AMICUS THERAPEUTICS INC
Form 10-K
March 03, 2014

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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, 2013
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 o No ý

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

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 ý No o

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 ý No o

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

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, 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 o	Accelerated filer ý	Non-accelerated filer o (Do not check if a smaller reporting company)	Smaller Reporting Company o

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

The aggregate market value of the 22,851,645 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, 2013) was approximately $53,244,333. 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, 2014, there were 62,263,325 shares of common stock outstanding.

DOCUMENTS INCORPORATED BY REFERENCE: Portions of the Proxy Statement for the registrant's 2014 Annual Meeting of Stockholders which is to be filed subsequent to the date hereof are incorporated by reference into Part III of this Annual Report on Form 10-K.

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SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This annual report on Form 10-K contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts, included in this annual report on Form 10-K regarding our strategy, future operations, future financial position, future revenues, projected costs, prospects, plans and objectives of management are forward-looking statements. The words "anticipate," "believe," "estimate," "expect," "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;
- the cost of manufacturing drug supply for our clinical and preclinical studies, including the significant cost of ERT cell line development and manufacturing as well as the cost of manufacturing the vIGF-2 peptide tag;
- 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 and technologies;
- our ability to successfully incorporate Callidus Biopharma, Inc. ("Callidus") and its product candidates and technology into our business; 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.

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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 development programs include next-generation enzyme replacement therapies ("ERTs") for LSDs, including Fabry disease, Pompe disease and Mucopolysaccharoidosis Type I ("MPS I"). We are also developing novel small molecules as monotherapy treatments for Fabry disease and Parkinson's disease. 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 Pompe and Fabry, 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 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 Chaperone-Advanced Replacement Therapy, or CHART , platform has been used to develop our next-generation ERTs by co-formulating therapeutic enzymes with our proprietary pharmacological chaperones. 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 lead CHART program is a next-generation ERT in preclinical development for Fabry disease. This next-generation ERT consists of a proprietary human recombinant alpha-Galactosidase enzyme ("alpha-Gal") enzyme (designated "AT-B100") co-formulated with our pharmacological chaperone migalastat HCl. We completed an initial human proof-of-concept Phase 2 study ("Study 013") that evaluated the effects of a single oral dose of migalastat HCl co-administered with the currently marketed ERTs for Fabry disease (Fabrazyme® or Replagal®) in males with Fabry disease. Results from this study demonstrated a consistent increase in levels of active alpha-Gal activity, the enzyme deficient in Fabry patients, in plasma and increased uptake of alpha-Gal enzyme in skin compared to ERT alone. This study has served as the foundation for further development of our next-generation Fabry ERT, which is anticipated to enter the clinic in 2014.

We are also developing migalastat HCl as a monotherapy in two Phase 3 global registration studies ("Study 011" and "Study 012") for Fabry patients with genetic mutations that were amenable to this pharmacological chaperone in a cell-based assay. Study 011 is a 24-month study consisting of a 6-month double-blind, placebo-controlled treatment period (Stage 1); a 6-month open-label follow-up period (Stage 2); and a 12-month open-label extension phase. In December 2012, we announced top-line six-month (Stage 1) results from Study 011. Data from Stage 2 and the open label extension for months 13-24 are anticipated in the first half of 2014.

In Study 012, we are comparing open-label migalastat HCl to current standard of care ERTs (Fabrazyme® and Replagal®) to potentially support global registration. In December 2012, this study achieved full enrollment of 60 patients, who were randomized 1.5:1 to switch from ERT to migalastat HCl or remain on ERT. Data are anticipated in the second half of 2014 on the primary outcome

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measure, which is change in renal function assessed by iohexol (measured) Glomerular Filtration Rate ("GFR") at 18 months. The analysis will compare migalastat to ERT at study month 18.

In November 2013, we acquired Callidus Biopharma, Inc. ("Callidus"), a privately held biotechnology company. Callidus was engaged in developing a next-generation Pompe ERT and complimentary enzyme targeting technologies. Through our acquisition of Callidus, we now own a uniquely-engineered, proprietary recombinant human acid-alpha glucosidase (rhGAA called "AT-B200") for Pompe disease that is in late preclinical development. Acid alpha-glucosidase ("GAA") is the enzyme deficient in Pompe patients. AT-B200 is differentiated from other Pompe ERTs by its unique carbohydrate structure. This ERT may be further optimized by applying our proprietary peptide tagging technology for better targeting. Preclinical results have shown that AT-B200 was better than Lumizyme for clearing glycogen (the accumulated substrate in Pompe disease) in skeletal muscles in Gaa knock-out mice.

AT-B200 may deliver further benefits through co-formulation with our pharmacological chaperone AT2220 (duvoglustat HCl). In preclinical studies of AT2220 co-administered and co-formulated with Myozyme/Lumizyme, greater enzyme uptake in disease-relevant tissues led to greater glycogen reduction compared to either of these ERTs alone. Similar to Study 013 in Fabry disease, we completed a Phase 2 ("Study 010") safety and pharmacokinetics study of AT2220 co-administered with currently approved recombinant rhGAA Myozyme®/Lumizyme® in Pompe patients. Results from Study 010 demonstrated initial human proof-of-concept for this chaperone-ERT combination in Pompe disease, showing an increase in GAA enzyme activity in plasma and muscle compared to ERT alone. Taken together these results support the further investigation of a next-generation ERT that combines AT-B200 with a pharmacological chaperone for Pompe disease.

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 enzyme activity and stability; 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 six LSDs were approximately $3.0 billion in 2013.

In addition to our programs addressing LSDs, we are researching the use of pharmacological chaperones for the treatment of Parkinson's disease. In September 2013, we entered into a collaboration agreement with Biogen Idec ("Biogen") to discover, develop and commercialize novel small molecules for the treatment of Parkinson's disease. Under terms of the multi-year agreement, we will collaborate in the discovery of a new class of small molecules that target the glucocerobrosidase ("GCase") enzyme, for further development and commercialization by Biogen. Biogen will be responsible for funding all discovery, development and commercialization activities.

Our Pharmacological Chaperone Technology

We are leveraging its 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 results 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.

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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 protein lysosomal enzymes for proper trafficking to the lysosome, which also alleviates the toxic 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 unfolding of infused enzyme include:

reduced stability and 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 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.

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Enzyme Targeting Technology

Most lysosomal enzyme replacement therapies (ERTs) contain a specialized carbohydrate called mannose 6-phosphate (M6P) which enables binding and delivery of therapeutic drug to lysosomes via M6P receptors on cell surfaces. We are developing next-generation ERTs with significantly higher amounts of M6P for improved lysosomal targeting compared to existing ERTs. We are also developing an approach to attach a peptide tag onto ERTs to ensure good drug targeting. This novel approach capitalizes on the unique feature of the M6P receptor which can simultaneously bind both M6P and a small peptide which naturally circulates in blood called insulin-like growth factor 2 (IGF-2) and is therefore called the IGF2/M6P receptor. We developed methodologies for attaching a proprietary peptide Lag (a variant of IGF-2, or vIGF-2) onto our next-generation ERT's to Further enhance lysosomal targeting to key tissues of disease. The vIGF-2 peptide was specifically designed to bind the intended IGF-2/M6P receptor with high affinity, potentially minimizing off-target effects such as hypoglycermia and cellular proliferation. *In vivo* proof-of-concept for this peptide tagging approach has been achieved to demonstrate better substrate clearance in animal models for two different LSDs. 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 HCl for Fabry Disease

Overview

Our most advanced product candidate, migalastat HCl, is an investigational, small molecule pharmacological chaperone for the treatment of Fabry disease. As an orally administered monotherapy, migalastat HCl is designed to bind to and stabilize, or "chaperone" a patient's own alpha-Gal enzyme in those patients with genetic mutations identified as amenable to this chaperone in a cell-based assay. For all other Fabry patients, migalastat HCl in combination with ERT may improve patient outcomes by keeping infused alpha-Gal enzyme in its properly folded and active form.

Clinical Studies of Migalastat HCl Monotherapy for Fabry Disease

Study 011 is a 24-month study consisting of a 6-month double-blind, placebo-controlled treatment period (Stage 1); a 6-month open-label follow-up period (Stage 2); and a 12-month open-label extension phase. The study randomized 67 patients (24 males and 43 females) diagnosed with Fabry disease who had genetic mutations amenable to chaperone monotherapy in a cell-based assay. During Stage 1, patients were randomized 1:1 to migalastat HCl 150 mg or placebo on an every-other-day (QOD) oral dosing schedule. Patients continued treatment with migalastat HCl or switched from placebo to migalastat HCl during Stage 2 and the open-label extension phase. Change from baseline in kidney interstitial capillary globotriaosylceramide (GL-3) is being assessed by histology in kidney biopsies at the end of Stage 1 and Stage 2. GL-3 is the lipid substrate that accumulates in tissues of patients with Fabry disease, and is measured in kidney biopsies. Safety and tolerability, and kidney function as measured by estimated glomerular filtration rate (eGFR), are being assessed throughout the 24-month study.

Top-line Stage 1 data from Study 011 was reported in December 2012 and presented at the Lysosomal Disease Network WORLD Symposium (LDN WORLD) in February 2013. During Stage 1, no drug-related serious adverse events were observed. No subjects discontinued migalastat HCl therapy due to a treatment emergent adverse event and the majority of adverse events in both treatment groups were mild in nature. The primary analysis compared the number of responders in the migalastat HCl versus placebo groups, based on a 50% or greater reduction in interstitial capillary GL-3 during Stage 1. In the primary responder analysis, 13/32 (41%) in the migalastat HCl group versus 9/32 (28%) in the placebo group demonstrated a 50% or greater reduction in kidney interstitial capillary GL-3

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from baseline to month 6 which was not statistically significant (p=0.3). Certain 6-month secondary endpoints presented included urine GL-3 and renal function as measured by eGFR.

