$ 8,977	$ 14,718
Afegostat tartrate (Gaucher Disease — Phase 2*)	15	80	186
Combination Studies			
Fabry CHART (Fabry Disease — Phase 2)	1,050	623	2,689
Pompe CHART (Pompe Disease — Phase 2)	7,478	3,748	2,367
Gaucher CHART (Gaucher Disease — Preclinical)	—	21	—
Neurodegenerative Diseases (Preclinical)	280	144	417
Total third party direct project expenses	22,390	13,593	20,377
Other project costs [1]			
Personnel costs	18,366	20,257	21,086
Other costs [2]	6,868	8,094	8,810
Total other project costs	25,234	28,351	29,896
Total research and development costs	$ 47,624	$ 41,944	$ 50,273

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

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

* We do not plan to advance our afegostat tartrate monotherapy program into Phase 3 development at this time.

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

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

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

General and Administrative Expense

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

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 debt agreements.

Stock Option Grants

In accordance with the applicable guidance, we measure stock-based compensation at a fair value which is determined by measuring the cost of employee services received in exchange for an award of equity instruments based upon the grant date fair value of the award. We chose the "straight-line" attribution method for allocating compensation costs and recognized the fair value of each stock option on a straight-line basis over the vesting period of the related awards.

We use the Black-Scholes option pricing model when estimating the value for stock-based awards. Use of a valuation model requires management to make certain assumptions with respect to selected model inputs. Expected volatility was calculated based on a blended weighted average of historical information of our stock and the weighted average of historical information of similar public entities for which historical information was available. We will continue to use a blended weighted average approach using our own historical volatility and other similar public entity volatility information until our historical volatility is relevant to measure expected volatility for future option grants. The average expected life was determined using a "simplified" method of estimating the expected exercise term which is the mid-point between the vesting date and the end of the contractual term. As our stock price volatility has been over 75% and we have experienced significant business transactions, we believe that we do not have sufficient reliable exercise data in order to justify a change from the use of the "simplified" method of estimating the expected exercise term of employee stock option grants. 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 expected turnover 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,		
	2014	2013	2012
Expected stock price volatility	81.3%	82.0%	77.2%
Risk free interest rate	1.9%	1.3%	0.8%
Expected life of options (years)	6.25	6.25	6.25
Expected annual dividend per share	$ 0.00	$ 0.00	$ 0.00

Restricted Stock Units

In April 2014, the Compensation Committee made awards of restricted stock units ("RSUs") to certain employees of the Company. The RSUs were awarded under the Plan are generally subject to graded vesting of 50% of the RSUs on the 13 th month anniversary of the grant date and the remaining 50% of the RSUs on the 20 th month anniversary of the grant date, in each case, contingent on an employee's continued service on such date. RSUs are generally subject to forfeiture if employment terminates prior to the release of vesting restrictions. We expense the cost of the RSUs, which is determined to be the fair market value of the shares of common stock underlying the RSUs at the date of grant, ratably over the period during which the vesting restrictions lapse.

In April 2014, our Board of Director approved the Company's Restricted Stock Unit Deferral Plan (the "Deferred Compensation Plan"), which provides selected employees with an opportunity to defer receipt of RSUs until the first to occur of termination of the employee's employment or a date selected by the employee. Any RSUs deferred under the Deferred Compensation Plan would be fully vested once the original vesting conditions of the RSUs were satisfied.

Warrants

The warrants issued in connection with the March 2010 registered direct offering were being classified as a liability in the December 2013 and 2012 balance sheets. The fair value of the warrants liability was evaluated at each balance sheet date using the Black-Scholes valuation model. Any changes in the fair value of the warrants liability were recognized in the consolidated statement of operations. The warrants expired on March 2, 2014.

The warrants issued in connection with our 2013 SPA are classified as equity. As part of the 2013 SPA, a total of 7.5 million common shares and 1.6 million warrants were issued at $2.00 per share, for

total cash received of $15 million. The warrants are included in stockholder's equity and were initially measured at fair value of $1.0 million using the Black Scholes valuation model.

Business Combinations

We allocate the purchase price of acquired businesses to the tangible and intangible assets acquired and liabilities assumed based upon their estimated fair values on the acquisition date. The purchase price allocation process requires management to make significant estimates and assumptions, especially at the acquisition date with respect to intangible assets and in-process research and development ("IPR&D"). In connection with the purchase price allocations for acquisitions, we estimate the fair value of contingent acquisition consideration payments utilizing a probability-based income approach inclusive of an estimated discount rate.

Although we believe the assumptions and estimates made are reasonable, they are based in part on historical experience and information obtained from the management of the acquired businesses and are inherently uncertain. Examples of critical estimates in valuing any contingent acquisition consideration issued or which may be issued and the intangible assets we have acquired or may acquire in the future include but are not limited to:

- the feasibility and timing of achievement of development, regulatory and commercial milestones;
- expected costs to develop the in-process research and development into commercially viable products; and
- future expected cash flows from product sales.

Unanticipated events and circumstances may occur which may affect the accuracy or validity of such assumptions, estimates or actual results.

Intangible Assets and Goodwill

We record goodwill in a business combination when the total consideration exceeds the fair value of the net tangible and identifiable intangible assets acquired. Purchased in-process research and development is accounted for as an indefinite lived intangible asset until the underlying project is completed, at which point the intangible asset will be accounted for as a definite lived intangible asset, or abandoned, at which point the intangible asset will be written off or partially impaired. Goodwill and indefinite lived intangible assets are assessed annually for impairment on October 1 and whenever events or circumstances indicate that the carrying amount of an asset may not be recoverable. If it is determined that the full carrying amount of an asset is not recoverable, an impairment loss is recorded in the amount by which the carrying amount of the asset exceeds its fair value.

Valuation of Contingent Consideration Payable

Each period we reassess the fair value of the contingent acquisition consideration payable associated with certain acquisitions and record changes in the fair value as contingent consideration expense. Increases or decreases in the fair value of the contingent acquisition consideration payable can result from changes in estimated probability adjustments with respect to regulatory approval, changes in the assumed timing of when milestones are likely to be achieved and changes in assumed discount periods and rates. Significant judgment is employed in determining the appropriateness of these assumptions each period. Accordingly, future business and economic conditions, as well as changes in any of the assumptions described in the accounting for business combinations above can materially impact the amount of contingent consideration expense that we record in any given period.

Accrued Expenses

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

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

Nonqualified Cash Deferral Plan

In July 2014, our Board of Directors approved the Cash Deferral Plan (the "Deferral Plan"), which provides certain key employees and other service providers as selected by the Compensation Committee, with an opportunity to defer the receipt of such Participant's base salary, bonus and director's fees, as applicable. The Deferral Plan is intended to be a nonqualified deferred compensation plan that complies with the provisions of Section 409A of the Internal Revenue Code (the "Code").

As of December 31, 2014, the amounts deferred under the Deferral Plan have not been invested. The investments are expected to be made in the first quarter of fiscal year 2015. All of the investments held in the Deferral Plan will be classified as investments trading securities and recorded at fair value with changes in the investments' fair value recognized as earnings in the period they occur. The corresponding liability for the Deferral Plan is included in other non-current liability in our consolidated balance sheets.

Results of Operations

Year Ended December 31, 2014 Compared to Year Ended December 31, 2013

Revenue. We recognized reimbursements for research and development costs under the Biogen agreement as Research Revenue. For 2014, we recognized $1.2 million as Research Revenue as compared to $0.4 million in 2013 for reimbursed research and development costs.

Research and Development Expense. Research and development expense was $47.6 million in 2014, representing an increase of $5.7 million or 13.6% from $41.9 million in 2013. The increase in research and development costs was primarily due to an increase in contract manufacturing and research costs. Contract research increased by $3.9 million and contract manufacturing by $4.6 million due to timing of studies and changes in research plans for the Fabry CHART and the Pompe CHART programs. The Fabry migalastat program also saw increased spending due to the revised agreement where we were responsible for 100% of the Fabry program costs in 2014 as compared to 40% in 2013. These increases were offset by decreases in personnel costs of $1.9 million, consultants of $0.4 million and savings in facilities costs of $0.5 million.

General and Administrative Expense. General and administrative expense was $20.7 million in 2014, an increase of $1.8 million or 9.5% from $18.9 million in 2013. The increase was primarily due to personnel costs of $0.7 million, legal and filing fees of $0.7 million and consulting fees of $0.2 million.

Changes in Fair Value of Contingent Consideration Payable. For 2014, we recorded expense of $0.1 million representing an increase in fair value of contingent consideration payable, which was related to the Callidus acquisition.

Restructuring Charges. Adjustments to the restructuring liability were $0.1 million in 2014 and were due to the change in fair value of future minimum lease payments. Restructuring charges were $2.0 million in 2013 due to the corporate restructuring implemented in the fourth quarter of 2013. This measure was intended to reduce costs and to align our resources with our key strategic priorities.

Depreciation and Amortization. Depreciation and amortization expense was $1.5 million in 2014, representing a decrease of $0.2 million or 11.8% as compared to $1.7 million in 2013. The decrease was mainly due to asset disposals from closure of San Diego office in December 2013.

Interest Income. Interest income was $0.2 million in both 2014 and 2013.

Interest Expense. Interest expense was $1.5 million in 2014 as compared to $0.05 million in 2013. Interest expense was higher due to the $15 million loan secured in December 2013.

Change in Fair Value of Warrant Liability. In connection with the sale of our common stock and warrants from the registered direct offering in March 2010, we recorded the warrants as a liability at their fair value using a Black-Scholes model and remeasured the fair value at each reporting date until the warrants were exercised or expired. Changes in the fair value of the warrant liability were reported in the statements of operations as non-operating income or expense. As these warrants expired in March 2014, for the year ended December 31, 2014, there was no expense or income as compared to an income of $0.9 million related to the decrease in fair value of the warrant liability from the year ended December 31, 2013.

Other Income/Expense. Other income/expenses for 2014 included charges of $77 thousand for the increase in the fair value of the success fee payable, which was related to the $15 million secured loan in 2013. There was no other income/expense for 2013.

Tax Benefit. During 2014 and 2013, we sold a portion of our New Jersey state net operating loss carry forwards and research and development credits, which resulted in the recognition of $1.1 million and $3.5 million in income tax benefits for the years ended December 31, 2014 and 2013, respectively.

Net Operating Loss Carry forwards. As of December 31, 2014, we had federal and state net operating loss carry forwards, or NOLs, of approximately $268.5 million and $235.5 million, respectively. The federal carry forward will expire in 2028 through 2034. Most of the state carry forwards generated prior to 2009 began to expire in 2012 and will continue to expire through 2015. The remaining state carry forwards including those generated from 2009 through 2012 will expire in 2028 through 2034 due to a change in the New Jersey state law regarding the net operating loss carry forward period. Section 382 of the Code contains provisions which limit the amount of NOLs that companies may utilize in any one year in the event of cumulative changes in ownership over a three-year period in excess of 50%. We completed a detailed study of our NOLs and determined that there was an ownership change in excess of 50% and the federal NOLs subject to the 382 limitations were written down to their net realizable value. Additionally, we determined that the annual limitation on the utilization of the pre-ownership change loss will be approximately $3.0 million. Ownership changes in future periods may place additional limits on our ability to utilize net operating loss and tax credit carry forwards.

Year Ended December 31, 2013 Compared to Year Ended December 31, 2012

Revenue. For the year ended December 31, 2013, we recognized $0.4 million as Research Revenue for reimbursed research and development costs as a result of our collaboration with Biogen.

Under the Original License and Collaboration Agreement, GSK paid us an initial, non-refundable license fee of $30 million and a premium of $3.2 million related to GSK's purchase of an equity investment in Amicus which was being recognized as Collaboration and Milestone Revenue on a straight-line basis over the development period. In addition, in June 2012, we recognized a $3.5 million payment for a clinical development milestone as Collaboration and Milestone Revenue. For the year ended December 31, 2012 we recognized $6.8 million as Collaboration and Milestone Revenue.

The reimbursements for research and development costs under the Original License and Collaboration Agreement that met the criteria for revenue recognition were recognized as Research Revenue. For the year ended December 31, 2012, we recognized $11.6 million as Research Revenue.

In July 2012, we entered into the Expanded Collaboration Agreement with GSK. Due to a change in the accounting for revenue recognition for the Expanded Collaboration Agreement, all revenue recognition was suspended until the total arrangement consideration becomes fixed and determinable. As a result, we no longer recognized any revenue related to Collaboration and Milestone Revenue or Research Revenue as of the date of the Expanded Collaboration Agreement. There was no cash effect of this change in accounting, and there is no scenario where we would have to refund any of its upfront payments, milestone payments, or research reimbursement payments.

Research and Development Expense. Research and development expense was $41.9 million in 2013, representing a decrease of $8.4 million or 16.7% from $50.3 million in 2012. The variance was primarily attributable to an $8.1 million decrease in contract research, partially offset by manufacturing increases of $1.8 million. The decreases were mainly driven by a $7.8 million decrease in the Fabry migalastat study and the increase was from $1.0 million in the Pompe CHART study. Other decreases were in personnel costs of $0.8 million and license fees of $0.4 million.

General and Administrative Expense. General and administrative expense was $18.9 million in 2013, a decrease of $0.5 million or 2.6% from $19.4 million in 2012. The variance was primarily due to a decrease in personnel costs of $0.6 million, $0.2 million in consulting fees and $0.1 million in recruitment fees. These decreases were partially offset by increases of $0.2 million in tax assessments and $0.2 million in legal fees relating to business development activities.

Restructuring Charges. Restructuring charges were $2.0 million in 2013 due to the corporate restructuring implemented in the fourth quarter of 2013. This measure was intended to reduce costs and to align the Company's resources with its key strategic priorities. The restructuring charges included $1.2 million for employment termination costs payable in cash and a facilities consolidation restructuring charge of $0.8 million, consisting of lease payments of $0.7 million related to the net present value of the net future minimum lease payments at the cease-use date and the write-down of the net book value of fixed assets in the vacated building of $0.1 million.

Depreciation and Amortization. Depreciation and amortization expense was $1.7 million in both 2012 and 2013. There was no increase in depreciation and amortization expense due to less property, plant and equipment purchased in 2013 as compared to prior years.

Interest Income Interest income was $0.2 million in 2013, a decrease of $0.1 million or 33% from $0.3 million in 2012. The decrease in interest income was due to the overall lower average cash and investment balances in 2013, compared to 2012.

Interest Expense. Interest expense was $0.05 million in 2013, a decrease of $0.04 million or 44% from $0.09 million in 2012. The decrease was due to a lower outstanding debt balance for most of 2013, prior to the secured loan obtained in December 2013.