Updated Stage 1 data from Study 011, including a post-hoc analysis of the mean change from baseline in inclusions per capillary as a continuous variable ("mean change in GL-3"), were presented at LDN WORLD in February 2014. Following the unblinding of the Stage 1 data, and while still blinded to the Stage 2 data, we collaborated with GSK and external statistical consultants to identify a more appropriate way to measure the biological effect of migalastat HCL given the variability in patients' GL-3 levels in Study 011. We used this revised methodology to analyze the mean change in GL-3 from baseline to month 6 in both the modified intent-to-treat (mITT) population (n=60, 30 per group) as well as in the subgroup of patients who had amenable mutations in a GLP-validated HEK assay ("GLP HEK amenable") (n=46, 25 in the migalastat HCl group and 21 in the placebo group). From baseline to month 6 in the mITT population, the mean change in GL-3 was -0.22 ± 0.11 in the migalastat group compared to +0.06 ± 0.09 in the placebo group (p=0.052).

All subjects enrolled in Study 011 had amenable mutations in a clinical trial human embryonic kidney (HEK) cell-based in vitro assay available at study initiation ("clinical trial assay"). During the course of the study and prior to unblinding, a more robust GLP-validated version of the HEK assay ("GLP HEK assay") was created and used to reanalyze the mutations in subjects enrolled in the study. Approximately 90% of mutations remained in the same category of "amenable" or "non-amenable" using the same pre-defined in vitro criteria, however, there were some changes in categorization since the clinical trial and GLP HEK assays are not identical. Using the more robust GLP HEK amenable assay, the mean change in GL-3 from baseline to month 6 in the HEK amenable subgroup was -0.31 ± 0.12 in the migalastat group compared to +0.10 ± 0.13 in the placebo group (p=0.002).

The Stage 2 treatment period in Study 011 was completed in December 2012 and the 12-month extension phase was completed in December 2013. The Stage 2 results, including mean change in GL-3 at 12 months, as well as complete data from the 24-month study, including clinical outcome measures such as eGFR and proteinuria, are expected during the second quarter of 2014.

Study 012 is a randomized, open-label 18-month Phase 3 study investigating the safety and efficacy of oral migalastat HCl (150 mg, every-other-day) compared to standard-of-care infused ERTs (Fabrazyme® and Replagal®). The study enrolled a total of 60 patients (males and females) with Fabry disease and genetic mutations identified as amenable to migalastat HCl monotherapy in a cell-based assay. Subjects were randomized 1.5:1 to switch to migalastat HCl 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. The primary outcome measure is renal function assessed by measured GFR at 18 months, evaluated in the migalastat HCl and ERT groups using descriptive statistics. This study achieved full enrollment in December 2012 and top-line results are expected in the second half of 2014.

We expect to receive final data from Study 011 and Study 012 in 2014. Based on the outcome of those studies, we will initiate discussions with U.S. and EU regulatory authorities to discuss a potential regulatory path to approval.

Next-Generation ERT for Fabry Disease

We have investigated the use of migalastat HCl co-administered with currently marketed ERTs (Fabrazyme® and Replagal®) in preclinical and clinical studies, as well as migalastat HCl co-formulated with a proprietary human recombinant alpha-Gal enzyme (JCR Pharmaceutical Co Ltd's JR-051) in preclinical studies. Based on these studies we are leveraging our CHART platform to advance migalastat HCl co-formulated with human recombinant alpha-Gal enzyme (designated AT-B100).

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CHART for Fabry Disease: Clinical and Preclinical Chaperone-ERT Combination Studies

Amicus, together with GSK and JCR Pharmaceutical Co Ltd ("JCR"), completed preclinical studies to evaluate migalastat HCl co-formulated with JCR's proprietary investigational ERT (JR-051, recombinant human alpha-Gal enzyme) in. Results from these preclinical studies suggest that migalastat HCl co-formulated with JR-051 may provide greater alpha-Gal enzyme uptake into tissue and markedly reduced levels of GL-3 in Fabry disease-relevant tissues compared to JR-051 alone. Based on these results, we plan to advance migalastat HCl co-formulated with ERT for Fabry disease. The first planned clinical study will investigate the PK of IV migalastat HCl in healthy volunteers to identify optimal doses for a Phase 2 clinical study of migalastat HCl co-formulated with ERT in Fabry patients. For the Phase 1/2 study, we expect to use migalastat HCl co-formulated with JR-051. In parallel, we are currently evaluating our long-term strategy for supplying late-stage clinical and commercial ERT, which may include developing or in-licensing a recombinant alpha-Gal A enzyme comparable to JR-051. We completed an open-label Phase 2 drug-drug interaction study in 23 males with Fabry disease to evaluate the safety and pharmacokinetic (PK) effects of two doses of migalastat HCl (150 mg and 450 mg) co-administered with currently marketed ERTs infused alpha-Gal enzymes, Fabrazyme® (agalsidase beta) and Replagal® (agalsidase alfa). 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 HCl is designed to bind to and stabilize the recombinant 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 HCl (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 increased alpha-Gal enzyme in skin following co-administration compared to ERT alone.

Causes of Fabry Disease and Rationale for Use of Migalastat HCl

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

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

Because migalastat HCl increases levels of a patient's naturally produced α-Gal, Fabry disease patients most likely to respond to treatment with migalastat HCl 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 enzyme activity. We estimate that approximately thirty to fifty percent of patients with Fabry disease may have alpha-Gal mutations that are amenable to migalastat HCl as a monotherapy. Patients with genetic mutations leading to a partially made alpha-Gal enzyme or

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alpha-Gal enzyme with an irreversible loss of activity are less likely to respond to treatment with migalastat HCl as a monotherapy. However, we believe that all Fabry patients are potentially treatable with migalastat HCl in combination with ERT.

The combination of migalastat HCl and ERT is designed to bind and stabilize infused enzyme in circulation as patients receive ERT. We believe migalastat HCl 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 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 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 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.

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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 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 two-hundred and sixty-three thousand newborns, and found the incidence of Fabry mutations to be between 1:2,400 to 1: 3859. This high incidence was attributed to a large number of newborn males with alpha-Gal mutations often associated with later-onset Fabry disease, which may not have been identified in previous screening studies that relied on diagnosis based on development of symptoms of classic Fabry disease.

Fabry Disease Market Opportunity

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

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

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 with some residual enzyme activity, we believe patients with these mutations may benefit from treatment with monotherapy migalastat HCl. We also believe that other types of genetic mutations may result in misfolded alpha-Gal and therefore may also respond to treatment with monotherapy migalastat HCl. Based on this, we believe that approximately thirty to fifty percent of the Fabry disease patient population may benefit from treatment with migalastat HCl as a

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

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

Currently, two ERT products are approved for the treatment of Fabry disease: Fabrazyme® (agalsidase beta) and Replagal® (agalsidase alfa). Fabrazyme® is approved globally (conditionally in the U.S.) and commercialized by sanofi aventis through Genzyme Corporation, while Replagal® is commercialized by Shire and approved in the EU and other countries but not in the U.S. Orphan drug exclusivity for both Fabrazyme® and Replagal® has expired in the EU and for Fabrazyme® , in the U.S. as well. The net product sales of Fabrazyme® and Replagal® for 2013 were approximately $525 million as publicly reported by sanofi aventis and $468 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 HCl, we believe that the use of migalastat HCl may have advantages relative to the use of Fabrazyme® and Replagal®. Published data for patients treated with Fabrazyme® and Replagal® for periods of up to five years demonstrate that these drugs can lead to the reduction of GL-3 in multiple cell types in the skin, heart and kidney. However, because they are large protein molecules, Fabrazyme® and Replagal® are believed to have difficulty penetrating some tissues and cell types. In particular, it is widely believed that Fabrazyme® and Replagal® are unable to cross the blood-brain barrier and thus are unlikely to address the neurological symptoms of Fabry disease. As a small molecule therapy that has demonstrated high oral bioavailability and good biodistribution properties in preclinical testing, migalastat HCl has the potential to reach cells of all the target tissues of Fabry disease. Furthermore, treatment with Fabrazyme® and Replagal® requires intravenous infusions every other week, frequently on-site at health care facilities, presenting an inconvenience to Fabry patients. Hence, oral treatment with migalastat HCl may be much more convenient for patients. Lastly, Fabrazyme® and Replagal® 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 HCl 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 HCl in combination with ERT may improve key characteristics of the infused enzymes used in ERT by allowing for increased transport of enzymes to the lysosomes and degradation of substrate, thereby potentially increasing ERT's efficacy. Importantly, patients who may not have alpha-Gal mutations amendable to migalastat HCl monotherapy treatment may benefit from migalastat HCl in combination with ERT, making migalastat HCl potentially available to all Fabry patients.

Next-Generation ERT for Pompe Disease

We are utilizing our CHART platform in combination with our uniquely-engineered, proprietary recombinant human acid-alpha glucosidase (rhGAA designated AT-B200) to develop a next-generation ERT for Pompe disease. We are currently investigating AT-B200, with and without a pharmacological chaperone, in preclinical studies.

We acquired AT-B200 as well as our enzyme targeting technology through our purchase of Callidus. AT-B200 is differentiated from other Pompe ERTs by its unique carbohydrate structure, and may be further optimized by applying our proprietary peptide tagging technology for better targeting.

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AT-B200 may also deliver further benefits through co-formulation with our pharmacological chaperone AT2220 (duvoglustat HCl).

The results from preclinical studies of AT-B200 taken together with data from our clinical and preclinical studies of AT2220 in combination with ERT support our further development of a next-generation ERT for Pompe disease.

Preclinical Studies of AT-B200 for Pompe Disease

In preclinical studies, AT-B200 was shown to have superior uptake and activity in disease-relevant tissues that correlated with clearance of accumulated glycogen substrate when compared to current standard of care. AT-B200 may be further improved through the application of the Company's proprietary conjugation technology to attach vIGF2 (a variant of the insulin growth factor 2 receptor) to further enhance drug targeting. The vIGF2 peptide binds the intended IGF2 receptor (responsible for lysosomal targeting), but does not bind to the insulin receptor or the IGF1 receptor. Preclinical results have shown that AT-B200 and AT-B200 conjugated with vIGF-2 were better than Lumizyme for clearing glycogen in skeletal muscles in Gaa knock-out mice.