Change in Fair Value of Warrant Liability. In connection with the sale of our common stock and warrants from the registered direct offering in March 2010, we recorded the warrants as a liability at

their fair value using a Black-Scholes model and remeasure the fair value at each reporting date until the warrants are exercised or expired. Changes in the fair value of the warrant liability are reported in the statements of operations as non-operating income or expense. During 2012, there were approximately 0.5 million warrants exercised; there were no warrants exercised in 2013. For the year ended December 31, 2013, we reported a gain of $0.9 million related to the decrease in fair value of the warrant liability from the year ended December 31, 2012.

Other Income/Expense. There was no other income or other expense for the year ended December 31, 2013. Other income for the year ended December 31, 2012 was $21 thousand and represents cash received from the sale of property, plant and equipment.

Tax Benefit. During 2012 and 2013, we sold a portion of our New Jersey state net operating loss carry forwards and research and development credits, which resulted in the recognition of $3.2 million and $3.5 million in income tax benefits for the years ended December 31, 2012 and 2013, respectively.

Net Operating Loss Carry forwards. As of December 31, 2013, we had federal and state net operating loss carry forwards, or NOLs, of approximately $203.8 million and $179.9 million, respectively. The federal carry forward will expire in 2028 through 2032. Most of the state carry forwards generated prior to 2009 began to expire in 2012 and will continue to expire through 2015. The remaining state carry forwards including those generated from 2009 through 2012 will expire in 2028 through 2032 due to a change in the New Jersey state law regarding the net operating loss carry forward period. Section 382 of the Internal Revenue Code of 1986, as amended, contains provisions which limit the amount of NOLs that companies may utilize in any one year in the event of cumulative changes in ownership over a three-year period in excess of 50%. During 2013, there was no ownership change in excess of 50%; therefore there was no write-down to net realizable value of the federal NOLs subject to the 382 limitations.

Liquidity and Capital Resources

Sources of Liquidity

In November 2014, we sold a total of 15.9 million shares of our common stock at a public offering price of $6.50 per share. The offering generated gross proceeds of $103.5 million. After deducting the underwriting fee of $6.2 million and other offering expenses of $0.1 million, which included legal fees, the net proceeds of the offering were approximately $97.2 million. We expect to use the net proceeds of the offering for investment in the global commercialization infrastructure for migalastat monotherapy for Fabry disease, the continued clinical development of its product candidates and for other general corporate purposes.

In March 2014, we entered into a Sales Agreement with Cowen and Company, LLC ("Cowen") to create an at-the-market ("ATM") equity program under which we would from time to time offer and sell shares of our common stock having an aggregate offering price of up to $40 million ("ATM Shares") through Cowen. Upon delivery of a placement notice and subject to the terms and conditions of the ATM agreement, Cowen used its commercially reasonable efforts to sell the ATM Shares, based upon our instructions. Cowen was entitled to a commission at a fixed commission rate of up to 3.0% of the gross proceeds per ATM Share sold. Sales of the ATM Shares under the Agreement were made in transactions that were deemed to be "at the market offerings" as defined in Rule 415 under the Securities Act, including sales made by means of ordinary brokers' transactions, including on The NASDAQ Global Market, at market prices or as otherwise agreed with Cowen. We began the sales of ATM Shares in May 2014 and sold 14.3 million shares of common stock resulting in net proceeds of $38.6 million, which included Cowen's commission of $1.1 million and legal fees of $0.3 million. All sales under the ATM equity program have been completed.

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, $317.6 million of gross proceeds from our stock offerings, $130.0 million from investments by collaborators and non-refundable license fees from those collaborations.

In December 2013, we entered into a credit and security agreement with a lending syndicate which provided an aggregate of $25 million credit available. We drew $15 million of the aggregate principal amount in December 2013 and did not draw the additional $10 million that was available through the end of the fourth quarter of 2014.

As of December 31, 2014, we had cash and cash equivalents and marketable securities of $169.1 million. We invest cash in excess of our immediate requirements with regard to liquidity and capital preservation in a variety of interest-bearing instruments, including obligations of U.S. government agencies and money market accounts. Wherever possible, we seek to minimize the potential effects of concentration and degrees of risk. Although we maintain cash balances with financial institutions in excess of insured limits, we do not anticipate any losses with respect to such cash balances.

Net Cash Used in Operating Activities

Net cash used in operations for the year ended December 31, 2014 was $51.7 million due primarily to the net loss for the year ended December 31, 2014 of $68.9 million and the change in operating assets and liabilities of $9.4 million. The change in operating assets and liabilities consisted of a decrease in receivables from collaboration agreements of $1.1 million; a decrease of $2.3 million in prepaid assets primarily related to a receivable from the 2013 sale of state net operating loss carry forwards, or NOLs; and an increase in accounts payable and accrued expenses of $6.2 million, mainly related to program expenses.

Net cash used in operations for the year ended December 31, 2013 was $45.8 million due primarily to the net loss for the year ended December 31, 2013 of $59.6 million and the change in operating assets and liabilities of $4.8 million. The change in operating assets and liabilities consisted of a decrease in receivables from collaboration agreements of $2.1 million; an increase of $2.9 million in prepaid assets primarily related to a receivable from the 2013 sale of state net operating loss carry forwards, or NOLs; an increase in deferred reimbursements of $6.3 million due to the deferral of all revenue as a result of the Expanded Collaboration Agreement with GSK; and a decrease in accounts payable and accrued expenses of $0.7 million, mainly related to program expenses.

Net Cash Used in and Provided by Investing Activities

Net cash used in investing activities for the year ended December 31, 2014 was $107.1 million. Our investing activities have consisted primarily of purchases and sales and maturities of investments and capital expenditures. Net cash used in investing activities reflects $162.8 million for the purchase of marketable securities, $0.2 million for the acquisition of property and equipment, partially offset by $55.9 million for the sale and redemption of marketable securities.

Net cash provided by investing activities for the year ended December 31, 2013 was $26.1 million. Our investing activities have consisted primarily of purchases and sales and maturities of investments and capital expenditures. Net cash used in investing activities reflects $83.3 million for the sale and redemption of marketable securities partially offset by $56.6 million for the purchase of marketable securities and $0.7 million for the acquisition of property and equipment.

Net Cash Provided by Financing Activities

Net cash provided by financing activities for the year ended December 31, 2014 was $139.2 million and reflects $135.8 million in proceeds from the issuance of common stock and $3.7 million from exercise of stock options, partially offset by $0.3 million on payment on our secured loan agreement.

Net cash provided by financing activities for the year ended December 31, 2013 was $29.4 million and reflects $15.0 million in proceeds from the issuance of common stock, $14.9 million in proceeds from secured loan arrangement, partially offset by the $0.4 million in payments of our secured loan agreement and $0.1 million in deferred financing costs.

Funding Requirements

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

- the progress and results of our clinical trials of our drug candidates, including migalastat;
- the cost of manufacturing drug supply for our clinical and preclinical studies, including the significant cost of new ERT cell line development and manufacturing as well as the cost of manufacturing Pompe ERT;
- the scope, progress, results and costs of preclinical development, laboratory testing and clinical trials for our product candidates including those testing the use of pharmacological chaperones co-formulated and co-administered with ERT and for the treatment of lysosomal storage diseases;
- 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 or technologies; and
- our ability to establish collaborations and obtain milestone, royalty or other payments from any such collaborators.

We do not anticipate that we will generate revenue from commercial sales until at least 2016, if at all. In the absence of additional funding, we expect our continuing operating losses to result in increases in our cash used in operations over the next several quarters and years. We may seek additional funding through public or private financings of debt or equity. We believe that our existing cash and cash equivalents and short-term investments will be sufficient to fund the current operating plan into 2017.

Financial Uncertainties Related to Potential Future Payments

Milestone Payments / Royalties

Under our license agreements, if we owe royalties on net sales for one of our products to more than one licensor, then we have the right to reduce the royalties owed to one licensor for royalties paid to another. The amount of royalties to be offset is generally limited in each license and can vary under each agreement.

Under the Revised Agreement, GSK is eligible to receive post-approval and sales-based milestones, as well as tiered royalties in the mid-teens in eight major markets outside the U.S. for migalastat. In addition, because we reacquired worldwide rights to migalastat, we are no longer eligible to receive any milestones or royalties we would have been eligible to receive under the Original Collaboration Agreement. We will owe royalties to Mt. Sinai School of Medicine ("MSSM") in addition to those owed to GSK.

To date, we have not made any royalty payments on sales of our products.

Contractual Obligations

The following table summarizes our significant contractual obligations and commercial commitments at December 31, 2014 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	$ 7,916	$ 2,033	$ 3,808	$ 2,075	$ —
Debt obligations	15,450	4,035	11,415	—	—
Total fixed contractual obligations [1]	$ 23,366	$ 6,068	$ 15,223	$ 2,075	$ —

(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 December 2013, we entered into a credit and security agreement with a lending syndicate consisting of MidCap Funding III, LLC, Oxford Finance LLC, and Silicon Valley Bank which provides an aggregate of $25 million (the "Term Loan"). We drew $15 million of the aggregate principal amount of the Term Loan at the end of December 2013 (the "First Tranche") and did not draw on the additional $10 million, which was available to us through December 2014 (the "Second Tranche"). The principal outstanding balance of the First Tranche bears interest at a rate per annum fixed at 8.5%. We made interest-only payments on the Term Loan beginning January 1, 2014 and will continue through April 1, 2015, after which we will repay the aggregate principal outstanding balance of the Term Loan in 33 equal monthly installments of principal, plus accrued interest at the applicable rate. The Term Loan matures on December 27, 2017. The Term Loan contains customary terms and conditions which we have at all times been in compliance with throughout the duration of the Term Loan. We also recorded payments made and a contingent payable to the lenders at December 31, 2014. These payments include a debt facility fee of $0.1 million which was paid on the date of the First Tranche, $0.4 million exit fee that will be payable upon repayment of the term loan and $0.3 million representing the fair value of a contingent payment of up to $0.4 million related to a success fee payable within six months of trigger event, with the trigger event being regulatory acceptance of NDA or MAA submission. This is effective five years from the closing of the Term Loan. The success fee payable to the lender was probability adjusted and discounted utilizing an appropriate discount rate. We have included in the debt obligations of the contractual obligations above, the principal payments and the exit fees that we are contractually obligated to pay.

In April 2014, we entered into a revised employment agreement with our chairman and chief executive officer, John F. Crowley replacing his June 2011 employment agreement. The new agreement provides for an annual base salary, a cash bonus of up to 60% of base salary, and monthly payments up

to an annual maximum of $0.8 million in 2014 for out of pocket medical expenses, and the corresponding tax gross-up payments. The remaining terms of the revised employment agreement are substantially similar to Mr. Crowley's prior employment agreement. The revised agreement will continue for successive one-year terms until either party provides written notice of termination to the other in accordance with the terms of the agreement.

We currently lease laboratory and office space in Cranbury, New Jersey. The initial term of the lease, which commenced in March 2012, runs for seven years and may be extended for two additional five-year periods. The facility at San Diego, California, was closed as part of the restructuring process in December 2013, but we will continue to make payments until lease expires in September 2016. In May 2014, we entered into a sublease agreement with a tenant for the remainder of our original lease term for the San Diego, California facility.

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

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

Off-Balance Sheet Arrangements

We had no off-balance sheet arrangements as of December 31, 2014 and 2013.

Recent Accounting Pronouncements

In November 2014, the FASB issued ASU 2014-17, *Business Combinations (Topic 805): Pushdown Accounting* . The amendments in ASU 2014-17 provide an acquired entity with an option to apply pushdown accounting in its separate financial statements upon occurrence of an event in which an acquirer obtains control of the acquired entity. The ASU is effective on November 18, 2014. After the effective date, an acquired entity can make an election to apply the guidance to future change-in-control events or to its most recent change-in-control event. However, if the financial statements for the period in which the most recent change-in-control event occurred already have been issued or made available to be issued, the application of this guidance would be a change in accounting principle. We are currently assessing the impact that this standard will have on our consolidated financial statements.

In August 2014, the FASB issued ASU 2014-15, *Presentation of Financial Statements-Going Concern (Subtopic 205-40): Disclosure of Uncertainties about an Entity's Ability to Continue as a Going Concern* , which defines management's responsibility to assess an entity's ability to continue as a going concern, and to provide related footnote disclosures if there is substantial doubt about its ability to continue as a going concern. The pronouncement is effective for annual reporting periods ending after December 15, 2016 with early adoption permitted. The adoption of this guidance is not expected to have a significant impact on our consolidated financial statements.

In June 2014, the FASB issued ASU 2014-10 that removes the definition of development stage entity from the accounting standards codification, thereby removing the financial reporting distinction between development stage entities and other reporting entities from U.S. GAAP. In addition, the ASU eliminates the requirements for development stage entities to (i) present inception-to-date information in the statement of income, cash flow and stockholders' equity, (ii) label the financial statements as those of a development stage entity, (iii) disclose a description of the development stage activities in

which the entity is engaged, and (iv) disclose in the first year in which the entity is no longer a development stage entity that in prior years it had been in the development stage. We applied the ASU effective from the financial statements as of June 30, 2014.

In May 2014, FASB issued ASU 2014-09, *Revenue From Contracts With Customers* , that outlines a single comprehensive model for entities to use in accounting for revenue arising from contracts with customers and supersedes most current revenue recognition guidance, including industry-specific guidance. The ASU is based on the principle that an entity should recognize revenue to depict the transfer of goods or services to customers in an amount that reflects the consideration to which the entity expects to be entitled in exchange for those goods or services. The ASU also requires additional disclosure about the nature, amount, timing and uncertainty of revenue and cash flows arising from customer contracts, including significant judgments and changes in judgments and assets recognized from costs incurred to fulfill a contract. Entities have the option of using either a full retrospective or a modified retrospective approach for the adoption of the new standard. The ASU becomes effective for us at the beginning of its 2017 fiscal year; early adoption is not permitted. We are currently assessing the impact that this standard will have on its consolidated financial statements.

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

Market risk is the risk of change in fair value of a financial instrument due to changes in interest rates, equity prices, creditworthiness, financing, exchange rates or other factors. Our primary market risk exposure relates to changes in interest rates in our cash, cash equivalents and marketable securities. We place our investments in high-quality financial instruments, primarily money market funds, corporate debt securities, asset backed securities and U.S. government agency notes with maturities of less than one year, which we believe are subject to limited interest rate and credit risk. The securities in our investment portfolio are not leveraged, are classified as available-for-sale and, due to the short-term nature, are subject to minimal interest rate risk. We currently do not hedge interest rate exposure and consistent with our investment policy, we do not use derivative financial instruments in our investment portfolio. At December 31, 2014, we held $169.1 million in cash, cash equivalents and available for sale securities and due to the short-term maturities of our investments, we do not believe that a 10% change in average interest rates would have a significant impact on our interest income. As December 31, 2014, our cash, cash equivalents and available for sale securities were all due on demand or within one year. Our outstanding debt has a fixed interest rate and therefore, we have no exposure to interest rate fluctuations.

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

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

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

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

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

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

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

We assessed the effectiveness of our internal control over financial reporting as of December 31, 2014. In making this assessment, we used the criteria set forth by the Committee of Sponsoring Organizations of the Treadway Commission (2013 framework) ("COSO") in Internal Control — Integrated Framework. Based on our assessment we believe that, as of December 31, 2014, our internal control over financial reporting is effective based on those criteria.

The effectiveness of the Company's internal control over the financial reporting as of December 31, 2014 has been audited by Ernst & Young LLP, an independent registered public accounting firm, as stated in their report. This report appears on the following page.

Dated March 3, 2015

/s/ JOHN F. CROWLEY	/s/ WILLIAM D. BAIRD III
Chairman and Chief Executive Officer	Chief Financial Officer

Report of Independent Registered Public Accounting Firm

The Board of Directors and Stockholders of
Amicus Therapeutics, Inc.

We have audited Amicus Therapeutics, Inc.'s internal control over financial reporting as of December 31, 2014, based on criteria established in Internal Control — Integrated Framework issued by the Committee of Sponsoring Organizations of the Treadway Commission (2013 framework)(the "COSO criteria"). Amicus Therapeutics, Inc.'s management is responsible for maintaining effective internal control over financial reporting, and for its assessment of the effectiveness of internal control over financial reporting included in the accompanying report on consolidated financial statements and internal control over financial reporting. Our responsibility is to express an opinion on the company's internal control over financial reporting based on our audit.

We conducted our audit 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 effective internal control over financial reporting was maintained in all material respects. Our audit included obtaining an understanding of internal control over financial reporting, assessing the risk that a material weakness exists, testing and evaluating the design and operating effectiveness of internal control based on the assessed risk, and performing such other procedures as we considered necessary in the circumstances. We believe that our audit provides a reasonable basis for our opinion.

A company's internal control over financial reporting is a process designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles. A company's internal control over financial reporting includes those policies and procedures that (1) pertain to the maintenance of records that, in reasonable detail, accurately and fairly reflect the transactions and dispositions of the assets of the company; (2) provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that receipts and expenditures of the company are being made only in accordance with authorizations of management and directors of the company; and (3) provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use, or disposition of the company's assets that could have a material effect on the financial statements.

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

In our opinion, Amicus Therapeutics, Inc. maintained, in all material respects, effective internal control over financial reporting as of December 31, 2014, based on the COSO criteria.

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States), the consolidated balance sheets of Amicus Therapeutics, Inc. as of December 31, 2014 and 2013, and the related consolidated statements of operations, comprehensive loss, changes in stockholders' equity and cash flows for each of the three years in the period ended December 31, 2014 of Amicus Therapeutics, Inc., and our report dated March 3, 2015 expressed an unqualified opinion thereon.

/s/ Ernst & Young LLP

MetroPark, New Jersey
March 3, 2015

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. as of December 31, 2014 and 2013, and the related consolidated statements of operations, comprehensive loss, changes in stockholders' equity and cash flows for each of the three years in the period ended December 31, 2014. These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on these financial statements based on our audits.

We conducted our audits in accordance with the standards of the Public Company Accounting Oversight Board (United States). Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement. An audit includes examining, on a test basis, evidence supporting the amounts and disclosures in the financial statements. An audit also includes assessing the accounting principles used and significant estimates made by management, as well as evaluating the overall financial statement presentation. We believe that our audits provide a reasonable basis for our opinion.

In our opinion, the financial statements referred to above present fairly, in all material respects, the consolidated financial position of Amicus Therapeutics, Inc. at December 31, 2014 and 2013, and the consolidated results of its operations and its cash flows for each of the three years in the period ended December 31, 2014 in conformity with U.S. generally accepted accounting principles.

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States), Amicus Therapeutics, Inc.'s internal control over financial reporting as of December 31, 2014, based on criteria established in Internal Control — Integrated Framework issued by the Committee of Sponsoring Organizations of the Treadway Commission (2013 framework) and our report dated March 3, 2015 expressed an unqualified opinion thereon.

/s/ Ernst & Young LLP

MetroPark, New Jersey
March 3, 2015

Amicus Therapeutics, Inc.

Consolidated Balance Sheets
(in thousands, except share and per share amounts)

	December 31, 2014	December 31, 2013
Assets:		
Current assets:		
Cash and cash equivalents	$ 24,074	$ 43,640
Investments in marketable securities	127,601	38,360
Receivable due from collaboration agreements	—	1,083
Prepaid expenses and other current assets	2,902	5,195
Total current assets	154,577	88,278
Investments in marketable securities	17,464	—
Property and equipment, less accumulated depreciation and amortization of $11,520 and $9,973 at December 31, 2014 and 2013, respectively	2,811	4,120
In-process research & development	23,000	23,000
Goodwill	11,613	11,613
Other non-current assets	502	552
Total Assets	$ 209,967	$ 127,563
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable and accrued expenses	$ 16,345	$ 10,162
Current portion of secured loan	3,840	299
Total current liabilities	20,185	10,461
Deferred reimbursements	36,620	36,677
Secured loan, less current portion	10,510	14,174
Contingent consideration payable	10,700	10,600
Deferred tax liability	9,186	9,186
Other non-current liability	588	714
Commitments and contingencies		
Stockholders' equity:		
Common stock, $.01 par value, 125,000,000 shares authorized, 95,556,277 shares issued and outstanding at December 31, 2014 61,975,416 shares issued and outstanding at December 31, 2013,	1,015	679
Additional paid-in capital	568,743	423,593
Accumulated other comprehensive income	(132)	1
Accumulated deficit	(447,448)	(378,522)
Total stockholders' equity	122,178	45,751
Total Liabilities and Stockholders' Equity	$ 209,967	$ 127,563

See accompanying notes to consolidated financial statements

Amicus Therapeutics, Inc.

Consolidated Statements of Operations
(in thousands, except share and per share amounts)

	Years Ended December 31,		
	2014	2013	2012
Revenue:			
Research revenue	$ 1,224	$ 363	$ 11,591
Collaboration and milestone revenue	—	—	6,820
Total revenue	1,224	363	18,411
Operating Expenses:			
Research and development	47,624	41,944	50,273
General and administrative	20,717	18,893	19,364
Changes in fair value of contingent consideration payable	100	—	—
Restructuring charges	(63)	1,988	—
Depreciation and amortization	1,547	1,719	1,705
Total operating expenses	69,925	64,544	71,342
Loss from operations	(68,701)	(64,181)	(52,931)
Other income (expenses):			
Interest income	223	174	316
Interest expense	(1,484)	(46)	(89)
Change in fair value of warrant liability	—	908	653
Other (expense)/income	(77)	—	21
Loss before income tax benefit	(70,039)	(63,145)	(52,030)
Income tax benefit	1,113	3,512	3,245
Net loss attributable to common stockholders	$ (68,926)	$ (59,633)	$ (48,785)
Net loss attributable to common stockholders per common share — basic and diluted	$ (0.93)	$ (1.16)	$ (1.07)
Weighted-average common shares outstanding — basic and diluted	74,444,157	51,286,059	45,565,217

Amicus Therapeutics, Inc.

Consolidated Statements of Comprehensive Loss
(in thousands, except share and per share amounts)

	Years Ended December 31,		
	2014	2013	2012
Net loss	$ (68,926)	$ (59,633)	$ (48,785)
Other comprehensive income/(loss):			
Unrealized (loss)/ gain on available-for-sale securities	(133)	(13)	10
Other comprehensive (loss)/income before income taxes	(133)	(13)	10
Provision for income taxes related to other comprehensive (loss)/income items [(a)]	—	—	—
Other comprehensive (loss)/income	$ (133)	$ (13)	$ 10
Comprehensive loss	$ (69,059)	$ (59,646)	$ (48,775)

(a) — Taxes have not been accrued on unrealized gain on securities as the Company is in a loss position for all periods presented.

Amicus Therapeutics, Inc.

Consolidated Statements of Changes in Stockholders' Equity
For the Years ended December 31, 2012, 2013 and 2014
(in thousands, except share amounts)

	Common Stock		Additional Paid-In Capital	Other Comprehensive Gain/ (Loss)	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balance at December 31, 2011	34,654,206	$ 407	$ 299,285	$ 4	$ (270,104)	$ 29,592
Stock issued from exercise of stock options, net	436,952	4	1,626	—	—	1,630
Stock issued from exercise of warrants	90,933	1	386	—	—	387
Stock issued from collaboration agreement	2,949,581	29	18,111	—	—	18,140
Stock issued from public offering	11,500,000	115	61,940	—	—	62,055
Stock-based compensation	—	—	6,191	—	—	6,191
Unrealized holding gain on available-for-sale securities	—	—	—	10	—	10
Net loss	—	—	—	—	(48,785)	(48,785)
Balance at December 31, 2012	49,631,672	$ 556	$ 387,539	$ 14	$ (318,889)	$ 69,220
Stock and warrants issued in financing	7,500,000	75	14,925	—	—	15,000
Stock issued for Callidus acquisition	4,843,744	48	14,952	—	—	15,000
Stock-based compensation	—	—	6,177	—	—	6,177
Unrealized holding loss on available-for-sale securities	—	—	—	(13)	—	(13)
Net loss	—	—	—	—	(59,633)	(59,633)
Balance at December 31, 2013	61,975,416	$ 679	$ 423,593	$ 1	$ (378,522)	$ 45,751
Stock issued from exercise of stock options, net	965,544	10	3,663	—	—	3,673
Stock issued for Callidus acquisition	2,359,593	24	(24)	—	—	—
Stock issued from public offering	15,927,500	159	97,010	—	—	97,169
Stock issued from ATM transactions	14,328,224	143	38,493	—	—	38,636
Stock-based compensation	—	—	6,008	—	—	6,008
Unrealized holding loss on available-for-sale securities	—	—	—	(133)	—	(133)
Net loss	—	—	—	—	(68,926)	(68,926)
Balance at December 31, 2014	95,556,277	$ 1,015	$ 568,743	$ (132)	$ (447,448)	$ 122,178

Amicus Therapeutics, Inc.

Consolidated Statements of Cash Flows
(in thousands)

	Years Ended December 31,		
	2014	2013	2012
Operating activities			
Net loss	$ (68,926)	$ (59,633)	$ (48,785)
Adjustments to reconcile net loss to net cash used in operating activities:			
Non-cash interest expense	277	—	—
Depreciation and amortization	1,547	1,719	1,705
Stock-based compensation	6,008	6,177	6,191
Restructuring charges	(63)	1,988	—
Change in fair value of warrant liability	—	(908)	(653)
Non-cash changes in the fair value of contingent consideration payable	100	—	—
Loss on disposal of asset	—	—	28
Changes in operating assets and liabilities:			
Receivable due from collaboration agreements	1,083	2,142	1,818
Prepaid expenses and other current assets	2,293	(2,925)	3,633
Other non-current assets	26	—	267
Account payable and accrued expenses	6,169	(613)	(863)
Non-current liabilities	(126)	—	—
Deferred reimbursements	(57)	6,259	2,915
Net cash used in operating activities	(51,669)	(45,794)	(33,744)
Investing activities			
Sale and redemption of marketable securities	55,914	83,337	83,352
Purchases of marketable securities	(162,752)	(56,559)	(118,459)
Purchases of property and equipment	(238)	(695)	(4,324)
Net cash (used in)/ provided by investing activities	(107,076)	26,083	(39,431)
Financing activities			
Proceeds from issuance of common stock and warrants, net of issuance costs	135,805	15,000	80,195
Payments of secured loan agreement	(299)	(398)	(1,342)
Payments related to deferred financing	—	(110)	—
Proceeds from exercise of stock options	3,673	—	1,630
Proceeds from secured loan agreement	—	14,888	995
Net cash provided by financing activities	139,179	29,380	81,478
Net (decrease)/ increase in cash and cash equivalents	(19,566)	9,669	8,303
Cash and cash equivalents at beginning of year/ period	43,640	33,971	25,668
Cash and cash equivalents at end of year/period	$ 24,074	$ 43,640	$ 33,971
Supplemental disclosures of cash flow information			
Cash paid during the period for interest	$ 1,186	$ 30	$ 84
Non-cash activities			
Conversion of warrants to common stock	$ —	$ —	$ 386

Amicus Therapeutics, Inc.

Notes To Consolidated Financial Statements

1. Description of Business

Corporate Information, Status of Operations, and Management Plans

Amicus Therapeutics, Inc. (the "Company") was incorporated on February 4, 2002 in Delaware and is a biopharmaceutical company focused on the discovery, development and commercialization of next-generation medicines for a range of rare and orphan diseases, with a focus on improved therapies for lysosomal storage diseases ("LSDs"). The Company's lead product candidate is a small molecule that can be used as a monotherapy and in combination with enzyme replacement therapy ("ERT") for Fabry disease. The Company's development programs also include next-generation ERTs for LSDs, including Fabry disease, Pompe disease and Mucopolysaccharoidosis Type I (MPS I). The Company's activities since inception have consisted principally of raising capital, establishing facilities, and performing research and development.

Throughout 2014 and in early 2015 we announced positive 12- and 24-months results from Study 011 and longer-term data from Study 041 in patients with amenable mutations who were naïve to ERT. Following a meeting with the European Medicines Agency ("EMA") held in the fourth quarter of 2014, the Company is on track to submit a marketing application in Europe in mid-2015. The Company has also scheduled a meeting with the US FDA in the first quarter of 2015 as it works to make migalastat available for all amenable Fabry patients as quickly as possible.

In November 2014, the Company issued a total of 15.9 million shares through public offering at a price of $6.50 per share, with net proceeds of $97.2 million. The Company expects to use the net proceeds of the offering for investment in the global commercialization infrastructure for migalastat monotherapy for Fabry disease, the continued clinical development of its product candidates and for other general corporate purposes.

In July 2014, the Company completed a $40 million at the market ("ATM") equity offering under which the Company sold shares of its common stock, par value $0.01 per share, with Cowen and Company LLC as sales agent. Under the ATM equity program, the Company sold 14.3 million shares of common stock raising approximately $38.6 million in net proceeds. For further information on the ATM Agreement, see "— Note 9. Stockholder's Equity".

In November 2013, the Company completed the acquisition of Callidus Biopharma, Inc. ("Callidus"). Callidus was a privately-held biologics company focused on developing best-in-class ERTs for lysosomal storage diseases LSDs. Callidus lead ERT is a recombinant human acid-alpha glucosidase (rhGAA, called "ATB200") for Pompe disease in late preclinical development. For further information, see "— Note 3. Acquisition of Callidus Biopharma, Inc."

In November 2013, the Company entered into the Revised Agreement (the "Revised Agreement") with GlaxoSmithKline plc ("GSK"), pursuant to which Amicus has obtained global rights to develop and commercialize migalastat as a monotherapy and in combination with ERT for Fabry disease. The Revised Agreement amends and replaces in its entirety the Expanded Agreement entered into between Amicus and GSK in July 2012 (the "Expanded Collaboration Agreement"). Under the terms of the Revised Agreement, Amicus obtained global commercial rights to migalastat, both as a monotherapy and co-formulated with ERT. For migalastat monotherapy, GSK is eligible to receive post-approval and sales-based milestones, as well as tiered royalties in the mid-teens in eight major markets outside the U.S. There was no other consideration paid to GSK as part of the Revised Agreement.