CHART for Pompe Disease: Clinical and Preclinical Chaperone-ERT Combination Studies

In January 2013, we announced positive preliminary results from all 4 dose cohorts in a Phase 2 open-label, multi-center study (Study 010) that evaluated the safety and PK effects of the pharmacological chaperone AT2220 (duvoglustat HCl) co-administered with Myozyme® or Lumizyme® (alglucosidase alfa or recombinant human GAA enzyme rhGAA), the only approved treatments for Pompe disease. Male and female Pompe patients enrolled in Study 010 were given a regularly scheduled ERT infusion. One hour prior to the initiation of the next ERT infusion, patients received a single oral dose of AT2220 (50 mg, 100 mg, 250 mg, or 600 mg). Plasma rhGAA activity and protein levels were evaluated during each infusion. Each patient underwent muscle biopsies two or seven days after each infusion to measure tissue GAA enzyme activity with and without the chaperone, as well as to measure the level of AT2220 in the muscle. The results from all 4 dose cohorts established human proof-of-concept that co-administration of AT2220 just prior to infusing ERT increases GAA enzyme activity in muscle tissue compared to ERT alone. In February 2013, we presented data from preclinical studies of AT2220 co-formulated with rhGAA enzyme (Myozyme®/Lumizyme®) for the first time. These data showed that this chaperone-ERT co-formulation resulted in up to 2.5-fold greater enzyme uptake and glycogen reduction in multiple disease-relevant tissues compared to rhGAA alone in GAA knock-out mice. Collectively these data suggest that AT2220 directly binds to and stabilizes rhGAA, potentially leading to a larger fraction of properly folded, active enzyme in the circulation that is more accessible for tissue uptake. AT2220 co-formulated with ERT may also mitigate Pompe ERT-related immunogenicity since properly folded proteins are less prone to aggregation and less immunogenic. Results from these studies support the development of our next-generation ERT for Pompe disease.

Pompe Disease Background

Like Fabry disease, Pompe disease is a 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

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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 a Revised Agreement (the "Revised Agreement") with GSK, pursuant to which we have obtained global rights to develop and commercialize migalastat HCl as a monotherapy and in combination with ERT for Fabry disease. The Revised Agreement amends and replaces in its entirety the Expanded Agreement between us and GSK in July 2012. Under the terms of the Revised Agreement, there is no upfront payment from Amicus to GSK. For the next-generation Fabry ERT (migalastat HCl co-formulated with ERT), GSK is eligible to receive single-digit royalties on net sales in eight major markets outside the U.S. For migalastat HCl 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. This agreement also terminates the co-exclusive license from GSK to use JR051 in development of Co-Form Product. We have the sole responsibility, at our sole cost and expense, to seek such a license from JCR Pharmaceuticals, Co. Ltd. if it is deemed necessary.

In November 2013, we entered into securities purchase agreement (the "2013 SPA") with GSK and certain entities controlled by Redmile Group, LLC for the private placement of (a) shares of the Company's common stock, par value $0.01 (the "Common Stock") and (b) 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 shareholders prior to consummation of these transactions. Pursuant to the 2013 SPA, we agreed to issue (a) 1.5 million Shares at $2.00 per Share to GSK and (b) 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. We received total proceeds of $15 million for general corporate and working capital purposes as a result of the

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private placement and the transaction closed on November 20, 2013. As of December 31, 2013, GSK's resulting equity stake in the Company was 17.6%.

In September 2013, the Company entered into a collaboration agreement with Biogen Idec ("Biogen") to discover, develop and commercialize novel small molecules for the treatment of Parkinson's disease. The collaboration will build upon our preclinical studies and independent published research that suggest increasing activity of the lysosomal enzyme GCase in the brain may correct alpha-synuclein pathology and other deficits associated with Parkinson's disease. Under terms of the multi-year agreement, the Company and Biogen 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 will be responsible for funding all discovery, development, and commercialization activities. In addition, the Company will be reimbursed for all full-time employees working on the project. The Company is also eligible to receive development and regulatory milestones, as well as modest royalties on global net sales.

We will continue to evaluate other business development opportunities as appropriate that build shareholder value and provide us with access to the financial, technical, clinical and commercial resources necessary to develop and market pharmacological chaperone therapeutics 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

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methods of increasing deficient enzyme activity to treat genetic diseases. The patent positions for migalastat HCl, pharmacological chaperone and ERT combination therapy, diseases of neurodegeneration, afegostat and its derivates including AT3375 for Gaucher disease and AT2220 (duvoglustat HCl) for Pompe disease are described below and include both patents and patent applications we own or exclusively license:

We have an exclusive license to six issued U.S. patents that cover use of migalastat HCl to treat Fabry disease, as well as corresponding European, Japanese and Canadian patents. These exclusively licensed U.S. patents relating to migalastat HCl 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 HCl. In addition, we own pending U.S. applications directed to dosing regimens with migalastat HCl, 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 HCl, 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 HCl 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 HCl 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 HCl 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 HCl with ERT (recombinant α-galactosidase A), afegostat with ERT (recombinant glucocerebrosidase) and AT2220 (duvoglustat HCl) with ERT(recombinant acid α-glucosidase). Patents covering specific combinations have issued in 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, 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 hydrochloride to treat Fabry disease in combination with ERT (recombinant α-galactosidase A). Similarly, we own a U.S. provisional patent application that covers specific doses and dosing regimens of duvoglustat HCl to treat Pompe disease in combination with ERT (recombinant acid α-glucosidase). If patents issue from these applications, expiration will be in 2032 to 2033.

We own an international patent application covering a high concentration co-formulation of recombinant acid α-glucosidase and pharmacological chaperone. If patents issue from this international application, expiration will be in 2033. We also own an international patent application covering stable parenteral compositions containing duvoglustat HCl. If patents issue from this international application, expiration will be in 2034.

We own an international patent application covering a co-formulation of recombinant α-galactosidase A and migalastat. If patents issue from this international application, expiration will be in 2033.

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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.

We own several US and foreign pending patent applications which cover the use of pharmacological chaperones to treat diseases of neurodegeneration. In particular, we own two issued patents and two U.S. patent applications that cover the use of afegostat and/or its derivatives to treat Parkinson's disease as well as one patent application covering novel compounds, including AT3375, for the treatment of Parkinson's disease. We own another patent application covering the use of the same novel compounds, including AT3375, for the treatment of Gaucher disease as a monotherapy as well as in combination with ERT. If patents issue from these applications, expiration dates will range from 2026 to 2031.

We have an exclusive license to several U.S. patents covering the use of afegostat to treat Gaucher disease. These patents expire in 2018 (not including the Hatch-Waxman statutory extension, which is described below). There are no ex-U.S. counterparts to the exclusively licensed U.S. patents, which expire in 2018 in the U.S., covering afegostat to treat Gaucher disease. We also have an exclusive license to two U.S. patents claiming afegostat, the active chemical moiety in Plicera, which expire in 2015 and 2016 (not including the Hatch-Waxman statutory extension, which is described below); and corresponding patents in the UK, France, Sweden, Germany, Switzerland and Japan all of which expire in 2015 (not including the SPC extensions, which are described below). We own a U.S. patent and its corresponding foreign patents covering afegostat, which is the specific salt form or the active pharmaceutical ingredient in Plicera, which expires in 2027. We own issued U.S., Australian, Japanese and Mexican patents directed to the synthesis of afegostat.

We have an exclusive license to several U.S. patents that cover the use of AT2220 (duvoglustat) to treat Pompe disease as a monotherapy. These U.S. patents will expire in 2018 (not including the Hatch-Waxman statutory extension, which is described below). There are no ex-U.S. counterparts to the exclusively licensed U.S. patents, which expire in 2018 in the U.S., covering the monotherapy use of AT2220 to treat Pompe disease.

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,

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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.

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License Agreements

We have acquired rights to develop and commercialize our product candidates through licenses granted by various parties. For information regarding our migalastat HCl 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 HCl, afegostat and duvoglustat HCl 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.

University of Maryland, Baltimore County We have acquired exclusive U.S. patent rights to develop and commercialize afegostat for the treatment of Gaucher disease from the University of Maryland, Baltimore County. Under this agreement, to date we have paid aggregate upfront and annual license fees of $45 thousand. We are required to make a milestone payment upon the demonstration of safety and efficacy of afegostat for the treatment of Gaucher disease in a Phase 2 study, and another payment upon receiving FDA approval for afegostat for the treatment of Gaucher disease. We are also required to pay royalties on net sales. Upon satisfaction of both milestones, we could be required to make up to $0.2 million in aggregate payments. This agreement expires upon expiration of the last of the licensed patent rights in 2015.

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

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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 HCl and AT2220, we will owe royalties only to MSSM and will owe no milestone payments. We would expect to pay royalties to all three licensors with respect to afegostat.

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

Trademarks

In addition to our patents and trade secrets, we own certain trademarks in the U.S. and/or abroad, including A 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. Although we previously filed for approval of the tradename "Amigal", we will re-apply for registration of a new tradename for migalastat HCl based on feedback from FDA prohibiting the use of Amigal for migalastat HCl.

Manufacturing

We continue to rely on contract manufacturers to supply the active pharmaceutical ingredients and final drug product for migalastat HCl, 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, along with our collaboration with GSK, provide us with competitive advantages, we face potential competition from many different sources, including commercial enterprises, academic institutions, government agencies and private and public research institutions. Any product candidates that we successfully develop and commercialize will compete with both existing and new therapies that may become available in the future.