In November 2013, the Company entered into a securities purchase agreement (the "2013 SPA") with GSK and certain entities controlled by Redmile Group, LLC for the private placement of a

Amicus Therapeutics, Inc.

Notes To Consolidated Financial Statements — (Continued)

combination of shares of the Company's common stock and warrants to purchase shares of the Company's common stock. The warrants have a term of one year and are exercisable between July 1, 2014 and June 30, 2015 at an exercise price of $2.50 per share. The aggregate offer proceeds were $15 million. In October 2014, GSK sold all their shares and no longer have an ownership position in the Company as of December 31, 2014.

In September 2013, the Company entered into a collaboration agreement with Biogen Idec ("Biogen") to discover, develop and commercialize novel small molecules that target the glucocerobrosidase ("GCase") enzyme for the treatment of Parkinson's disease. In September 2014, the Company and Biogen concluded their research collaboration. Amicus' most advanced Parkinson's candidate is AT3375, which was developed outside the collaboration and is wholly-owned by Amicus. For further information, see "— Note 15. Collaborative Agreements."

The Company had an accumulated deficit of approximately $447.4 million at December 31, 2014 and anticipates incurring losses through the fiscal year ending December 31, 2015 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 its initial public offering ("IPO") and subsequent stock offerings, payments from partners during the terms of the collaboration agreements and other financing arrangements. The Company believes that its existing cash and cash equivalents and short-term investments will be sufficient to fund the current operating plan into 2017.

2. Summary of Significant Accounting Policies

Basis of Presentation

The accompanying consolidated financial statements have been prepared in accordance with 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 subsidiaries, Amicus Therapeutics UK Limited and Callidus Biopharma, Inc. All significant intercompany transactions and balances are eliminated in consolidation. These subsidiaries are not material to the overall financial statements of the Company.

Use of Estimates

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

Cash, Money Market Funds, and Marketable Securities

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

Marketable securities consist of fixed income investments with a maturity of greater than three months and other highly liquid investments that can be readily purchased or sold using established markets. These investments are classified as available-for-sale and are reported at fair value on the

Amicus Therapeutics, Inc.

Notes To Consolidated Financial Statements — (Continued)

Company's balance sheet. Unrealized holding gains and losses are reported within comprehensive income/ (loss) in the statements of comprehensive loss. Fair value is based on available market information including quoted market prices, broker or dealer quotations or other observable inputs. See "— Note 6. Cash, Money Market Funds and Marketable Securities", for a summary of available-for-sale securities as of December 31, 2014 and 2013.

Concentration of Credit Risk

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

Property and Equipment

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

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

Revenue Recognition

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

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

The Company's current revenue recognition policies, provide that, when a collaboration arrangement contains multiple deliverables, such as license and research and development services, the

Amicus Therapeutics, Inc.

Notes To Consolidated Financial Statements — (Continued)

Company allocates revenue to each separate unit of accounting based on a selling price hierarchy. The selling price hierarchy for a deliverable is based on (i) its vendor specific objective evidence ("VSOE") if available, (ii) third party evidence ("TPE") if VSOE is not available, or (iii) best estimated selling price ("BESP") if neither VSOE nor TPE is available. The Company would establish the VSOE of selling price using the price charged for a deliverable when sold separately. The TPE of selling price would be established by evaluating largely similar and interchangeable competitor products or services in standalone sales to similarly situated customers. The BESP would be established considering internal factors such as an internal pricing analysis or an income approach using a discounted cash flow model.

The Company also considers the impact of potential future payments it makes in its role as a vendor to its customers and evaluates if these potential future payments could be a reduction of revenue from that customer. If the potential future payments to the customer are:

- a payment for an identifiable benefit; and
- the identifiable benefit is separable from the existing relationship between the Company and its customer; and
- the identifiable benefit can be obtained from a party other than the customer; and
- the Company can reasonably estimate the fair value of the identifiable benefit

then the payments are accounted for separate from the revenue received from that customer. If, however, all these criteria are not satisfied, then the payments are treated as a reduction of revenue from that customer.

If the Company determines that any potential future payments to its customers are to be considered as a reduction of revenue, it must evaluate if the total amount of revenue to be received under the arrangement is fixed and determinable. If the total amount of revenue is not fixed and determinable due to the uncertain nature of the potential future payments to the customer, then any customer payments cannot be recognized as revenue until the total arrangement consideration becomes fixed and determinable.

The reimbursements for research and development costs under collaboration agreements that meet the criteria for revenue recognition are included in Research Revenue and the costs associated with these reimbursable amounts are included in research and development expenses.

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

Fair Value Measurements

The Company records certain asset and liability balances under the fair value measurements as defined by the FASB guidance. Current FASB fair value guidance emphasizes that fair value is a market-based measurement, not an entity-specific measurement. Therefore, a fair value measurement should be determined based on the assumptions that market participants would use in pricing the asset

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Notes To Consolidated Financial Statements — (Continued)

or liability. As a basis for considering market participant assumptions in fair value measurements, current FASB guidance establishes a fair value hierarchy that distinguishes between market participant assumptions based on market data obtained from sources independent of the reporting entity (observable inputs that are classified within Levels 1 and 2 of the hierarchy) and the reporting entity's own assumptions that market participants assumptions would use in pricing assets or liabilities (unobservable inputs classified within Level 3 of the hierarchy).

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

Research and Development Costs

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

Interest Income and Interest Expense

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

Income Taxes

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

Other Comprehensive Income/ (Loss)

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

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Notes To Consolidated Financial Statements — (Continued)

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.

Nonqualified Cash Deferral Plan

In July 2014, the Board of Directors approved the Company's Cash Deferral Plan (the "Deferral Plan"), which provides certain key employees and members of the Board of Directors as selected by the Compensation Committee of the Board of Directors of the Company (the "Compensation Committee"), with an opportunity to defer the receipt of such Participant's base salary, bonus and director's fees, as applicable. The Deferral Plan is intended to be a nonqualified deferred compensation plan that complies with the provisions of Section 409A of the Internal Revenue Code (the "Code").

As of December 31, 2014, the amounts deferred under the Deferral Plan have not been invested. The investments are expected to be made in the first quarter of fiscal year 2015. All of the investments held in the Deferral Plan will be classified as trading securities and recorded at fair value with changes in the investments' fair value recognized as earnings in the period they occur. The corresponding liability for the Deferral Plan is included in other non-current liability in our consolidated balance sheets.

Equity Incentive Plan

In June 2014, our stockholders approved the Amended and Restated 2007 Equity Incentive Plan (the "Plan"). The amendment to the Plan makes an additional 6.0 million shares of our common stock available for issuance and increases the maximum number of shares within the Plan that may be issued as restricted stock, restricted stock units ("RSUs"), stock grants and any other similar awards from 1.1 million to 1.5 million shares. As of December 31, 2014, awards issued under the Plan include both stock options and RSUs.

Stock-Based Compensation

At December 31, 2014, the Company had three stock-based employee compensation plans, which are described more fully in "— Note 9. Stockholders' Equity." The Company applies the fair value method of measuring stock-based compensation, which requires a public entity to measure the cost of employee services received in exchange for an award of equity instruments based on the grant-date fair value of the award.

Loss per Common Share

The Company calculates net loss per share as a measurement of the Company's performance while giving effect to all dilutive potential common shares that were outstanding during the reporting period. The Company had a net loss for all periods presented; accordingly, the inclusion of common stock

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Notes To Consolidated Financial Statements — (Continued)

options, unvested restricted stock units ("RSUs") and warrants would be anti-dilutive. Therefore, the weighted average shares used to calculate both basic and diluted earnings per share are the same. See "— Note 19. Earnings per Share" for further discussion on net loss per share.

Dividends

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

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.

Business Combinations

The Company allocates the purchase price of acquired businesses to the tangible and intangible assets acquired and liabilities assumed based upon their estimated fair values on the acquisition date. The purchase price allocation process requires management to make significant estimates and assumptions, especially at the acquisition date with respect to intangible assets and in-process research and development ("IPR&D"). In connection with the purchase price allocations for acquisitions, the Company estimates the fair value of contingent payments utilizing a probability-based income approach inclusive of an estimated discount rate.

Contingent Consideration Payable

The Company determines the fair value of contingent acquisition consideration payable on the acquisition date using a probability-based income approach utilizing an appropriate discount rate. Contingent acquisition consideration payable is shown as a non-current liability on the Company's consolidated balance sheets. Changes in the fair value of the contingent acquisition consideration payable will be determined each period end and recorded on the consolidated statements of operations.

Intangible Assets and Goodwill

The Company records goodwill in a business combination when the total consideration exceeds the fair value of the net tangible and identifiable intangible assets acquired. Purchased IPR&D is accounted for as an indefinite lived intangible asset until the underlying project is completed, at which point the intangible asset will be accounted for as a definite lived intangible asset, or abandoned, at which point the intangible asset will be written off or partially impaired. Goodwill and indefinite lived intangible assets are assessed annually for impairment and whenever events or circumstances indicate that the carrying amount of an asset may not be recoverable. If it is determined that the full carrying amount of an asset is not recoverable, an impairment loss is recorded in the amount by which the carrying amount of the asset exceeds its fair value.

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Notes To Consolidated Financial Statements — (Continued)

Restructuring

Restructuring charges are recognized as a result of actions to streamline operations and rationalize manufacturing facilities. Judgment is used when estimating the impact of restructuring plans, including future termination benefits and other exit costs to be incurred when the actions take place. Actual results could vary from these estimates.

Recent Accounting Pronouncements

In November 2014, the FASB issued ASU 2014-17, *Business Combinations (Topic 805): Pushdown Accounting* which provides an acquired entity with the option to apply pushdown accounting in its separate financial statements upon occurrence of an event in which an acquirer obtains control of the acquired entity. The ASU is effective on November 18, 2014. After the effective date, an acquired entity can make an election to apply the guidance to future change-in-control events or to its most recent change-in-control event. However, if the financial statements for the period in which the most recent change-in-control event occurred already have been issued or made available to be issued, the application of this guidance would be a change in accounting principle. The Company is currently assessing the impact that this standard will have on its consolidated financial statements.

In August 2014, the FASB issued ASU 2014-15, Presentation of Financial Statements-Going Concern (Subtopic 205-40): Disclosure of Uncertainties about an Entity's Ability to Continue as a Going Concern, which defines management's responsibility to assess an entity's ability to continue as a going concern, and to provide related footnote disclosures if there is substantial doubt about its ability to continue as a going concern. The pronouncement is effective for annual reporting periods ending after December 15, 2016 with early adoption permitted. The adoption of this guidance is not expected to have a significant impact on the Company's consolidated financial state.

In June 2014, the FASB issued ASU 2014-10 that removes the definition of development stage entity from the accounting standards codification, thereby removing the financial reporting distinction between development stage entities and other reporting entities from U.S. GAAP. In addition, the ASU eliminates the requirements for development stage entities to (i) present inception-to-date information in the statement of income, cash flow and stockholders' equity, (ii) label the financial statements as those of a development stage entity, (iii) disclose a description of the development stage activities in which the entity is engaged, and (iv) disclose in the first year in which the entity is no longer a development stage entity that in prior years it had been in the development stage. The Company has applied the ASU to its financial statements as of June 30, 2014.

In May 2014, FASB issued ASU 2014-09, *Revenue From Contracts With Customers* , that outlines a single comprehensive model for entities to use in accounting for revenue arising from contracts with customers and supersedes most current revenue recognition guidance, including industry-specific guidance. The ASU is based on the principle that an entity should recognize revenue to depict the transfer of goods or services to customers in an amount that reflects the consideration to which the entity expects to be entitled in exchange for those goods or services. The ASU also requires additional disclosure about the nature, amount, timing and uncertainty of revenue and cash flows arising from customer contracts, including significant judgments and changes in judgments and assets recognized from costs incurred to fulfill a contract. Entities have the option of using either a full retrospective or a modified retrospective approach for the adoption of the new standard. The ASU becomes effective for the Company at the beginning of its 2017 fiscal year; early adoption is not permitted. The Company is currently assessing the impact that this standard will have on its consolidated financial statements.

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3. Acquisition of Callidus Biopharma, Inc.

In November 2013, the Company acquired Callidus, through the merger of the Company's subsidiary, CB Acquisition Corp. with and into Callidus (see "— Note 1. Description of Business"). Callidus was a privately-held biologics company focused on developing best-in-class ERTs for LSDs and its lead ERT is ATB200 for Pompe disease in late preclinical development. The acquisition of the Callidus assets and technology compliments Amicus' CHART™ platform for the development of next generation ERTs.

In consideration for the merger, the Company agreed to issue an aggregate of 7.2 million shares of its common stock, par value $0.01 per share, to the former stockholders of Callidus. As of December 31, 2014, approximately 25 thousand shares remain issuable to former Callidus stockholders. In addition, the Company will be obligated to make additional payments to the former stockholders of Callidus upon the achievement by Callidus of certain clinical milestones of up to $35 million and regulatory approval milestones of up to $105 million as set forth in the Merger Agreement, provided that the aggregate consideration shall not exceed $130 million. The Company may, at its election, satisfy certain milestone payments identified in the Merger Agreement aggregating $40 million in shares of its Common Stock (calculated based on a price per share equal to the average of the last closing bid price per share for the Common Stock on The NASDAQ Global Market for the ten (10) trading days immediately preceding the date of payment). The milestone payments not permitted to be satisfied in Common Stock (as well as any payments that the Company is permitted to, but chooses not to, satisfy in Common Stock), as a result of the terms of the Merger Agreement, the rules of The NASDAQ Global Market, or otherwise, will be paid in cash.

The fair value of the contingent acquisition consideration payments on the acquisition date was $10.6 million and was estimated by applying a probability-based income approach utilizing an appropriate discount rate. This estimation was based on significant inputs that are not observable in the market, referred to as Level 3 inputs. Key assumptions included a discount rate of 13.0% and various probability factors. As of December 31, 2014, the range of outcomes and assumptions used to develop these estimates has changed to better reflect the probability of certain milestone outcomes. (see "— Note 10. Assets and Liabilities Measured at Fair Value", for additional discussion regarding fair value measurements of the contingent acquisition consideration payable). The Company determined the fair value of the contingent consideration to be $10.7 million at December 31, 2014, resulting in an increase in the contingent consideration payable and related income of $0.1 million for the year ended December 31, 2014.