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

Our commercial opportunities could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer side effects, are more convenient or

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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	2013 Sales (in millions USD)
sanofi aventis	Fabry disease	Fabrazyme®	Enzyme Replacement Therapy	Marketed	$ 525
	Gaucher disease	Cerezyme®	Enzyme Replacement Therapy	Marketed	$ 943
	Pompe disease	Myozyme®/Lumizyme®	Enzyme Replacement Therapy	Marketed	$ 685
	Gaucher disease	Eliglustat tartrate	Substrate Reduction Therapy	Phase 3	N/A
	Fabry disease	GZ402671	Oral GCS Inhibitor	Phase 1	N/A
	Pompe disease	GZ402666 ("neo GAA")	Enzyme Replacement Therapy	Phase 1	N/A
Shire	Fabry disease	Replagal®	Enzyme Replacement Therapy	Marketed	$ 468
	Gaucher disease	VPRIV®	Enzyme Replacement Therapy	Marketed	$ 343
Biomarin Pharmaceutical, Inc.	Pompe disease	BMN-701	Enzyme Replacement Therapy	Phase 2/3	N/A
	Mucopolysaccharidosis I (MPS I)	Aldurazyme®	Enzyme Replacement Therapy	Marketed	$ 212
Actelion, Ltd.	Gaucher disease	Zavesca®	Substrate Reduction Therapy	Marketed	$ 108
Protalix Biotherapeutics	Gaucher disease	Elelyso®	Enzyme Replacement Therapy	Marketed	N/A
	Fabry disease	PRX-102	Enzyme Replacement Therapy	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 new drug applications (NDAs) or biologic product license applications (BLAs),

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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 evaluation, 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 new drug application (NDA) or biologic product license (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.

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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 a 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 new drug applications. 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

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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 (sBLA) 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

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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 ANDAs 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 current good manufacturing practices, or 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.

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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

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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 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

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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 EEA for migalastat HCl for the treatment of Fabry disease and for afegostat for the treatment of Gaucher disease. We anticipate filing for orphan medicinal product designation from the EMA for AT2220 for the treatment of Pompe 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

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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.

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Employees

As of December 31, 2013, we had 92 full-time employees, 64 of whom were primarily engaged in research and development activities and 28 of whom provide administrative services. A total of 23 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 A AMICUS THERAPEUTICS® and design and AMICUS THERAPETUICS®. Fabrazyme®, Cerezyme®, Myozyme®, Lumizyme®, Replagal® , VPRIV® and Zavesca® are the property of their respective owners.

ITEM 1A. RISK FACTORS

The occurrence of any of the following risks could harm our business, financial condition, results of operations and/or growth prospects. In that case, the trading price of our common stock could decline, and you may lose all or part of your investment. 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 Financial Position and Need for Additional Capital

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

Since inception, we have incurred significant operating losses. Our cumulative net loss attributable to common stockholders since inception was $398.7 million and we had an accumulated deficit of $378.5 million as of December 31, 2013. 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 HCl for the treatment of Fabry disease to support regulatory approval in the United States and worldwide;

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begin Phase 1 clinical studies of migalastat HCl in 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 HCl. Further, subject to obtaining regulatory approval of any of our product candidates including migalastat HCl, 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 HCl as of January 1, 2014. With the exception of our Parkinson's disease program which is funded through our collaboration with Biogen, we are responsible for all research and development costs for all of our programs.

In order to complete clinical trials related to migalastat HCl, seek regulatory approvals of migalastat HCl, 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, especially in light of the current challenging economic environment. If adequate funds are not available to us on a timely basis, we may be required to reduce or eliminate research development programs or commercial efforts.

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

the progress and results of our clinical trials of migalastat HCl;

the cost of manufacturing drug supply for our clinical and preclinical studies, including the significant cost of ERT cell line development and manufacturing as well as the cost of manufacturing the vIGF-2 peptide tag;

the scope, progress, results and costs of preclinical development, laboratory testing and clinical trials for our other product candidates including those testing the use of pharmacological

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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;

our ability to successfully incorporate Callidus Biopharma, Inc. (Callidus) and its product candidates and technology into our business; and

our ability to establish additional collaborations and obtain milestone, royalty or other payments from any such collaborators.

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

Until such time, if ever, as we generate product revenue to finance our operations, we expect to finance our cash needs through public or private equity offerings and debt financings, corporate collaboration and licensing arrangements and grants from patient advocacy groups, foundations and government agencies. If we are able to raise capital by issuing equity securities, 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 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, pursuant to which we have drawn-down $15.0 million, contains various covenants that limit our ability to engage in specified types of transactions. Those 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;

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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 prepayments 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 have no experience with acquiring other companies and limited experience with forming collaborations. 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

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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 are a development stage company. We commenced operations in February 2002. Our operations to date have been limited to organizing and staffing our company, acquiring and developing our technology and undertaking preclinical studies and clinical trials of our most advanced product candidates. We have not yet generated any commercial sales for any of our product candidates. We have not yet demonstrated our ability to 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.

The anticipated benefits of the Callidus acquisition may not be fully realized and may take longer to realize than expected.

The Callidus acquisition involved the integration of Callidus' operations, product candidates and technology with our existing operations and programs, and there are uncertainties inherent in such integration. We have devoted and will continue to devote significant management attention and resources to the Callidus integration and to the further development of Callidus' product candidate and other programs. Delays, unexpected difficulties in the integration process or failure to retain key management personnel could adversely affect our business, financial results and financial condition. Even if we were able to conduct the integration successfully, we may not realize the full achievement of the benefits of the Callidus acquisition within a reasonable period of time.

In addition, we may have not yet discovered during the due diligence process, all known and unknown factors regarding Callidus that could produce unintended and unexpected consequences for us. Undiscovered factors could cause us to incur potentially material financial liabilities, and prevent us from achieving the expected benefits from the acquisition within our desired time frames, if at all.

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 HCl, 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 HCl. 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 collaborations 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;

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successful completion of preclinical studies and clinical trials;

obtaining regulatory agreement in the structure and design of our clinical programs;

obtaining marketing approvals from the United States Food and Drug Administration (FDA) and similar regulatory authorities outside the U.S.;

establishing commercial-scale manufacturing arrangements with third party manufacturers whose manufacturing facilities are operated in compliance with current good manufacturing practice (cGMP) regulations;

launching commercial sales of the product, whether alone or in collaboration with others;

acceptance of the product by patients, the medical community and third party payors;

competition from other companies and their therapies;

successful protection of our intellectual property rights from competing products in the U.S. and abroad; and

a continued acceptable safety and efficacy profile of our product candidates following approval.

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

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

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

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

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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. For example, in December 2012, we announced top-line six-month (Stage 1) results from Study 011. While we believe these data are encouraging, the results did not achieve statistical significance (p=0.3) according to the pre-specified primary endpoint analysis. Although it is understood that FDA will consider the totality of the data from all clinical trials, including Study 011 and Study 012 to support a potential U.S. conditional approval of migalastat HCl monotherapy, there can be no assurance that such data will support such approval or that the FDA will interpret these data in the same way that we may, which could delay, limit or prevent regulatory approval. Similarly, there can be no assurance that the data from Study 011 and Study 012 will support regulatory approval in territories outside the U.S.

In addition, each of our product candidates is based on our pharmacological chaperone technology. To date, we are not aware that any product based on chaperone technology has been approved by the FDA. As a result, 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.

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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. Although we announced top-line six-month Stage 1 results for our Phase 3 study of migalastat HCl (Study 011) in December 2012, the results did not achieve statistical significance according to the primary endpoint analysis, and we have not yet completed a Phase 3 clinical trial for any of our product candidates. Our limited experience might prevent us from successfully designing or implementing a clinical trial. We have limited experience in conducting and managing the application process necessary to obtain regulatory approvals and we might not be able to demonstrate that our product candidates meet the appropriate standards for regulatory approval. If we are not successful in conducting and managing our preclinical development activities or clinical trials or obtaining regulatory approvals, we might not be able to commercialize our lead product candidates, or might be significantly delayed in doing so, which will materially harm our business.

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

Each of the diseases that our lead product candidates are intended to treat is 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 ongoing Phase 3 study in migalastat HCl for Fabry disease to support approval in the United States (Study 011) requires that patients must have a genetic mutation that we believe is responsive to migalastat HCl, 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;

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- 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 HCl 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

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requirements for potentially costly post-market surveillance, post-approval studies or clinical trials. For example, any labeling ultimately approved by the FDA for migalastat HCl, 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 HCl 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 current Good Manufacturing Practice, or 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 HCl 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 HCl or any of our other product candidates as part of a REMS plan. If we receive marketing approval for migalastat HCl 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.

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

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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 HCl, 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.

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

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

If we are unable to establish sales and marketing capabilities or enter into agreements with third parties to market and sell our product candidates, including migalastat HCl, 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;

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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.

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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 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

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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.

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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 HCl, 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 HCl under the Revised Agreement with GSK. If we were to obtain marketing approval for migalastat HCl 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;

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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 secure a new third party manufacturer for a key component of migalastat HCl co-formulated with human recombinant alpha-Gal enzyme.

Our current co-formulated product candidate for Fabry Disease that we developed as part of our collaboration with GSK utilizes migalastat HCl co-formulated with a proprietary human recombinant alpha-Gal enzyme produced by JCR, JR-051. JCR had provided JR-051 through a direct agreement with GSK. As a result of changes to our collaboration with GSK, we no longer have a long term supply agreement for the production of JR-051 for us. JCR has committed to supplying us with enough JR-051 to conduct our planned Phase 1/2 clinical trial of the co-formulated product candidate, but for future pre-clinical studies, clinical trials and, if approved, commercialization, we will need to secure a long term supply agreement or license from JCR or another new third party supplier for human recombinant alpha-Gal enzyme or develop our own internal cell line.