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Notes To Consolidated Financial Statements — (Continued)

The following table presents the allocation of the purchase consideration, including the contingent acquisition consideration payable, based on fair value:

	(in thousands)
Upfront equity payments	$ 15,000
Contingent acquisition consideration payable	10,600
Total consideration	25,600
Cash and cash equivalents	34
Property, plant and equipment	173
Intangible assets — IPR&D	23,000
Total identifiable assets acquired	$ 23,207
Accounts payable	(34)
Deferred tax liability	(9,186)
Total liabilities assumed	(9,220)
Net identifiable assets acquired	13,987
Goodwill	11,613
Net assets acquired	$ 25,600

A substantial portion of the assets acquired consisted of intangible assets related to Callidus lead ERT. The Company determined that the estimated acquisition-date fair values of the IPR&D related to the lead ERT was $23.0 million.

The $9.2 million of deferred tax liabilities relates to the tax impact of future amortization or possible impairments associated with the identified intangible assets acquired, which are not deductible for tax purposes. The goodwill results from the recognition of the deferred tax liability on the intangible assets as well as synergies expected from the acquisition and other benefits that do not qualify for separate recognition as acquired intangible assets. None of the goodwill is expected to be deductible for income tax purposes. The Company recorded the goodwill in the Company's consolidated balance sheet as of the acquisition date.

The Company recognized $0.5 million of acquisition-related transaction costs in selling, general and administrative expenses during 2013, which consisted primarily of legal fees and severance related to the acquisition.

The results of operations of Callidus since November 19, 2013 have been included in the Company's consolidated statements of operations and are de minimis as of December 31, 2013.

The following unaudited consolidated pro forma financial information presents the combined results of operations of the Company and Callidus as if the acquisition had occurred as of January 1, 2013. The unaudited pro forma consolidated financial information is not necessarily indicative of what the Company's consolidated results of operations actually would have been had the acquisition been completed as of January 1, 2013. In addition, the unaudited pro forma consolidated financial

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information does not attempt to project the future results of operations of the Company combined with Callidus.

Unaudited Pro Forma Consolidated Information: (in thousands)	Years Ended December 31, 2013	2012
Revenue	$ 363	$ 18,411
Net income (loss)	$ (61,804)	$ (49,807)

4. Goodwill

In connection with the acquisition of Callidus as discussed in "— Note 3. Acquisition of Callidus Biopharma, Inc.", the Company recognized goodwill of $11.6 million. Goodwill is assessed annually for impairment on October 1 and whenever events or circumstances indicate that the carrying amount of an asset may not be recoverable. If it is determined that the full carrying amount of an asset is not recoverable, an impairment loss is recorded in the amount by which the carrying amount of the asset exceeds its fair value. During the 2014 impairment assessment, it was determined that the goodwill had not been impaired thus there were no changes to the goodwill balance in 2014.

5. Intangible Assets

In connection with the acquisition of Callidus as discussed in "— Note 3. Acquisition of Callidus Biopharma, Inc.", the Company recognized IPR&D of $23.0 million. Intangible assets related to IPR&D assets are considered to be indefinite-lived until the completion or abandonment of the associated research and development efforts. During the period the assets are considered indefinite-lived, they will not be amortized but will be tested for impairment on an annual basis on October 1 and between annual tests if the Company becomes aware of any events occurring or changes in circumstances that would indicate a reduction in the fair value of the IPR&D assets below their respective carrying amounts. During the 2014 impairment assessment, it was determined that the IPR&D had not been impaired thus there was no change in the IPR&D balance in 2014.

6. Cash, Money Market Funds and Marketable Securities

As of December 31, 2014, the Company held $24.1 million in cash and cash equivalents and $145.1 million of available-for-sale securities which are reported at fair value on the Company's balance sheet. Unrealized holding gains and losses are reported within accumulated other comprehensive income/ (loss) in the statements of comprehensive loss. If a decline in the fair value of a marketable security below the Company's cost basis is determined to be other than temporary, such marketable security is written down to its estimated fair value as a new cost basis and the amount of the write-down is included in earnings as an impairment charge. To date, only temporary impairment adjustments have been recorded.

Consistent with the Company's investment policy, the Company does not use derivative financial instruments in its investment portfolio. The Company regularly invests excess operating cash in deposits with major financial institutions, money market funds, notes issued by the U.S. government, as well as fixed income investments and U.S. bond funds both of which can be readily purchased and sold using established markets. The Company believes that the market risk arising from its holdings of these financial instruments is mitigated as many of these securities are either government backed or of the highest credit rating. Investments that have original maturities of greater than 3 months but less than

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1 year are classified as short-term and investments with maturities that are greater than 1 year are classified as long-term.

Cash and available for sale securities consisted of the following as of December 31, 2014 and December 31, 2013 (in thousands):

	As of December 31, 2014			
	Cost	Unrealized Gain	Unrealized Loss	Fair Value
Cash balances	$ 24,074	$ —	$ —	$ 24,074
Corporate debt securities, current portion	115,862	—	(110)	115,752
Corporate debt securities, non-current portion	17,508	—	(44)	17,464
Commercial paper	11,477	22	—	11,499
Certificate of deposit	350	—	—	350
	$ 169,271	$ 22	$ (154)	$ 169,139
Included in cash and cash equivalents	$ 24,074	$ —	$ —	$ 24,074
Included in marketable securities	145,197	22	(154)	145,065
Total cash and marketable securities	$ 169,271	$ 22	$ (154)	$ 169,139

	As of December 31, 2013			
	Cost	Unrealized Gain	Unrealized Loss	Fair Value
Cash balances	$ 43,640	$ —	$ —	$ 43,640
Corporate debt securities	30,817	1	(6)	30,812
Commercial paper	7,192	6	—	7,198
Certificate of deposit	350	—	—	350
	$ 81,999	$ 7	$ (6)	$ 82,000
Included in cash and cash equivalents	$ 43,640	$ —	$ —	$ 43,640
Included in marketable securities	38,359	7	(6)	38,360
Total cash and marketable securities	$ 81,999	$ 7	$ (6)	$ 82,000

Unrealized gains and losses are reported as a component of other comprehensive gain/ (loss) in the statements of comprehensive loss. For the year ended December 31, 2014 and 2013, unrealized holding loss of $132 thousand and $13 thousand respectively, were included in the statements of comprehensive loss.

For the years ended December 31, 2014 and 2013, there were no realized gains or losses. The cost of securities sold is based on the specific identification method.

Unrealized loss positions in the available for sale securities as of December 31, 2014 and December 31, 2013 reflect temporary impairments that have been in a loss position for less than twelve months and as such are recognized in other comprehensive gain/ (loss). The fair value of these available for sale securities in unrealized loss positions was $129.2 million and $23.6 million as of December 31, 2014 and 2013, respectively.

The Company holds available-for-sale investment securities which are reported at fair value on the Company's balance sheet. Unrealized holding gains and losses are reported within accumulated other

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Notes To Consolidated Financial Statements — (Continued)

comprehensive income ("AOCI") in the statements of comprehensive loss. The changes in AOCI associated with the unrealized holding gain on available-for-sale investments during the years ended December 31, 2014 and 2013, were as follows (in thousands):

	Year Ended December 31,		
	2014	2013	2012
Balance, beginning	$ 1	$ 14	$ 4
Current period changes in fair value,	(133)	(13)	10
Reclassification of earnings,	—	—	—
Balance, ending	$ (132)	$ 1	$ 14

7. Property and Equipment

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

	December 31,	
	2014	2013
Property and equipment consist of the following:		
Computer equipment	$ 3,555	$ 3,537
Computer software	1,102	1,064
Research equipment	5,986	5,918
Furniture and fixtures	1,547	1,527
Leasehold improvements	2,141	2,047
	14,331	14,093
Less accumulated depreciation and amortization	(11,520)	(9,973)
	$ 2,811	$ 4,120

Depreciation and amortization expense was $1.5 million and $1.7 million each for the years ended December 31, 2014 and 2013, respectively. There were no capital lease obligations outstanding as of December 31, 2014.

8. Accounts Payable and Accrued Expenses

Accounts payable and accrued expenses consist of the following (in thousands):

	December 31,	
	2014	2013
Accounts payable	$ 5,874	$ 2,146
Accrued professional fees	473	498
Accrued contract manufacturing & contract research costs	3,321	1,499
Accrued compensation and benefits	5,051	4,781
Accrued facility costs	557	963
Contingent success fee payable	341	—
Accrued other	728	275
	$ 16,345	$ 10,162

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Notes To Consolidated Financial Statements — (Continued)

Other long-term liabilities consist of the following (in thousands):

	December 31,	
	2014	2013
Exit fees	$ 450	$ 450
Success fees	—	264
Employee compensation and benefits	124	—
Security deposits	14	—
	$ 588	$ 714

9. Stockholders' Equity

Common Stock and Warrants

As of December 31, 2014, the Company was authorized to issue 125 million 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 that are appropriate for stockholder voting and is entitled to one vote for each share held.

In November 2014, we sold a total of 15.9 million shares of our common stock, par value $0.01 per share, at a public offering price of $6.50 per share. The offering generated gross proceeds of $103.5 million. J.P. Morgan Securities LLC acted as sole book-running manager for the offering. Cowen and Company, LLC, Leerink Partners and Janney Montgomery Scott LLC acted as lead managers for the offering. After deducting the underwriting fee of $6.2 million and other offering expenses of $0.1 million, which included legal fees, the net proceeds of the offering were approximately $97.2 million. We expect to use the net proceeds of the offering for investment in the global commercialization infrastructure for migalastat monotherapy for Fabry disease, the continued clinical development of its product candidates and for other general corporate purposes.

In March 2014, the Company entered into the Sales Agreement with Cowen to create an ATM equity program under which the Company sold shares of its common stock, par value $0.01 per share, with an aggregate offering price of up to $40 million ("ATM Shares") through Cowen. Upon delivery of a placement notice and subject to the terms and conditions of the ATM agreement, Cowen used its commercially reasonable efforts to sell the ATM Shares, based upon the Company's instructions. The Company had provided Cowen with customary indemnification rights, and Cowen was entitled to a commission at a fixed commission rate of up to 3.0% of the gross proceeds per Share sold. Sales of the ATM Shares under the Sales Agreement were to be made in transactions that were deemed to be "at the market offerings" as defined in Rule 415 under the Securities Act of 1933, as amended, including sales made by means of ordinary brokers' transactions, including on The NASDAQ Global Market, at market prices or as otherwise agreed with Cowen. The Company began the sale of ATM Shares in May 2014. The Company sold 14.3 million shares of common stock resulting in net proceeds of $38.6 million, which included Cowen's commission of $1.1 million and other fees of $0.3 million. The Company completed all sales under the ATM equity program in July 2014.

In November 2013, the Company entered into the 2013 SPA with GSK and certain entities controlled by Redmile Group, LLC for the private placement of a combination of shares of common stock (the "Shares") and warrants (the "Warrants") to purchase shares of the common stock (collectively, the "Units"). Each of the investors was one of the Company's stockholders prior to consummation of these transactions. The Shares and the Units sold to the investors were offered and

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sold in reliance on exemptions from registration pursuant to Rule 506 of Regulation D promulgated under the Securities Act based on the nature of such investors and certain representations made to the Company. Pursuant to the 2013 SPA, Amicus agreed to issue 1.5 million Shares at $2.00 per Share to GSK and 6 million Units at $2.00 per Unit to Redmile Group, with each Unit consisting of one Share and .267 Warrants resulting in an aggregate of 6 million Shares and 1.6 million Warrants underlying the Units to be issued. Each Warrant is exercisable between July 1, 2014 and June 30, 2015 with an exercise price of $2.50, subject to certain adjustments. The Company received total proceeds of $15 million for general corporate and working capital purposes as a result of the private placement and the transaction closed in November 2013. In October 2014, GSK sold all their shares and no longer have an ownership position in the Company as of December 31, 2014.

The Company evaluated the warrants against current accounting guidance and determined that these warrants should be accounted as a component of equity. As such, these warrants are valued at issuance date using the Black Scholes valuation model using inputs such as the underlying price of the shares issued when the warrant is exercised, volatility, risk free interest rate and expected life of the instrument. The six inputs used to determine the value of the warrants were: (1) the closing price of Amicus stock on the day of evaluation of $2.12; (2) the exercise price of the warrants of $2.50; (3) the remaining term of the warrants of 1 year; (4) the volatility of Amicus' stock for the one year term of 93.5%; (5) the annual rate of dividends of 0%; and (6) the riskless rate of return of 0.12%. The annual rate of dividends is based on the Company's historical practice of not granting dividends. The resulting Black Scholes value of the warrants was $1.0 million.

In November 2013, in connection with its acquisition of Callidus, the Company agreed to issue an aggregate of 7.2 million shares of its common stock, par value $0.01 per share, to the former stockholders of Callidus. As of December 31, 2014, approximately 25 thousand shares remain issuable to former Callidus stockholders.

Nonqualified Cash Deferral Plan

In July 2014, the Board of Directors approved the Company's Deferral Plan, which provides certain key employees and members of the Board of Directors as selected by the Compensation Committee, with an opportunity to defer the receipt of such Participant's base salary, bonus and director's fees, as applicable. The Deferral Plan is intended to be a nonqualified deferred compensation plan that complies with the provisions of Section 409A of the Code.

As of December 31, 2014, the amounts deferred under the Deferral Plan have not been invested. The investments are expected to be made in the first quarter of fiscal year 2015. Deferred compensation as of December 31, 2014 totaled approximately $0.1 million and is included in other long-term liabilities within the accompanying consolidated balance sheets. All of the investments held in the Deferral Plan will be classified as trading securities and recorded at fair value with changes in the investments' fair value recognized in the period they occur. The corresponding liability for the Deferral Plan is included in other non-current liability in the Company's consolidated balance sheets.

Stock Option Plans

In June 2014, the Company's stockholders approved the Amended and Restated 2007 Equity Incentive Plan (the "Plan"). The amendment to the Plan makes an additional 6 million shares of the Company's common stock available for issuance and increases the maximum number of shares within the Plan that may be issued as restricted stock, RSUs, stock grants and any other similar awards from

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Notes To Consolidated Financial Statements — (Continued)

1.1 million to 1.5 million shares. As of December 31, 2014, awards issued under the Plan include both stock options and RSUs.

In May 2007, the Company's Board of Directors and stockholders approved the Company's 2007 Director Option Plan (the "2007 Director Plan"). The Plan provides 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 Plan is 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 2007 Director Plan is intended to promote the recruiting and retention of highly qualified eligible directors and strengthen the commonality of interest between directors and stockholders by encouraging ownership of common stock of the Company. 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 Plan generally vest 25% on the first year anniversary date of grant plus an additional 1/48th for each month thereafter and may be exercised in whole or in part for 100% of the shares vested at any time after the date of grant. Options under the 2007 Director Plan may be granted to new directors upon joining the Board and vest in the same manner as options under the Plan. In addition, options are automatically granted to all directors at each annual meeting of stockholders and vest on the date of the annual meeting of stockholders of the Company in the year following the year during which the options were granted.

As of December 31, 2014, the Company has reserved up to 6,595,880 shares for issuance under the Plan and the 2007 Director Plan.

Stock Option Grants

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

The Company uses the Black-Scholes option pricing model when estimating the fair value for stock-based awards. Use of a valuation model requires management to make certain assumptions with respect to selected model inputs. Expected volatility was calculated based on a blended weighted average of historical information of the Company's stock and the weighted average of historical information of similar public entities for which historical information was available. The Company will continue to use a blended weighted average approach using its own historical volatility and other similar public entity volatility information until the Company's historical volatility is relevant to measure expected volatility for future option grants. The average expected life was determined using the "simplified" method of estimating the expected exercise term which is the mid-point between the vesting date and the end of the contractual term. As the Company's stock price volatility has been over 75% and it has experienced significant business transactions, the Company does not have sufficient reliable exercise data in order to justify a change in the use of the "simplified" method of estimating the expected exercise term of employee stock option grants. 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.

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

	Years Ended December 31,		
	2014	2013	2012
Expected stock price volatility	81.3%	82.0%	77.2%
Risk free interest rate	1.9%	1.3%	0.8%
Expected life of options (years)	6.25	6.25	6.25
Expected annual dividend per share	$ 0.00	$ 0.00	$ 0.00

The weighted average grant-date fair value per share of options granted during 2014, 2013 and 2012 were $2.12, $2.14 and $3.31, respectively.

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, 2011	6,653.5	$ 6.87		
Granted	2,846.6	$ 5.34		
Exercised	(437.0)	$ 3.73		
Forfeited	(1,088.9)	$ 7.95		
Options outstanding, December 31, 2012	7,974.2	$ 6.35		
Granted	2,481.8	$ 3.04		
Exercised	—	—		
Forfeited	(1,414.9)	$ 5.01		
Options outstanding, December 31, 2013	9,041.1	$ 5.65		
Granted	2,993.1	$ 2.99		
Exercised	(965.6)	$ 3.80		
Forfeited	(1,047.9)	$ 5.76		
Options outstanding, December 31, 2014	10,020.7	$ 5.02	6.8 years	$ 36.9
Vested and unvested expected to vest, December 31, 2014	9,441.0	$ 5.13	6.7 years	$ 34.0
Exercisable at December 31, 2014	5,447.9	$ 6.45	5.2 years	$ 14.0

The aggregate intrinsic value of options exercised during the years ended December 31, 2014 and 2012 was $2.8 million and $0.9 million, respectively. There were no options exercised during the year ended December 31, 2013. As of December 31, 2014, the total unrecognized compensation cost related to non-vested stock options granted was $7.7 million and is expected to be recognized over a weighted average period of 2.3 years. Cash proceeds from stock options exercised during the years ended December 31, 2014 and 2012 were $3.7 million and $1.6 million respectively.

Restricted Stock Units

In April 2014, the Compensation Committee made awards of RSUs to certain employees of the Company. The RSUs awarded under the Plan are generally subject to graded vesting of 50% of the RSUs on the 13 th month anniversary of the grant date and the remaining 50% of the RSUs on the

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20 th month anniversary of the grant date, in each case, contingent on such employee's continued service on such date.

RSUs are generally subject to forfeiture if employment terminates prior to the release of vesting restrictions. The Company expenses the cost of the RSUs, which is determined to be the fair market value of the shares of common stock underlying the RSUs at the date of grant, ratably over the period during which the vesting restrictions lapse.

A summary of non-vested RSU activity under the Plan for the year ended December 31, 2014 is as follows:

	Number of Shares (in thousands)	Weighted Average Grant Date Fair Value	Weighted Average Remaining Years	Aggregate Intrinsic Value (in millions)
Non-vested units as of December 31, 2013	—	$ —		
Granted	975	$ 2.28		
Vested	—	$ —		
Forfeited	(20)	$ 2.15		
Non-vested units as of December 31,2014	955	$ 2.28	0.74	$ 5.8

For the year ended December 31, 2014, there were no RSUs that vested and all non-vested units are expected to vest over their normal term. As of December 31, 2014, there was $1.1 million of total unrecognized compensation cost related to unvested RSUs with service-based vesting conditions. These costs are expected to be recognized over a weighted average period of 0.74 years.

In April 2014, the Board of Directors approved the Company's Restricted Stock Unit Deferral Plan ("the Deferred Compensation Plan"), which provides selected employees with an opportunity to defer receipt of RSUs until the first to occur of termination of the employee's employment or a date selected by the employee. Any RSUs deferred under the Deferred Compensation Plan would be fully vested once the original vesting conditions of the RSU were satisfied.

Compensation Expense Related to Equity Awards

The following table summarizes the stock-based compensation expense recognized in the statements of operations (in thousands):

	Years Ended December 31,		
	2014	2013	2012
Stock compensation expense recognized in:			
Research and development expense	$ 2,703	$ 3,583	$ 3,603
General and administrative expense	3,305	2,594	2,588
Total stock compensation expense	$ 6,008	$ 6,177	$ 6,191

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10. Assets and Liabilities Measured at Fair Value

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

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

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

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

Cash, Money Market Funds and Marketable Securities

The Company classifies its cash and money market funds within the fair value hierarchy as Level 1 as these assets are valued using quoted prices in active market for identical assets at the measurement date. The Company considers its investments in marketable securities as available for sale and classifies these assets within the fair value hierarchy as Level 2 primarily utilizing broker quotes in a non-active market for valuation of these securities. No changes in valuation techniques or inputs occurred during the year ended December 31, 2014. No transfers of assets between Level 1 and Level 2 of the fair value measurement hierarchy occurred during the year ended December 31, 2014.

Secured Debt

As disclosed in "— Note 16. Short Term Borrowings and Long Term Debt", the Company has a new loan and security agreement with MidCap Financial, Oxford Finance and Silicon Valley Bank, in addition to an earlier existing loan with Silicon Valley Bank. The carrying amount of the Company's borrowings approximates fair value at December 31, 2014. The Company's secured debt is classified as Level 2 and the fair value is estimated using quoted prices for similar liabilities in active markets, as well as inputs that are observable for the liability (other than quoted prices), such as interest rates that are observable at commonly quoted intervals.

In connection with the Term Loan, as disclosed in "— Note 16. Short Term Borrowings and Long Term Debt", the Company recorded a contingent liability of approximately $0.3 million representing the fair value of a contingent payment of up to $0.4 million related to a success fee payable within six months of trigger event, with the trigger event being regulatory acceptance of NDA or MAA submission. This is effective 5 years from the closing of the Term Loan. The success fee payable to the lender was probability adjusted and discounted utilizing an appropriate discount rate and hence classified as Level 3.

Contingent Consideration Payable

The contingent consideration payable resulted from acquisition of Callidus, as discussed in "— Note 3. Acquisition of Callidus Biopharma, Inc." Our most recent valuation was determined using a probability-based income approach utilizing a discounted rate of 11.5%, which is a measure of the credit risk associated with settling the liability. For 2014 compared to 2013, the net increase in the fair value of this obligation was primarily due to the elapse of time of when the contingent consideration could be paid. Some of the more significant assumptions used in the valuation include (i) the probability and timing related to the achievement of certain developmental milestones and (ii) and the discount rate. The contingent consideration is revalued each reporting period. There is no assurance that any of the conditions for the milestone payments will be met.

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Notes To Consolidated Financial Statements — (Continued)

The contingent consideration payable has been classified as a Level 3 liability as its valuation requires substantial judgment and estimation of factors that are not currently observable in the market. If different assumptions were used for the various inputs to the valuation approach the estimated fair value could be significantly higher or lower than the fair value the Company determined. The Company may be required to record losses in future periods.

A summary of the fair value of the Company's assets and liabilities aggregated by the level in the fair value hierarchy within which those measurements fall as of December 31, 2014 are identified in the following table (in thousands):

	Level 1	Level 2	Total
Assets:			
Cash/money market funds	$ 24,074	$ —	$ 24,074
Commercial paper	—	11,499	11,499
Corporate debt securities	—	133,216	133,216
Certificate of deposit	—	350	350
	$ 24,074	$ 145,065	$ 169,139

	Level 1	Level 2	Level 3	Total
Liabilities:				
Secured debt	$ —	$ 14,350	$ —	$ 14,350
Contingent success fee payable	—	—	341	341
Contingent consideration payable	—	—	10,700	10,700
	$ —	$ 14,350	$ 11,041	$ 25,391

A summary of the fair value of the Company's assets and liabilities aggregated by the level in the fair value hierarchy within which those measurements fall as of December 31, 2013 are identified in the following table (in thousands):

	Level 1	Level 2	Total
Assets:			
Cash/money market funds	$ 43,640	$ —	$ 43,640
Commercial paper	—	7,198	7,198
Corporate debt securities	—	30,812	30,812
Certificate of deposit	—	350	350
	$ 43,640	$ 38,360	$ 82,000

	Level 1	Level 2	Level 3	Total
Liabilities:				
Secured debt	$ —	$ 14,473	$ —	$ 14,473
Contingent success fee payable	—	—	264	264
Contingent consideration payable	—	—	10,600	10,600
	$ —	$ 14,473	10,864	25,337

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The change in the fair value of Level 3 liabilities at December 31, 2014 was an increase of $0.2 million due to the increase in fair value of contingent consideration payable of $0.1 million and contingent success fee payable of $77 thousand. The change in the fair value of Level 3 liabilities at December 31, 2013 was an increase of $10.0 million due to the addition of contingent consideration payable of $10.6 million and contingent success fee payable of $0.3 million, offset by the decrease in the fair value of the warrant liability of $0.9 million.

11. 401(k) Plan

The Company has a 401(k) plan (the "401(k) Plan") covering all eligible employees and provides for a company match of up to 5% of salary and bonus paid during the year. In 2013, the Company changed the vesting policy whereby the match vests immediately upon enrollment. The Company's total contribution to the 401(k) Plan was $0.6 million, $0.7 million and $0.7 million for the years ended December 31, 2014, 2013 and 2012, respectively.

12. Leases

Operating Leases

The Company leases approximately 73,646 square feet of laboratory and office space in Cranbury, New Jersey. The lease will expire in March 2019 and may be extended by the Company for two additional five-year periods. The Company also has a lease for its laboratory and office space in San Diego, CA, which will expire in September 2016. The San Diego, CA, location was closed as of December 31, 2013, however lease payments will continue to be made until end of lease term. See "— Note 17. Restructuring Charges" for more information. Rent expenses for the Company's facilities are recognized over the term of the lease. The Company recognizes rent starting when possession of the facility is taken from the landlord. When a lease contains a predetermined fixed escalation of the minimum rent, the Company recognizes the related rent expense on a straight-line basis and records the difference between the recognized rental expense and the amounts payable under the lease as deferred rent liability. Tenant leasehold improvement allowances are reflected in accrued expenses on the consolidated balance sheets and are amortized as a reduction to rent expense in the statement of operations over the term of the lease.

At December 31, 2014, aggregate annual future minimum lease payments, net of income from subleases, under these leases are as follows:

(in thousands)	2015	2016	2017	2018	2019 and beyond	Total
Minimum lease payments	$ 2,033	$ 2,039	$ 1,769	$ 1,778	$ 297	$ 7,916
Less: income from sublease	(210)	(180)	—	—	—	(390)
Net minimum lease payments	$ 1,823	$ 1,859	$ 1,769	$ 1,778	$ 297	$ 7,526

Rent expense for the years ended December 31, 2014, 2013 and 2012 were $2.4 million, $2.6 million and $2.6 million respectively.

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Notes To Consolidated Financial Statements — (Continued)

13. Income Taxes

In June 2006, the FASB issued a single model to address accounting for uncertainty in tax positions. The model clarifies the accounting for income taxes, by prescribing a minimum recognition threshold a tax position is required to meet before being recognized in the financial statements. It also provides guidance on de-recognition, measurement, and classification of amounts relating to uncertain tax positions, accounting for and disclosure of interest and penalties, accounting in interim periods and disclosures required. The Company adopted the FASB requirements as of January 1, 2007 and determined that it did not have a material impact on the Company's financial position and results of operations. The Company did not recognize interest or penalties related to income tax during the period ended December 31, 2014 and did not accrue for interest or penalties as of December 31, 2014. The Company does not have an accrual for uncertain tax positions as of December 31, 2014. Tax returns for all years 2008 and thereafter are subject to future examination by tax authorities.

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,	
	2014	2013
Current deferred tax asset		
Non-cash stock issue	$ 8,990	$ 8,172
Others	2,023	1,343
	$ 11,013	9,515
Non-current deferred tax assets		
Amortization/depreciation	2,910	3,068
Research tax credit	14,288	13,680
Net operating loss carry forwards	105,274	79,984
Deferred revenue	14,626	14,649
Others	324	682
Gross deferred tax assets	148,435	121,578
Deferred tax liability related to business acquisition	(9,186)	(9,186)
Total net deferred tax asset	139,249	112,392
Less valuation allowance	(148,435)	(121,578)
Net deferred tax assets (liability)	$ (9,186)	$ (9,186)

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, 2013, and 2014, the Company recorded valuation allowances of $121.6 million and $148.4 million, respectively, representing an increase in the valuation allowance of $26.3 million in 2013 and an increase of $26.8 million in 2014, 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, 2014, the Company had federal and state net operating loss carry forwards ("NOLs") of approximately $268.5 million and $235.5 million, respectively. The federal carry forward

will expire in 2028 through 2034. Most of the state carry forwards generated prior to 2009 began to expire in 2012 and will continue to expire through 2015. The remaining state carry forwards including those generated in 2009 through 2012 will expire in 2029 through 2034 due to a change in the New Jersey state law regarding the net operating loss carry forward period. Utilization of NOLs may be subject to a substantial limitation pursuant to Section 382 of the Code as well as similar state statutes in the event of an ownership change. Such ownership changes have occurred in the past, and could occur again in the future As a result of these ownership changes, Section 382 places an annual limitation on the amount of NOLs that can be utilized to offset future taxable income each year, which is based on the value of the company at the change date. This limitation could result in expiration of those carry forwards before utilization. In general, an ownership change, as defined by Section 382, results from transactions that increase the ownership of certain stockholders or public groups in the stock of a corporation by more than 50 percentage points over a three year period. The Company completed a detailed study of its cumulative ownership changes for 2014 and determined that there was an ownership change in excess of 50% in October of 2014. Based on the value of the Company at the change date, the limitation amount is estimated to be sufficient to permit the utilization of up to approximately $397 million over the next 20 years, which exceeds the total of the Company's NOLs through December 31, 2014. Therefore, there was no need to write down the gross amount of the deferred tax asset related to the Company's federal NOLs. A tax benefit of $0.2 million associated with the exercise of stock options will be recorded in additional paid-in capital when the associated net operating loss is recognized.