There are risks involved with securing a new third party supplier for human recombinant alpha-Gal enzyme. In addition to the risks described above with respect to securing and using third party manufacturers, there is also a risk that we will not be able to secure a new third party supplier for human recombinant alpha-Gal enzyme on terms acceptable to us and that the FDA will require us to conduct new preclinical studies or clinical trials of the new human recombinant alpha-Gal enzyme. If we are required to conduct such additional preclinical studies or clinical trials, 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

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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 HCl 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

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pharmaceutical or biotechnology companies, such as our collaboration with Biogen for our Parkinson's program. 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;

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- 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 HCl to treat Fabry disease expire in 2018 in the U.S. and 2019 in Europe, Japan, and Canada. Patents that we have licensed claiming afegostat expire between 2015 and 2016 in the U.S. and in 2015 in the UK, France, Sweden, Germany, Switzerland and Japan. In the U.S., we have several issued patents that were licensed from the Mt. Sinai School of Medicine covering afegostat's methods of use which expire in 2018. We own a U.S. patent and its corresponding foreign patents and patent applications covering afegostat tartrate (a specific salt form of afegostat) and its use to treat Gaucher disease, which expires in 2027. Other than the patents and patent applications covering afegostat tartrate and its use to treat Gaucher disease, we currently have no pending or issued patents covering methods of using afegostat to treat Gaucher disease outside of the U.S. other than the pending applications covering the use of afegostat in combination with ERT to treat Gaucher disease. Patents and patent applications that we own or have licensed relating to the use of AT2220 (duvoglustat HCl) expire in 2018 in the U.S. (not including the Hatch-Waxman statutory extension, which is described above). Further, we currently do not have composition of matter protection for AT2220 (duvoglustat HCl) in the U.S. or either composition of matter or method of use protection outside of the U.S. Where we lack patent protection outside of the U.S., we intend to seek orphan medicinal product designation and to rely on statutory data exclusivity provisions in jurisdictions outside the U.S. where such protections are available, including Europe. If we are unable to obtain such protection outside the U.S., our competitors may be free to use and sell afegostat and/or AT2220 (duvoglustat HCl) outside of the U.S. and there will be no liability for infringement or any other barrier to competition. The patent rights that we own or have licensed relating to our product candidates are limited in ways that may affect our ability to exclude third parties from competing against us if we obtain regulatory approval to market these product candidates. In particular:

- We do not hold composition of matter patents covering migalastat HCl and AT2220 (duvoglustat HCl). 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.

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For some of our product candidates, the principal patent protection that covers or those we expect will cover, our product candidate is a method of use patent. This type of patent only protects the product when used or sold for the specified method. However, this type of patent does not limit a competitor from making and marketing a product that is identical to our product that is labeled for an indication that is outside of the patented method, or for which there is a substantial use in commerce outside the patented method.

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

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

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

We are a party to a number of license agreements including agreements with the Mount Sinai School of Medicine of New York University, the University of Maryland, Baltimore County and Novo Nordisk A/S, pursuant to which we license key intellectual property relating to our lead product candidates. We expect to enter into additional licenses in the future. Under our existing licenses, we have the right to enforce the licensed patent rights. Our existing licenses impose, and we expect that future licenses will impose, various 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.

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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.

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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 HCl, 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;

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- 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 HCl for the treatment of Fabry disease in February 2004, for the active ingredient in afegostat for the treatment of Gaucher disease in January 2006 and for AT2220 for the treatment of Pompe disease in June 2007. We also obtained orphan medicinal product designation in the EU for migalastat HCl in May 2006 and for afegostat in October 2007. We anticipate filing for orphan drug designation in the EU for AT2220 for the treatment of Pompe disease. Generally, if a

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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 HCl and afegostat may be important to each of the product candidate's success. Even if we obtain orphan drug exclusivity for our products, we may not be able to maintain it. For example, if a competitive product that is the same drug as our product candidate is shown to be clinically superior to our product candidate, any orphan drug exclusivity we have obtained will not block the approval of such competitive product and we may effectively lose what had previously been orphan drug exclusivity.

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

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

restrictions on such products, manufacturers or manufacturing processes;

warning letters;

withdrawal of the products from the market;

refusal to approve pending applications or supplements to approved applications that we submit;

voluntary or mandatory recall;

fines;

suspension or withdrawal of regulatory approvals or refusal to approve pending applications or supplements to approved applications that we submit;

refusal to permit the import or export of our products;

product seizure or detentions;

injunctions or the imposition of civil or criminal penalties; and

adverse publicity.

If we, or our suppliers, third party contractors, clinical investigators or collaborators are slow to adapt, or are unable to adapt, to changes in existing regulatory requirements or adoption of new regulatory requirements or policies, we or our collaborators may lose marketing approval for our

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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 Chief Operating Officer, and William D. Baird, III, our Chief Financial 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.

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Risks Related to Our Common Stock

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

Our executive officers, directors and affiliated stockholders beneficially own shares representing approximately 49% of our common stock as of December 31, 2013. As a result, if these stockholders were to choose to act together, they would be able to control all matters submitted to our stockholders for approval, as well as our management and affairs. For example, these persons, if they choose to act together, will control the election of directors and approval of any merger, consolidation, sale of all or substantially all of our assets or other business combination or reorganization. This concentration of voting power could delay or prevent an acquisition of us on terms that other stockholders may desire. The interests of this group of stockholders may not always coincide with the interests of other stockholders, and they may act, whether by meeting or written consent of stockholders, in a manner that advances their best interests and not necessarily those of other stockholders, including obtaining a premium value for their common stock, and might affect the prevailing market price for our common stock.

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

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

establish a classified board of directors, and, as a result, not all directors are elected at one time;

allow the authorized number of our directors to be changed only by resolution of our board of directors;

limit the manner in which stockholders can remove directors from our board of directors;

establish advance notice requirements for stockholder proposals that can be acted on at stockholder meetings and nominations to our board of directors;

require that stockholder actions must be effected at a duly called stockholder meeting and prohibit actions by our stockholders by written consent;

limit who may call stockholder meetings;

authorize our board of directors to issue preferred stock, without stockholder approval, which could be used to institute a "poison pill" that would work to dilute the stock ownership of a potential hostile acquirer, effectively preventing acquisitions that have not been approved by our board of directors; and

require the approval of the holders of at least 67% of the outstanding voting stock to amend or repeal certain provisions of our charter or bylaws.

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

An active trading market for our common stock may not be sustained.

We completed our initial public offering of equity securities in June 2007, and prior to such offering, there was no public market for our common stock. Although we are listed on The NASDAQ Global Market, an active trading market for our common stock only recently developed and may not be sustained, especially given the large percentage of our common stock held by insiders and affiliated stockholders. If an active market for our common stock is not sustained, it may be difficult for our stockholders to sell shares without depressing the market price for our common stock.

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

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

results of clinical trials of our product candidates or those of our competitors;

our entry into or the loss of a significant collaboration;

regulatory or legal developments in the U.S. and other countries, including changes in the health care payment systems;

variations in our financial results or those of companies that are perceived to be similar to us;

changes in the structure of healthcare payment systems;

market conditions in the pharmaceutical and biotechnology sectors and issuance of new or changed securities analysts' reports or recommendations;

general economic, industry and market conditions;

results of clinical trials conducted by others on drugs that would compete with our product candidates;

developments or disputes concerning patents or other proprietary rights;

public concern over our product candidates or any products approved in the future;

litigation;

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 marked value of their investment.

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

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

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

None.

Item 2. ***PROPERTIES.***

We currently lease approximately 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 runs to 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 and we are actively looking to sub lease the property. The lease for the San Diego, California location runs until September 2016. 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.

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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
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

	High	Low
2012		
First Quarter	$ 6.88	$ 3.50
Second Quarter	5.76	4.34
Third Quarter	6.51	4.70
Fourth Quarter	6.47	2.53

The closing price for our common stock as reported by the NASDAQ Global Market on February 24, 2014 was $2.61 per share. As of February 24, 2014, there were 56 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

On November 20, 2013, we entered into a securities purchase agreement (the "2013 SPA") with the investors Glaxo Group Ltd (an affiliate of GSK) and certain entities controlled by Redmile Group, LLC for the private placement of (a) shares of our common stock, par value $0.01 per share (the "Common Stock") and (b) a combination of shares of Common stock (the "Shares") and warrants (the "Warrants") to purchase shares of Common Stock (collectively, the "Units"). Each of the investors was one of our shareholders prior to the consummation of the transactions. The Shares and Units sold to the investors were offered and 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 us. Pursuant to the 2013 SPA, we issued (a) 1.5 million Shares at $2.00 per Share to GSK and (b) 6 million Units 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 $12 million on November 21, 2013 from Redmile Group and $3 million from GSK on November 27, 2013, for a total proceeds amount of $15 million for general corporate and working capital purposes as a result of the

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private placement. The Company closed the private placement on November 20, 2013. The shares issued to GSK and Redmile Group entities have been registered for resale with the Securities and Exchange Commission (the "Commission") on a selling stockholder shelf Registration Statement on FormS-3, File No. 333-192876, which was declared effective by the Commission on December 20, 2013.

On November 18, 2013, we acquired all the capital stock of Callidus, a privately held biotechnology company in exchange for 7.2 million shares of our common stock. Callidus was engaged in developing a next-generation Pompe ERT and complimentary enzyme targeting technologies. In addition to the consideration paid upon closing of the acquisition, we will be obligated to make additional payments to the former stockholders of Callidus upon 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. The shares issued to Callidus' former stockholders have been registered for resale with the Commission on a selling shareholder shelf Registration Statement on Form S-3 File No. 333-192747, which was declared effective by the Commission on February 12, 2014.

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Performance Graph

The following performance graph shows the total shareholder return of an investment of $100 cash on May 31, 2007, the date our common stock first started trading on the NASDAQ Global Market, for (i) our common stock, (ii) the NASDAQ Composite Index (U.S.) and (iii) the NASDAQ Biotechnology Index as of December 31, 2013. 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.

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

	5/31/07	12/31/07	12/31/08	12/31/09	12/31/10	12/31/11	12/31/12	12/31/2013
Amicus Therapeutics, Inc.	100	74	55	28	33	24	19	16
NASDAQ Composite	100	102	61	87	102	100	116	160
NASDAQ Biotechnology	100	100	87	101	116	130	171	284

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, 2013.

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

	Year Ended December 31,					Period from February 4, 2002 (inception) to December 31, 2013
	2009	2010	2011	2012	2013	
Statement of Operations Data:						
Revenue:						
Research revenue	$ 17,545	$	$ 14,794	$ 11,591	$ 363	$ 57,856
Collaboration and milestone revenue	46,813	922	6,640	6,820		64,382
Total revenue	64,358	922	21,434	18,411	363	122,238
Operating expenses:						
Research and development	48,081	39,042	50,856	50,273	41,944	357,837
General and administrative	19,973	15,660	19,880	19,364	18,893	151,506
Restructuring charges	1,522				1,988	3,510
Impairment of leasehold improvements						1,030
Depreciation and amortization	2,132	2,058	1,585	1,705	1,719	13,487
In-process research and development						418
Total operating expenses	71,708	56,760	72,321	71,342	64,544	527,788
Loss from operations	(7,350)	(55,838)	(50,887)	(52,931)	(64,181)	(405,550)
Other income (expenses):						
Interest income	997	156	160	316	174	14,563
Interest expense	(278)	(260)	(148)	(89)	(46)	(2,468)
Change in fair value of warrant liability		(1,410)	2,764	653	908	2,461
Other income	64	1,277	70	21		252
Loss before tax benefit	(6,567)	(56,075)	(48,041)	(52,030)	(63,145)	(390,742)
Income tax benefit		1,139	3,629	3,245	3,512	12,220
Net loss	(6,567)	(54,936)	(44,412)	(48,785)	(59,633)	(378,522)
Deemed dividend						(19,424)
Preferred stock accretion						(802)
Net loss attributable to common stockholders	$ (6,567)	$ (54,936)	$ (44,412)	$ (48,785)	$ (59,633)	$ (398,748)
Net loss attributable to common stockholders per common share basic and diluted	$ (0.29)	$ (1.98)	$ (1.28)	$ (1.07)	$ (1.16)	

Weighted-average common shares outstanding basic and diluted	22,624,134	27,734,797	34,569,642	45,565,217	51,286,059	

	As of December 31,				
	2009	**2010**	**2011**	**2012**	**2013**
Balance Sheet Data:					
Cash and cash equivalents and marketable securities	$ 78,224	$ 107,445	$ 55,702	$ 99,122	$ 82,000
Working capital	69,293	93,458	47,392	95,374	77,817
Total assets	85,370	112,552	69,795	110,088	127,563
Total liabilities	13,537	47,618	40,203	40,868	81,812
Deficit accumulated during the development stage	(170,756)	(225,692)	(270,104)	(318,889)	(378,522)
Total stockholders' equity	$ 71,833	$ 64,934	$ 29,592	$ 69,220	$ 45,751

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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 development programs include next-generation enzyme replacement therapies (ERTs) for LSDs, including Fabry disease, Pompe disease and Mucopolysaccharoidosis Type I (MPS I). We are also developing novel small molecules as monotherapy treatments for Fabry disease and Parkinson's disease. 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

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

Study 011 is a 24-month study consisting of a 6-month double-blind, placebo-controlled treatment period (Stage 1); a 6-month open-label follow-up period (Stage 2); and a 12-month open-label extension phase. The study randomized 67 patients (24 males and 43 females) diagnosed with Fabry disease that had genetic mutations amenable to chaperone monotherapy in a cell-based assay. During Stage 1 patients were randomized 1:1 to migalastat HCl 150 mg or placebo on an every-other-day (QOD) oral dosing schedule. Patients continued treatment with migalastat HCl or switched from placebo to migalastat HCl during Stage 2 and the open-label extension phase. Change from baseline in kidney interstitial capillary globotriaosylceramide (GL-3) is being assessed by histology in kidney biopsies at the end of Stage 1 and Stage 2. GL-3 is the lipid substrate that accumulates in tissues of patients with Fabry disease, and is measured in kidney biopsies. Secondary endpoints for Study 011 include safety and tolerability, urine GL-3 and kidney function as measured by estimated glomerular filtration rate (eGFR), are being assessed throughout the 24-month study.

Top-line Stage 1 data from Study 011 was reported in December 2012 and presented at the Lysosomal Disease Network WORLD Symposium (LDN WORLD) in February 2013. During Stage 1, no drug-related serious adverse events were observed. No subjects discontinued migalastat HCl therapy due to a treatment emergent adverse event and the majority of adverse events in both treatment groups were mild in nature. The primary analysis compared the number of responders in the migalastat HCl versus placebo groups, based on a 50% or greater reduction in interstitial capillary GL-3 during Stage 1. In the primary responder analysis, 13/32 (41%) in the migalastat HCl group versus 9/32 (28%) in the placebo group demonstrated a 50% or greater reduction in kidney interstitial capillary GL-3 from baseline to month 6 which was not statistically significant (p=0.3). Certain 6-month secondary endpoints presented included urine GL-3 and renal function as measured by eGFR.

Updated Stage 1 data from Study 011, including a post-hoc analysis of the mean change from baseline in inclusions per capillary as a continuous variable ("mean change in GL-3"), were presented at LDN WORLD in February 2014. Following the unblinding of the Stage 1 data, and while still blinded to the Stage 2 data, we identified the mean change in GL-3 as a more appropriate way to control for the variability in GL-3 levels in Study 011, as well as to measure the biological effect of migalastat HCl. The mean change in GL-3 from baseline to month 6 was analyzed in both the modified intent-to-treat (mITT) population (n=60, 30 per group) as well as in a subgroup of patients who had amenable mutations in a GLP-validated HEK assay ("GLP HEK amenable") (n=46, 25 in the migalastat HCl group and 21 in the placebo group). From baseline to month 6 in the mITT population, the mean change in GL-3 was -0.22 ± 0.11 in the migalastat group compared to +0.06 ± 0.09 in the placebo group (p=0.052). From baseline to month 6 in the GLP HEK amenable subgroup, the mean

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change in GL-3 was -0.31 ± 0.12 in the migalastat group compared to +0.10 ± 0.13 in the placebo group (p=0.002).

The Stage 2 treatment period in Study 011 was completed in December 2012 and the 12-month extension phase was completed in December 2013. The Stage 2 results, including mean change in GL-3 at 12 months, as well as complete data from the 24-month study, including clinical outcome measures such as eGFR and proteinuria, are expected during the second quarter of 2014.

Study 012 is our second Phase 3 study intended to support the worldwide registration of migalastat HCl for Fabry disease. Study 012 is a randomized, open-label 18-month Phase 3 study investigating the safety and efficacy of oral migalastat HCl (150 mg, every-other-day) compared to standard-of-care infused ERTs (Fabrazyme® and Replagal®). The study enrolled a total of 60 patients (males and females) with Fabry disease and genetic mutations identified as amenable to migalastat HCl monotherapy in a cell-based assay. Subjects were randomized 1.5:1 to switch to migalastat HCl 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. The primary outcome measure is renal function assessed by Glomerular Filtration Rate (GFR) at 18 months, evaluated in the migalastat HCl and ERT groups using descriptive statistics. This study achieved full enrollment in December 2012 and top-line results are expected in the second half of 2014.

We expect to receive final data from Study 011 and Study 012 in 2014. Based on the outcomes of those studies, we will initiate discussions with U.S. and EU regulatory authorities to discuss a potential regulatory path to approval.

Migalastat HCl Combination Programs for Fabry Disease

We completed an open-label Phase 2 drug-drug interaction study (Study 013) in 23 males with Fabry disease to evaluate the safety and pharmacokinetic (PK) effects of two doses of migalastat HCl (150 mg and 450 mg) co-administered with currently marketed ERTs infused α -Gal A enzymes, Fabrazyme® (agalsidase beta) and Replagal® (agalsidase alfa). Preliminary results from Study 013 showed increased levels of active alpha-Gal enzyme levels in plasma and increased alpha-Gal enzyme in skin following co-administration compared to ERT alone. We and GSK, in collaboration with JCR, completed preclinical studies to evaluate migalastat HCl co-formulated with JCR's proprietary investigational ERT (JR-051, recombinant human alpha-Gal enzyme). Based on these results, we plan to advance migalastat HCl co-formulated with ERT for Fabry disease. The first planned clinical study will investigate the PK of IV migalastat HCl in healthy volunteers to identify optimal doses for a Phase 1/2 clinical study of migalastat HCl co-formulated with ERT in Fabry patients. For the Phase 1/2 study, we expect to use migalastat HCl co-formulated with JR-051. In parallel, we are working to develop a more optimal cell-line of recombinant human alpha-Gal enzyme for co-formulation with migalastat HCl in subsequent clinical studies.

Next-Generation ERT for Pompe Disease

We are utilizing our CHART platform in combination with our uniquely-engineered, proprietary recombinant human acid-alpha glucosidase (rhGAA, designated AT-B200) to develop a next-generation ERT for Pompe disease. We are currently investigating AT-B200, with and without a pharmacological chaperone, in preclinical studies.

We acquired AT-B200 as well as our enzyme targeting technology through our purchase of Callidus Biopharma. AT-B200 is differentiated from other Pompe ERTs by its unique carbohydrate structure, and may be further optimized by applying our proprietary peptide tagging technology for better targeting. AT-B200 may also deliver further benefits through co-formulation with our pharmacological chaperone AT2220 (duvoglustat HCl).

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We completed a Phase 2 safety and Pharmacokinetics study (Study 010) that investigated single ascending oral doses of AT2220 (50 mg, 100 mg, 250 mg, and 600 mg) co-administered with Myozyme® or Lumizyme® (alglucosidase alfa. or recombinant human GAA enzyme, rhGAA), in patients with Pompe disease. Each patient received one infusion of ERT alone, and then a single dose of AT2220 just prior to the next ERT infusion. Results from this study showed an increase in GAA enzyme activity in plasma and muscle compared to ERT alone.