A reconciliation of the statutory tax rates and the effective tax rates for the years ended December 31, 2012, 2013 and 2014 are as follows:

	Years Ended December 31,		
	2014	2013	2012
Statutory rate	(34)%	(34)%	(34)%
State taxes, net of federal benefit	(4)	(5)	(3)
Permanent adjustments	1	(1)	(1)
R&D credit	(4)	(3)	(8)
Other	1	—	1
Valuation allowance	38	37	38
Net	(2)%	(6)%	(6)%

The Company recognized a tax benefit of $3.2 million, $3.5 million and $1.1 million in connection with the sale of net operating losses and research and development credits in the New Jersey Transfer Program for the years ended December 31, 2012, 2013 and 2014, respectively.

14. 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:

GSK — For discussion of the royalties and milestone payments potentially due to GSK, see "— Note 15. Collaborative Agreements."

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Mt. Sinai School of Medicine of New York University ("MSSM") — The Company acquired exclusive worldwide patent rights to develop and commercialize migalastat and other pharmacological chaperones for the prevention or treatment of human diseases or clinical conditions by increasing the activity of wild-type and mutant enzymes pursuant to a license agreement with MSSM. This agreement expires upon expiration of the last of the licensed patent rights, which will be in 2019, subject to any patent term extension that may be granted, or 2024 if the Company develops a product for combination therapy (pharmacological chaperone plus ERT) and a patent issues from the pending application covering combination therapy, subject to any patent term extension that may be granted. Under this agreement, to date the Company has paid no upfront or annual license fees and has no milestone or future payments other than royalties on net sales. In 2008, the Company amended and restated its license agreement with MSSM which consolidated previous amendments into a single agreement, clarified the portion of royalties and milestone payments the Company received from collaboration agreements that were payable to MSSM, and provided the Company with the sole right to control the prosecution of patent rights described in the amended and restated license agreement. Payments to MSSM are classified as research and development expenses in the Company's financial statements.

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 migalastat, the Company will owe royalties only to MSSM and will owe no milestone payments.

The Company's rights with respect to these agreements to develop and commercialize migalastat 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.

15. Collaborative Agreements

GSK

In November 2013, Amicus entered into the Revised Agreement with GSK, pursuant to which Amicus has obtained global rights to develop and commercialize migalastat as a monotherapy and in combination with ERT for Fabry disease. The Revised Agreement amends and replaces in its entirety the Expanded Agreement entered into between Amicus and GSK in July 2012. Under the terms of the Revised Agreement, there was no upfront payment from Amicus to GSK. For migalastat monotherapy, GSK is eligible to receive post-approval and sales-based milestones up to $40 million, as well as tiered royalties in the mid-teens in eight major markets outside the U.S.

Under the terms of the Revised Agreement, GSK will no longer jointly fund development costs for all formulations of migalastat.

In evaluating the impact of both the Expanded Collaboration Agreement and the Revised Agreement, the Company applied the accounting guidance regarding the impact of potential future payments it may make in its role as a vendor (i.e., Amicus) to its customer (i.e., GSK) and evaluated if these potential future payments could be a reduction of revenue from GSK. If the potential future payments to GSK are as follows:

- a payment for an identifiable benefit, and
- the identifiable benefit is separable from the existing relationship between the Company and GSK, and

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- the identifiable benefit can be obtained from a party other than GSK, and
- the Company can reasonably estimate the fair value of the identifiable benefit,

then the potential future payments would be treated separately from the collaboration and research revenue. However, if all these criteria are not satisfied, then the potential future payments are treated as a reduction of revenue.

Accordingly, the Company did not believe that, for accounting purposes, the new U.S. licensing rights to migalastat obtained from GSK under the Expanded Collaboration Agreement, nor the ex U.S. licensing rights to migalastat obtained from GSK under the Revised Agreement, represented a separate, identifiable benefit from the licenses in the Original Collaboration Agreement entered into between Amicus and GSK in 2010. The contingent amounts payable to GSK were not sufficiently separable from GSK's original license and the research and development reimbursements such that Amicus could not have entered into a similar exchange transaction with another party. Additionally, the Company cannot reasonably estimate the fair value of the worldwide licensing rights to migalastat.

The Company determined that the potential future payments to GSK would be treated as a reduction of revenue and that the total amount of revenue to be received under the arrangement is no longer fixed or determinable as the contingent milestone payments are subject to significant uncertainty.

As a result, the Company no longer recognized any of the upfront license fees and premiums on the equity purchase from GSK until such time as the arrangement consideration becomes fixed or determinable, because an indeterminable amount may ultimately be payable back to GSK. These amounts (the balance of the unrecognized upfront license fee and the premium on the equity purchases) are classified as deferred reimbursements on the balance sheet.

The recognition of Research Revenue was also affected by the determination that the overall total arrangement consideration was no longer fixed and determinable, despite the fact that the research activities continued and that the research expense reimbursements by GSK to Amicus were received as the research activities related to the reimbursement had been completed. Therefore the research reimbursements from GSK were recorded as deferred reimbursements on the balance sheet and would not recognized until the total arrangement consideration becomes fixed and determinable.

As a result, all revenue recognition was suspended until the total arrangement consideration would become fixed and determinable. In addition, future milestone payments made by the Company will be applied against the balance of this deferred reimbursements account. In the third quarter of 2013, the Company paid GSK a pass-through milestone payment of $0.8 million in connection with the development of the Co-formulated product. This payment was reflected as a reduction of the deferred reimbursements in the Consolidated Balance Sheet as of December 31, 2013.

Revenue recognition for research expense reimbursements, the original upfront license fee, and the equity premiums will resume once the total arrangement consideration becomes fixed and determinable which will occur when the balance of the deferred reimbursements account is sufficient to cover all the remaining contingent milestone payments.

Biogen

In September 2013, the Company entered into a license and collaboration agreement (the "Biogen Agreement") with Biogen to discover, develop and commercialize novel small molecules for the treatment of Parkinson's disease. Under terms of the multi-year agreement, the Company and Biogen

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Notes To Consolidated Financial Statements — (Continued)

will collaborate in the discovery of a new class of small molecules that target the GCase enzyme, for further development and commercialization by Biogen. Biogen was responsible for funding all discovery, development, and commercialization activities. In addition the Company was reimbursed for all full-time employees working on the project as part of a cost sharing arrangement. The Company was also eligible to receive development and regulatory milestones, as well as modest royalties in global net sales.

In accordance with the revenue recognition guidance related to reimbursement of research and development expenses, the Company identified all deliverables at the inception of the agreement. As the Company has not commenced its planned principal operations (i.e. selling commercial products) the Company is only performing development of its compounds, and therefore, development activities are part of the Company's ongoing central operations. Additionally, the Company has the following accounting policies:

- Research and development expenses related to a collaboration agreement will be recorded on a gross basis in the income statement and not presented net of any reimbursement received from a collaboration agreement; and

- The reimbursement of research and development expenses from a collaborator will be recognized in the income statement as "Research Revenue" for the period in which the research activity occurred.

As of December 31, 2014, the Company recognized $1.2 million in Research Revenue for work performed under the cost sharing arrangement of the Biogen Agreement.

The Company evaluated the contingent milestones included in the Biogen Agreement at the inception of the Biogen Agreement and determined that the contingent milestones are substantive milestones and would be recognized as revenue in the period that the milestone was achieved. The Company determined that the research based milestones are commensurate with the enhanced value of each delivered item as a result of the Company's specific performance to achieve the milestones. The research based milestones would relate to past performances when achieved and are reasonable relative to the other payment terms within the Biogen Agreement, including the cost sharing arrangement.

In September 2014, the Company and Biogen concluded their research collaboration. The Company's most advanced Parkinson's candidate is AT3375, which was developed outside the collaboration and is wholly-owned by the Company.

16. Short-Term Borrowings and Long-Term Debt

In December 2013, the Company entered into a credit and security agreement with a lending syndicate consisting of MidCap Funding III, LLC, Oxford Finance LLC, and Silicon Valley Bank ("SVB") which provides an aggregate of $25 million (the "Term Loan"). The Company drew $15 million of the aggregate principal amount of the Term Loan at the end of December 2013 (the "First Tranche") and did not draw the additional $10 million that was available through the end of the fourth quarter of 2014. The principal outstanding balance of the First Tranche bears interest at a rate per annum fixed at 8.5%. The Company made interest-only payments on the Term Loan beginning January 1, 2014 and will continue through April 1, 2015, after which the Company will repay the aggregate principal outstanding balance of the Term Loan in 33 equal monthly installments of principal, plus accrued interest at the applicable rate. The Term Loan matures on December 27, 2017. At December 31, 2014, the total principal amount due under the Term Loan was $15 million.

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In connection with the Term Loan, the Company recorded a debt discount of $0.8 million at December 31, 2013 which consists of payments to be made and a contingent payable to the lenders. These payments include a debt facility fee of $0.1 million which was paid on the date of the First Tranche, $0.4 million exit fee that will be payable upon repayment of the term loan and $0.3 million representing the fair value of a contingent payment of up to $0.4 million related to a success fee payable within six months of trigger event, with the trigger event being regulatory acceptance of NDA or MAA submission. This is effective 5 years from the closing of the Term Loan. The success fee payable to the lender was probability adjusted and discounted utilizing an appropriate discount rate and is shown as a current liability on the Company's consolidated balance sheet.

In February 2012, the Company borrowed approximately $1.0 million from a loan and security agreement (the "2011 Loan Agreement") with SVB which was to be repaid over the following 2.5 years. The 2011 Loan Agreement contains financial covenants and the Company has at all times been in compliance with these covenants. As of December 31, 2014, the 2011 Loan Agreement has been fully repaid and there is no amount currently due.

The carrying amount of the Company's borrowings approximates fair value at December 31, 2014.

The remaining future minimum payments of principal due as of December 31, 2014 are as follows (in thousands):

Years ending December 31:	
2015	$ 4,035
2016	5,444
2017	5,521
2018	—
2019 and beyond	—
Total principal obligation	15,000
Less short-term portion, net of short term debt discount of $195	(3,840)
Long-term portion	11,160
Less debt discount	(650)
Long term portion, net of debt discount	$ 10,510

17. Restructuring Charges

In November 2013, the Company announced a work-force reduction of approximately 14 percent, or 15 employees, as a part of a corporate restructuring. This measure was intended to reduce costs and to align the Company's resources with its key strategic priorities.

In December 2013, the Company initiated and completed a facilities consolidation effort, closing one of its leased locations in San Diego, CA. The Company recorded a total charge of $2.0 million during the fourth quarter of 2013 which included $1.2 million for employment termination costs payable and a facilities consolidation charge of $0.8 million consisting of lease payments of $0.7 million related to the net present value of the net future minimum lease payments at the cease-use date and the write-down of the net book value of the fixed assets in the vacated building of $0.1 million. At December 31, 2014, all of the restructuring charges related to employment termination costs were paid.

Amicus Therapeutics, Inc.

Notes To Consolidated Financial Statements — (Continued)

The following table summarizes the restructuring charges and utilization for the year ended December 31, 2014 (in thousands):

	Balance as of December 31, 2013	Charges	Cash Payments	Adjustments	Balance as of December 31, 2014
Employment termination costs	$ 1,139	$ —	$ (1,139)	$ —	$ —
Facilities consolidation	703	—	(357)	(63)	283
Total	$ 1,842	$ —	$ (1,496)	$ (63)	$ 283

The lease charges will be paid over the remaining lease term which expires in September 2016.

18. Subsequent Events

The Company evaluated events that occurred subsequent to December 31, 2014 and there were no material recognized or non-recognized subsequent events during this period.

19. Earnings per Share

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,		
	2014	2013	2012
Historical			
Numerator:			
Net loss attributable to common stockholders	$ (68,926)	$ (59,633)	$ (48,785)
Denominator:			
Weighted average common shares outstanding — basic and diluted	74,444,157	51,286,059	45,565,217

Dilutive common stock equivalents would include the dilutive effect of common stock options, restricted stock units and warrants for common stock equivalents. Potentially dilutive common stock equivalents were excluded from the diluted earnings per share denominator for all periods because of their anti-dilutive effect. The table below presents potential shares of common stock that were excluded from the computation as they were anti-dilutive using the treasury stock method (in thousands):

	Year ended December 31,		
	2014	2013	2012
Options to purchase common stock	10,021	9,041	7,974
Outstanding warrants, convertible to common stock	1,600	3,004	1,405
Unvested restricted stock units	955	—	—
Total number of potentially issuable shares	12,576	12,045	9,379

Amicus Therapeutics, Inc.

Notes To Consolidated Financial Statements — (Continued)

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

	Quarters Ended			
	March 31	June 30	September 30	December 31
2014				
Net loss	$ (15,943)	$ (14,614)	$ (17,149)	$ (21,220)
Basic and diluted net loss per common share (1)	(0.25)	(0.22)	(0.22)	(0.24)
2013				
Net loss	$ (17,458)	$ (15,349)	$ (14,589)	$ (12,237)
Basic and diluted net loss per common share (1)	(0.35)	(0.31)	(0.29)	(0.22)

(1) Per common share amounts for the quarters and full years have been calculated separately. Accordingly, quarterly amounts do not add to the annual amounts because of differences on the weighted-average common shares outstanding during each period principally due to the effect of the Company issuing shares of its common stock during the year.

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

None.

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

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our principal executive officer and principal financial officer, evaluated the effectiveness of our disclosure controls and procedures as of December 31, 2014. The term "disclosure controls and procedures," as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act, means controls and other procedures of a company that are designed to ensure that information required to be disclosed by us in the reports that we file or submit under the Exchange Act is recorded, processed, summarized and reported within the time periods specified in the SEC rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to the company's management, including its principal executive and principal financial officers, as appropriate to allow timely decisions regarding required disclosure. Management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Based on the evaluation of our disclosure controls and procedures as of December 31, 2014, our principal executive officer and principal financial officer concluded that, as of such date, our disclosure controls and procedures were effective at the reasonable assurance level.

There have been no changes in our internal controls over financial reporting during the fourth quarter of the year ended December 31, 2014 that have materially affected, or are reasonably likely to materially affect, our internal controls over financial reporting.

Management's Report on Internal Control Over Financial Reporting

The information required by this section which includes the "Management's Report on Consolidated Financial Statements and Internal Control over Financial Reporting" and the "Report of Independent Registered Public Accounting Firm" are incorporated by reference from "Item 8. Financial Statements and Supplementary Data."

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 2014 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 on 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.*

The information required by this item is incorporated by reference from the Proxy Statement under the caption "Management," "Section 16 (a) Beneficial Ownership Reporting Compliance," and "Proposal No. 1 — Election of Directors."

We have adopted a Code of Business Ethics and Conduct for Employees, Executive Officers and Directors that applies to our employees, officers and directors and incorporate guidelines designed to deter wrongdoing and to promote the honest and ethical conduct and compliance with applicable laws and regulations. In addition, the code of ethics incorporates our guidelines pertaining to topics such as conflicts of interest and workplace behavior. We have posted the text of our code on our website at *www.amicusrx.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 "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.