In preclinical studies, AT-B200 was shown to have superior uptake and activity in disease-relevant tissues that correlated with clearance of accumulated glycogen substrate when compared to current standard of care. AT-B200 may be further improved through the application of the Company's proprietary conjugation technology to attach vIGF2 (a variant of the insulin growth factor 2 receptor) to further enhance drug targeting. The vIGF2 peptide binds the intended IGF2 receptor, but does not bind to insulin or the IGF1 receptor. Preclinical results have shown that AT-B200 and AT-B200 conjugated with vIGF-2 were better than Lumizyme for clearing glycogen in skeletal muscles in Gaa knock-out mice.

The results from preclinical studies of AT-B200 taken together with data from our clinical and preclinical studies of AT2220 in combination with ERT support our further development of a next-generation ERT for Pompe disease.

Collaboration with GSK

In November 2013, we entered into the Revised Agreement (the "Revised Agreement") with GSK, pursuant to which we have obtained global rights to develop and commercialize migalastat HCl as a monotherapy and in combination with enzyme replacement therapy ("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 is no upfront payment from Amicus to GSK. For the next-generation Fabry ERT (migalastat HCl co-formulated with ERT), GSK is eligible to receive single-digit royalties on net sales in eight major markets outside the U.S. For migalastat HCl 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. Under the Revised Agreement, Amicus is entitled to payments of (1) a settlement fee of $1.9 million to reimburse development costs between November 19, 2013 and December 31, 2013, and (2) reimbursement of development costs for the period until November 19, 2013 according to the earlier Expanded Agreement.

In November 2013, we 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) shares of our common stock and b) a combination of shares of our common stock and warrants to purchase shares of our 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 and GSK's resulting equity stake in the Company was 17.6% at December 31, 2013.

Collaboration with Biogen

In September 2013, we entered into a collaboration agreement with Biogen to discover, develop and commercialize novel small molecules for the treatment of Parkinson's disease. The collaboration will build upon preclinical studies at the Company and independent published research that suggest increasing activity of the lysosomal enzyme glucocerobrosidase ("GCase") enzyme in the brain may correct alpha-synuclein pathology and other deficits associated with Parkinson's disease. Under terms of the multi-year agreement, the Company and Biogen 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 will be responsible for funding all discovery, development, and commercialization

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activities. In addition the Company will be reimbursed for all full-time employees working on the project. The Company is also eligible to receive development and regulatory milestones, as well as modest royalties on global net sales.

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 complimentary 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.

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 from a development stage company to a commercial biotechnology company.

Financial Operations Overview

Revenue

Biogen

In September 2013, we entered into a collaboration with Biogen to discover, develop and commercialize novel small molecules for the treatment of Parkinson's disease. For the three and twelve months ended December 31, 2013, we recognized $0.4 million as Research Revenue for reimbursed research and development costs.

GSK

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.

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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. Starting July 2012, any payments received from GSK are recorded as deferred reimbursements on the balance sheet. In addition, future milestone payments we may pay GSK will be applied against the balance of this deferred reimbursements account. Revenue recognition would resume once the total arrangement consideration becomes fixed and determinable which would occur when the balance of the deferred reimbursements account is sufficient to cover all the remaining contingent milestone payments due to GSK. As a result, we no longer recognize any revenue related to Collaboration and Milestone Revenue or Research Revenue as of the date of the Expanded Collaboration Agreement. There is 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.

In November 2013, we entered into a Revised Agreement with GSK, which amended and replaced in its entirety the Expanded Collaboration Agreement. Although there were changes to the terms of the agreement, for accounting purposes, it remains substantively the same. As such the accounting policy determined for the Expanded Agreement continued to be applied in the Revised Agreement for both the research and development reimbursements and the contingent milestone payments. Similar to our evaluations under the Expanded Agreement, any payments received from GSK are recorded as deferred reimbursements on the balance sheet and any future contingent payments to GSK under the Revised Agreement would be recorded against the deferred reimbursement account. GSK will no longer jointly fund development costs for all formulations of migalastat HCl as a result of the Revised Agreement.

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

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candidates. From our inception in February 2002 through December 31, 2013, we have incurred research and development expense in the aggregate of $357.8 million.

The following table summarizes our principal product development projects through December 31, 2013, including the related stages of development for each project, and the out-of-pocket, third party expenses incurred with respect to each project (in thousands):

	Years Ended December 31,			Period from February 4, 2002 (Inception) to December 31, 2013
	2011	2012	2013	
Projects				
Third party direct project expenses				
Mono-therapy Studies				
Migalastat HCl (Fabry Disease Phase 3)	$ 19,305	$ 14,718	$ 8,977	$ 89,030
Afegostat tartrate (Gaucher Disease Phase 2*)	(112)	186	80	26,381
AT2220 (Pompe Disease Phase 2)	109	9		13,252
Combination Studies				
Migalastat HCl Co-Administration (Fabry Disease Phase 2)	1,083	2,235	534	3,964
Migalastat HCl Co-Formulation (Fabry Disease Preclinical)		454	89	543
Afegostat tartrate Co-Administration (Gaucher Disease Preclinical)	7		21	55
AT2220 Co-Administration (Pompe Disease Phase 2)	1,647	2,367	3,403	7,531
AT2220 Co-Formulation (Pompe Disease Preclinical)			345	345
Neurodegenerative Diseases (Preclinical)	2,210	417	144	9,170
Total third party direct project expenses	24,249	20,386	13,593	150,271
Other project costs [1]				
Personnel costs	18,814	21,086	20,257	134,594
Other costs [2]	7,793	8,801	8,094	72,972
Total other project costs	26,607	29,887	28,351	207,566
Total research and development costs	$ 50,856	$ 50,273	$ 41,944	$ 357,837

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

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

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

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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 HCl 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. From our inception in February 2002 through December 31, 2013, we spent $151.5 million on general and administrative expense.

Interest Income and Interest Expense

Interest income consists of interest earned on our cash and cash equivalents and marketable securities. Interest expense consists of interest incurred on our debt agreements.

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

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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

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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.

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.

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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.

Stock-Based Compensation

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.

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The following table summarizes the stock compensation expense recognized in the income statement (in thousands):

	Years Ended December 31,		
	2011	2012	2013
Stock compensation expense recognized in:			
Research and development expense	$ 2,927	$ 3,603	$ 3,583
General and administrative expense	5,751	2,588	2,594
Total stock compensation expense	$ 8,678	$ 6,191	$ 6,177

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 (Shire and GSK collaborations, Callidus acquisition), 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,		
	2011	2012	2013
Expected stock price volatility	78.8%	77.2%	82.0%
Risk free interest rate	2.0%	0.8%	1.3%
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 2011, 2012 and 2013 were $4.11, $3.31 and $2.14, respectively.

Warrants

The warrants issued in connection with our 2013 SPA are classified as equity. As part of the 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.

The warrants issued in connection with our March 2010 registered direct offering are classified as a liability. The fair value of the warrant liability is evaluated at each balance sheet date using the Black-Scholes valuation model. This model uses inputs such as the underlying price of the shares issued when the warrant is exercised, volatility, risk free interest rate and expected life of the instrument. Any changes in the fair value of the warrants liability is recognized in the consolidated statement of operations.

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The weighted average assumptions used in the Black-Scholes valuation model for the warrants for December 31, 2012 and 2013 are as follows:

	December 31,	
	2012	2013
Expected stock price volatility	93.2%	45.9%
Risk free interest rate	0.17%	0.07%
Expected life of warrants (years)	1.17	0.17
Expected annual dividend per share	$ 0.00	$ 0.00

During 2013, no warrants were exercised and for the year ended December 31, 2013, we recorded a gain of $0.9 million due to the change in the fair value of the warrant liability. The resulting fair value of the warrant liability was de minimis at December 31, 2013.

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

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

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

		Years Ended December 31,		
		2011	2012	2013
Historical				
Numerator:				
Net loss attributable to common stockholders		$ (44,412)	$ (48,785)	$ (59,633)
Denominator:				
Weighted average common shares outstanding	basic and diluted	34,569,642	45,565,217	51,286,059

Dilutive common stock equivalents would include the dilutive effect of common stock options and warrants for common stock equivalents. Potentially dilutive common stock equivalents totaled approximately 8.5 million, 9.4 million and 12.0 million for the years ended December 31, 2011, 2012 and 2013, respectively. Potentially dilutive common stock equivalents were excluded from the diluted earnings per share denominator for all periods because of their anti-dilutive effect.

Results of Operations

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

Revenue. In September 2013, we entered into collaboration with Biogen to discover, develop and commercialize novel small molecules for the treatment of Parkinson's disease. For the year ended December 31, 2013, we recognized $0.4 million as Research Revenue for reimbursed research and development costs.

For the year ended December 31, 2012, we recognized $6.8 million, as Collaboration and Milestone Revenue from GSK which includes a $3.5 million payment for a clinical development

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milestone in 2012. The reimbursements for research and development costs under the Original License and Collaboration Agreement with GSK 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.

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 until entry into the Expanded Collaboration Agreement in July 2012 and the Revised Agreement in November 2013. Due to a change in the accounting for revenue recognition for the Expanded Collaboration Agreement, which continued into the Revised Agreement, all revenue recognition related to the collaboration will be suspended until the total arrangement consideration becomes fixed and determinable. Any payments received from GSK will be recorded as deferred reimbursements on the balance sheet. In addition, future milestone payments we may pay GSK will be applied against the balance of this deferred reimbursements account. Revenue recognition would resume once the total arrangement consideration becomes fixed and determinable which would occur when the balance of the deferred reimbursements account is sufficient to cover all the remaining contingent milestone payments. 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 is no cash effect of this change in accounting, and there is no scenario where we would have to refund any of the upfront payment, milestone payments, or research reimbursement payments. The new rights obtained from GSK under the Revised Agreement; do not represent a separate, identifiable benefit from the licenses in the Original and Expanded Agreement. Therefore, there is no change in accounting due to the Revised Agreement. We have not generated any commercial sales revenue since our inception.