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.	
2.1	Agreement and Plan of Merger, dated November 19, 2013, by and among Amicus Therapeutics, Inc., CB Acquisition Corp., Callidus BioPharma, Inc, and Cuong Do	Form 8-K	2/12/2014	2.1	
3.1	Restated Certificate of Incorporation of the Registrant.	Form 10-K Annual Report	2/28/12	3.1	
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	
10.1	2002 Equity Incentive Plan, as amended, and forms of option agreements thereunder	S-1/A (333-141700)	4/27/07	10.1	
+10.2	Amended and Restated License Agreement, dated October, 31, 2008, by and between the Registrant and Mount Sinai School of Medicine of New York University	Form 10-K	2/6/09	10.3	
+10.3	License Agreement, dated as of June 26, 2003, by and between the Registrant and University of Maryland, Baltimore County, as amended	S-1 (333-141700)	3/30/07	10.4	
+10.4	Exclusive License Agreement, dated as of June 8, 2005, by and between the Registrant and Novo Nordisk, A/S	S-1 (333-141700)	3/30/07	10.5	

Exhibit No.	Filed Exhibit Description	Incorporated by Reference to SEC Filing			Filed with this Form 10-K
		Form	Date	Exhibit No.	
10.5	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	
10.6	Form of Director and Officer Indemnification Agreement	S-1 (333-141700)	3/30/07	10.17	
10.7	Amended and Restated 2007 Director Option Plan and form of option agreement	Form 8-K Current Report	6/8/10	10.2	
10.8	2007 Employee Stock Purchase Plan	S-1/A (333-141700)	5/17/07	10.24	
10.9	Lease Agreement dated as of September 11, 2008 by and between the Registrant and A/G Touchstone, TP, LLC.	Form 8-K	9/15/08	10.1	
+10.10	First Amendment to lease dated April 15, 2011 by and between the Registrant and AG Touchstone, TP, LLC	Form 10-K	2/28/12	10.13	
10.11	Letter Agreement, dated as of December 30, 2008, by and between the Registrant and David Lockhart, Ph.D.	Form 8-K	12/31/08	10.4	
10.12	Letter Agreement, dated as of December 30, 2008, by and between the Registrant and John R. Kirk	Form 10-K	2/6/09	10.29	
10.13	Summary Management Bonus Program	Form 10-K	3/3/14	10.19	
10.14	Letter Agreement, dated as of May 10, 2010 by and between the Registrant and Ken Valenzano	Form 10-K	3/4/11	10.32	
10.15	Letter Agreement, dated as of January 3, 2011 by and between the Registrant and Kenneth Peist	Form 10-K	3/4/11	10.33	
10.16	Letter Agreement, dated as of January 3, 2011 by and between the Registrant and Enrique Dilone	Form 10-K	3/4/11	10.34	
10.17	Lease Agreement dated August 16, 2011 between the Registrant and Cedar Brook 3 Corporate Center, L.P.	Form 8-K	8/16/11	10.1	

Exhibit No.	Filed Exhibit Description	Incorporated by Reference to SEC Filing			Filed with this Form 10-K
		Form	Date	Exhibit No.	
10.18	Letter Agreement dated April 18, 2013 between Amicus Therapeutics, Inc. and David J. Lockhart	Form 8-K	4/24/13	10.4	
10.19	Second Amendment to Lease Agreement dated as of May 16, 2013 by and between Amicus Therapeutics, Inc and A/G Touchstone, TP, LLC.	Form 8-K	5/22/13	10.1	
10.20	Letter Agreement, dated as of June 5, 2013 by and between the Registrant and Jeffrey P. Castelli	Form 10-Q	8/7/13	10.6	
10.21	Letter Agreement, dated as of June 5, 2013 by and between the Registrant and Jayne Gershkowitz	Form 10-Q	8/7/13	10.7	
10.22	Letter Agreement, dated November 20, 2013 by and among the Company and the purchasers identified therein	Form 8-K	11/20/13	10.1	
10.23	Form of Warrant issued on November 20, 2013	Form 8-K	11/20/13	10.2	
10.24	Credit and Security Agreement, by and between MidCap Funding III, LLC, as administrative agent, the Lenders listed in the Credit Facility Schedule thereto, Amicus Therapeutics Inc., and Callidus Biopharma, Inc., dated as of December 27, 2013	Form 8-K	12/30/13	10.1	
10.25	Separation Agreement, by and between Amicus Therapeutics, Inc and Dr. David J. Lockhart, dated as of January 3, 2014	Form 8-K	1/8/14	10.1	
+10.26	Second Restated Agreement, dated November 19, 2013 by and between Amicus Therapeutics, Inc. and Glaxo Group Limited	Form 10-K	3/3/14	10.46	
10.27	Amicus Therapeutics, Inc. Cash Deferral Plan	Form 8-K	7/2/14	10.1	
10.28	Amendment No.1 to the Amicus Therapeutics, Inc. Cash Deferral Plan	Form 8-K	10/16/14	10.1	

Exhibit No.	Filed Exhibit Description	Incorporated by Reference to SEC Filing			Filed with this Form 10-K
		Form	Date	Exhibit No.	
10.29	Employment Agreement dated April 23, 2014, between Amicus Therapeutics, Inc. and John F. Crowley	Form 8-K	4/25/14	10.1	
10.30	Employment Agreement dated April 23, 2014, between Amicus Therapeutics, Inc. and William D. Baird, III	Form 8-K	4/25/14	10.2	
10.31	Employment Agreement dated April 23, 2014, between Amicus Therapeutics, Inc. and Bradley L. Campbell	Form 8-K	4/25/14	10.3	
10.32	Employment Agreement dated April 23, 2014, between Amicus Therapeutics, Inc. and Jay Barth, M.D.	Form 10-Q	5/5/14	10.6	
10.33	Letter Agreement dated April 24, 2014, between Amicus Therapeutics, Inc. and Julie Yu	Form 10-Q	5/5/14	10.7	
10.34	Letter Agreement dated April 30, 2014, between Amicus Therapeutics, Inc. and Daphne Quimi	Form 10-Q	5/5/14	10.8	
10.35	Amended and Restated 2007 Equity Incentive Plan	Form 8-K	6/23/14	10.1	
10.36	Amicus Therapeutics, Inc. Cash Deferral Plan	Form 8-K	7/2/14	10.1	
10.37	Employment Agreement dated November 16, 2013 between Amicus Therapeutics and Hung Do	Form 10-Q	8/7/14	10.3	
10.38	Letter Agreement dated June 28, 2014 between Amicus Therapeutics and Dipal Doshi	Form 10-Q	8/7/14	10.4	
10.39	Amendment No. 1 to the Amicus Therapeutics, Inc. Cash Deferral Plan	Form 8-K	10/16/14	10.1	
21	List of Subsidiaries				X
23.1	Consent of Independent Registered Public Accounting Firm.				X

Exhibit No.	Filed Exhibit Description	Incorporated by Reference to SEC Filing			Filed with this Form 10-K
		Form	Date	Exhibit No.	
31.1	Certification of Principal Executive Officer Pursuant to Rule 13a-14(a) of the Securities Exchange Act of 1934.				X
31.2	Certification of Principal Financial Officer Pursuant to Rule 13a-14(a) of the Securities Exchange Act of 1934.				X
32.1	Certificate of Principal Executive Officer pursuant to 18 U.S.C. Section 1350 and Section 906 of the Sarbanes-Oxley Act of 2002.				X
32.2	Certificate of Principal Financial Officer pursuant to 18 U.S.C. Section 1350 and Section 906 of the Sarbanes-Oxley Act of 2002.				X
101	The following financial information from this Annual Report on Form 10-K for the year ended December 31, 2014, formatted in XBRL (Extensible Business Reporting Language) and filed electronically herewith: (i) the Consolidated Balance Sheets; (ii) the Consolidated Statements of Operations; (iii) the Consolidated Statements of Comprehensive Loss; (iv) the Consolidated Statements of Cash Flows; (v) and the Notes to the Consolidated Financial Statements.				X

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

SIGNATURES

Pursuant to the requirements of Section 13 or 15 (d) of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized on March 3, 2015.

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)	Chairman and Chief Executive Officer (Principal Executive Officer)	March 3, 2015
/s/ William D. Baird III (William D. Baird III)	Chief Financial Officer (Principal Financial Officer)	March 3, 2015
/s/ Daphne Quimi (Daphne Quimi)	Vice President, Finance and Controller (Principal Accounting Officer)	March 3, 2015
/s/ Sol J. Barer, Ph.D. (Sol J. Barer, Ph.D.)	Director	March 3, 2015
/s/ Robert Essner (Robert Essner)	Director	March 3, 2015
/s/ Donald J. Hayden (Donald J. Hayden)	Director	March 3, 2015
/s/ Ted W. Love, M.D. (Ted W. Love, M.D.)	Director	March 3, 2015

Signature	Title	Date
/s/ Margaret G. McGlynn, R.Ph. (Margaret G. McGlynn, R.Ph.)	Director	March 3, 2015
/s/ Michael G. Raab (Michael G. Raab)	Director	March 3, 2015
/s/ Glenn Sblendorio (Glenn Sblendorio)	Director	March 3, 2015
/s/ James N. Topper, M.D., Ph.D. (James N. Topper, M.D., Ph.D.)	Director	March 3, 2015

Exhibit No.	Filed Exhibit Description	Incorporated by Reference to SEC Filing			Filed with this Form 10-K
		Form	Date	Exhibit No.	
21	List of Subsidiaries				X
23.1	Consent of Independent Registered Public Accounting Firm.				X
31.1	Certification of Principal Executive Officer Pursuant to Rule 13a-14(a) of the Securities Exchange Act of 1934.				X
31.2	Certification of Principal Financial Officer Pursuant to Rule 13a-14(a) of the Securities Exchange Act of 1934.				X
32.1	Certificate of Principal Executive Officer pursuant to 18 U.S.C. Section 1350 and Section 906 of the Sarbanes-Oxley Act of 2002.				X
32.2	Certificate of Principal Financial Officer pursuant to 18 U.S.C. Section 1350 and Section 906 of the Sarbanes-Oxley Act of 2002.				X
101	The following financial information from this Annual Report on Form 10-K for the year ended December 31, 2014, formatted in XBRL (Extensible Business Reporting Language) and filed electronically herewith: (i) the Consolidated Balance Sheets; (ii) the Consolidated Statements of Operations; (iii) the Consolidated Statements of Comprehensive Loss; (iv) the Consolidated Statements of Cash Flows; (v) and the Notes to the Consolidated Financial Statements.				X

Exhibit 21

List of Subsidiaries of the Registrant

Callidus Biopharma, Inc. (Delaware)
Amicus Therapeutics UK Limited (UK)

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Exhibit 21

List of Subsidiaries of the Registrant

EXHIBIT 23.1

Consent of Independent Registered Public Accounting Firm

We consent to the incorporation by reference in the following Registration Statements:

1. Registration Statement (Form S-8 No. 333-197202) pertaining to the Amicus Therapeutics, Inc. Cash Deferral Plan,
2. Registration Statement (Form S-8 No. 333-195194) pertaining to the Amicus Therapeutics, Inc. Restricted Stock Unit Deferral Plan,
3. Registration Statement (Form S-8 No. 333-145305) pertaining to the: 1) Amicus Therapeutics, Inc. 2002 Equity Incentive Plan, as Amended, 2) Amicus Therapeutics, Inc. 2007 Equity Incentive Plan, 3) Amicus Therapeutics, Inc. 2007 Director Option Plan, 4) Amicus Therapeutics, Inc. 2007 Employee Stock Purchase Plan,
4. Registration Statement (Form S-8 No. 333-157219) pertaining to the: 1) Amicus Therapeutics, Inc. Amended and Restated 2007 Equity Incentive Plan and 2) Amicus Therapeutics, Inc. 2007 Director Option Plan,
5. Registration Statement (Form S-8 No. 333-174900) pertaining to the: 1) Amicus Therapeutics, Inc. Amended and Restated 2007 Equity Incentive Plan and 2) Amicus Therapeutics, Inc. Amended and Restated 2007 Director Option Plan,
6. Registration Statement (Form S-3 No. 333-185307),
7. Registration Statement (Form S-3 No. 333-184531),
8. Registration Statement (Form S-3 No. 333-192747),
9. Registration Statement (Form S-3 No. 333-192876)

of our reports dated March 3, 2015 with respect to the consolidated financial statements of Amicus Therapeutics, Inc., and the effectiveness of internal control over financial reporting of Amicus Therapeutics, Inc included in this Annual Report (Form 10-K) for the year ended December 31, 2014.

/s/ Ernst & Young LLP

MetroPark, New Jersey
March 3, 2015

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EXHIBIT 23.1

Consent of Independent Registered Public Accounting Firm

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EXHIBIT 31.1

CERTIFICATIONS PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002
CERTIFICATION BY PRINCIPAL EXECUTIVE OFFICER

I, John F. Crowley, certify that:

1. I have reviewed this annual report on Form 10-K of Amicus Therapeutics, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15 (f) and 15d-15(f)) for the registrant and have:

a. designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

b. designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

c. evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

d. disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting;

5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

a. all significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

b. any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: March 3, 2015

/s/ John F. Crowley

John F. Crowley
Chairman and Chief Executive Officer

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EXHIBIT 31.2

**CERTIFICATIONS PURSUANT TO SECTION 302 OF
THE SARBANES-OXLEY ACT OF 2002
CERTIFICATION BY PRINCIPAL FINANCIAL OFFICER**

I, William D. Baird III, certify that:

1. I have reviewed this annual report on Form 10-K of Amicus Therapeutics, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15 (f) and 15d-15(f)) for the registrant and have:

a. designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

b. designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

c. evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

d. disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting;

5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

a. all significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

b. any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: March 3, 2015

/s/ William D. Baird III

William D. Baird III
Chief Financial Officer

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EXHIBIT 32.1

Certification by the Principal Executive Officer Pursuant to 18 U. S. C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002

Pursuant to 18 U. S. C. Section 1350, I, John F. Crowley, hereby certify that, to the best of my knowledge, Amicus Therapeutics Inc., (the "Company") Annual Report on Form 10-K for the year ended December 31, 2014 (the "Report"), as filed with the Securities and Exchange Commission on March 3, 2015, 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
Chairman and Chief Executive Officer
March 3, 2015

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EXHIBIT 32.1

Certification by the Principal Executive Officer Pursuant to 18 U. S. C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002

EXHIBIT 32.2

Certification by the Principal Financial Officer Pursuant to 18 U. S. C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002

Pursuant to 18 U. S. C. Section 1350, I, William D. Baird III, 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, 2014 (the "Report"), as filed with the Securities and Exchange Commission on March 3, 2015, 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/ William D. Baird III

William D. Baird III
Chief Financial Officer
March 3, 2015

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EXHIBIT 32.2

Certification by the Principal Financial Officer Pursuant to 18 U. S. C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002