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 HCl study and the increase was from $1.0 million in the Pompe AT2220 co-ad 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 and Interest Expense. 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

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lower average cash and investment balances in 2013, compared to 2012. 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. The market price for our common stock has been and may continue to be volatile. Consequently, future fluctuations in the price of our common stock may cause significant increases or decreases in the fair value of the warrants liability.

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. Should the State of New Jersey continue to fund this program, which is uncertain, the future amount of net operating loss and research and development credit carry forwards which we may sell will depend upon the allocation among qualifying companies of an annual pool established by the State of New Jersey.

Net Operating Loss Carry forwards. As of December 31, 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.

Year Ended December 31, 2012 Compared to Year Ended December 31, 2011

Revenue. For the years ended December 31, 2012 and 2011, we recognized $6.8 million and $6.6 million, respectively, as Collaboration and Milestone Revenue which includes a $3.5 million payment for a clinical development milestone in 2012. 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 years ended December 31, 2012 and 2011, we recognized $11.6 million and $14.8 million, respectively, as Research Revenue.

Research and Development Expense. Research and development expense was $50.3 million in 2012 representing a decrease of $0.6 million or 1.2% from $50.9 million in 2011. The variance was primarily attributable to a $2.5 million decrease in contract manufacturing and a $1.5 million decrease in contract

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research costs, partially offset by increases in personnel costs of $2.3 million, license fees of $0.5 million and $0.7 million in consulting fees.

General and Administrative Expense. General and administrative expense was $19.4 million in 2012, a decrease of $0.5 million or 2.5% from $19.9 million in 2011. The variance was primarily due to a decrease in personnel cost of $1.9 million which was related to two events in 2011: (1) additional stock option compensation expense recognized as a result of the change in the terms of the Chief Executive Officer's stock options resulting from his resignation and subsequent reappointment to the Chief Executive Officer position; and (2) a severance related compensation charge of $0.6 million related to the resignation of the former President and the vesting of his restricted stock award. In addition, there were decreases in accounting services and recruitment fees for $0.4 million. These decreases were partially offset by increases of $0.8 million in legal fees, $0.2 million in facility costs and $0.2 million in consulting fees.

Depreciation and Amortization. Depreciation and amortization expense was $1.7 million in 2012, an increase of $0.1 million or 7.6% from $1.6 million in 2011. The increase in depreciation was due to assets purchased in 2012 in connection with the new office and laboratory space in Cranbury, New Jersey.

Interest Income and Interest Expense. Interest income was $0.3 million in 2012, an increase of $0.1 million or50% from $0.2 million in 2011. The increase in interest income was due to the overall higher average cash and investment balances, due to cash raised in our March 2012 stock the public offering. Interest expense was $0.1 million for both 2012 and 2011.

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. For the year ended December 31, 2012, we reported a gain of $0.7 million related to the decrease in fair value of the warrant liability from the year ended December 31, 2011. The market price for our common stock has been and may continue to be volatile. Consequently, future fluctuations in the price of our common stock may cause significant increases or decreases in the fair value of the warrants liability.

Other Income/Expense. Other income for the year ended December 31, 2012 was $21 thousand and represents cash received from the sale of property, plant and equipment. Other income for the year ended December 31, 2011 was $70 thousand under the Qualified Therapeutic Discovery Projects tax credit and grant program.

Tax Benefit. During 2011 and 2012, 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 $3.6 million and $3.2 million in income tax benefits for the years ended December 31, 2011 and 2012, respectively. Should the State of New Jersey continue to fund this program, which is uncertain, the future amount of net operating loss and research and development credit carry forwards which we may sell will depend upon the allocation among qualifying companies of an annual pool established by the State of New Jersey.

Net Operating Loss Carry forwards. As of December 31, 2012, we had federal and state net operating loss carry forwards, or NOLs, of approximately $154 million and $162 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

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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 2012, 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

As a result of our significant research and development expenditures and the lack of any approved products to generate product sales revenue, we have not been profitable and have generated operating losses since we were incorporated in 2002. We have funded our operations principally with $148.7 million of proceeds from redeemable convertible preferred stock offerings, $75.0 million of gross proceeds from our IPO in June 2007, $18.5 million of gross proceeds from our Registered Direct Offering in March 2010, $65.6 million of gross proceeds from our stock offering in March 2012, $52.9 million from GSK's investments in the Company in October 2010 , July 2012, November 2013 and $80.0 million from non-refundable license fees from collaborations.

The following table summarizes our significant funding sources as of December 31, 2013:

Funding [2]	Year	No. Shares	Approximate Amount [1] (in thousands)
Series A Redeemable Convertible Preferred Stock	2002	444,443	$ 2,500
Series B Redeemable Convertible Preferred Stock	2004, 2005, 2006, 2007	4,917,853	31,189
Series C Redeemable Convertible Preferred Stock	2005, 2006	5,820,020	54,999
Series D Redeemable Convertible Preferred Stock	2006, 2007	4,930,405	60,000
Common Stock	2007	5,000,000	75,000
Upfront License Fee from Shire	2007		50,000
Registered Direct Offering	2010	4,946,524	18,500
Upfront License Fee from GSK	2010		30,000
Common Stock GSK	2010	6,866,245	31,285
Common Stock	2012	11,500,000	65,550
Common Stock GSK	2012	2,949,581	18,582
Common Stock Private Investment in Public Equity	2013	7,500,000	15,000
		54,875,071	$ 452,605

(1) Represents gross proceeds

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

In addition, in conjunction with the GSK collaboration agreement, we received reimbursement of research and development expenditures from the date of the agreement (October 28, 2010) through December 31, 2013 of $32.3 million. We also received $31.1 million in reimbursement of research and development expenditures from the Shire collaboration from the date of the agreement (November 7, 2007) through year end 2009.

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As of December 31, 2013, we had cash and cash equivalents and marketable securities of $82.0 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, 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 GSK related to the collaboration agreement of $2.5 million; an increase of $3.2 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 operations for the year ended December 31, 2012 was $33.7 million due primarily to the net loss for the year ended December 31, 2012 of $48.8 million and the change in operating assets and liabilities of $7.8 million. The change in operating assets and liabilities consisted of an decrease in receivables from GSK related to the collaboration agreement of $1.8 million; a decrease of $3.6 million in prepaid assets primarily related to a receivable from the 2011 sale of state net operating loss carry forwards, or NOLs; an increase in deferred reimbursements of $2.9 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 $0.9 million related to program expenses.

Net Cash Provided by and Used in Investing Activities

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 used in investing activities for the year ended December 31, 2012 was $39.4 million. Net cash used in investing activities reflects $118.5 million for the purchase of marketable securities and $4.3 million for the acquisition of property and equipment, partially offset by $83.3 million for the sale and redemption of marketable securities.

Net Cash Used in and Provided by Financing Activities

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.

Net cash provided by financing activities for the year ended December 31, 2012 was $81.5 million and reflects $80.2 million in proceeds from the issuance of common stock, $1.6 million of proceeds from exercise of stock options, $1.0 million in proceeds from secured loan arrangement, partially offset by the $1.3 million in payments of our secured loan agreement.

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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 HCl;
- the cost of manufacturing drug supply for our clinical and preclinical studies, including the significant cost of ERT cell line development and manufacturing as well as the cost of manufacturing the vIGF-2 peptide tag;
- 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 diseases of neurodegeneration;
- the costs, timing and outcome of regulatory review of our product candidates;
- the number and development requirements of other product candidates that we pursue;
- the costs of commercialization activities, including product marketing, sales and distribution;
- the emergence of competing technologies and other adverse market developments;
- the costs of preparing, filing and prosecuting patent applications and maintaining, enforcing and defending intellectual property related claims;
- the extent to which we acquire or invest in businesses, products or technologies;
- our ability to successfully incorporate Callidus and its product candidates and technology into our business; 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 of our current product pipeline until at least 2015, 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 believe that our existing cash and cash equivalents and short-term investments will be sufficient to cover our cash flow requirements into the second half of 2015.

Financial Uncertainties Related to Potential Future Payments

Milestone Payments

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

that would cause such payments, if any, to vary in size.

Under the Revised Collaboration Agreement, GSK is eligible to receive marketing approval milestones totaling $17.6 million for migalastat HCl monotherapy and migalastat HCl-ERT co-administration products, and additional sales performance milestone payments totaling up to $22 million for migalastat HCl monotherapy and migalastat HCl-ERT co-administration products. We

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will also be responsible for certain filing and approval milestone payments and single-digit royalties on the Co-formulated Product that GSK must pay to a third party. In addition, we are no longer eligible to receive any milestones or royalties we would have been eligible to receive under the Original Collaboration Agreement.

In connection with our acquisition of Callidus, under the Merger Agreement, we 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.

Royalties

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

In accordance with our license agreement with MSSM, in the third quarter of 2012, we paid $0.4 million of the $3.5 million milestone payment received from GSK to MSSM. In the fourth quarter of 2010, we paid $3 million of the $30 million upfront payment received from GSK to MSSM. We will also be obligated to pay MSSM royalties on worldwide net sales of migalastat HCl.

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

Contractual Obligations

The following table summarizes our significant contractual obligations and commercial commitments at December 31, 2013 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	$ 9,846	$ 1,930	$ 4,072	$ 3,547	$ 297
Debt obligations	15,749	299	9,479	5,971	
Total fixed contractual obligations [1]	$ 25,595	$ 2,229	$ 13,551	$ 9,518	$ 297

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

We 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.

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In August 2011, we entered into a loan and security agreement (the "2011 Loan Agreement") with Silicon Valley Bank ("SVB") in order to finance certain capital expenditures we made in connection with our move in March 2012 to our new office and laboratory space in Cranbury, New Jersey. The 2011 Loan Agreement provided for up to $3 million of equipment financing through January 2014. Borrowings under the 2011 Loan Agreement were collateralized by equipment purchased with the proceeds of the loan and bear interest at a variable rate of SVB prime + 2.5%. The current SVB prime rate is 4.0%. In February 2012, the Company borrowed approximately $1.0 million from the 2011 Loan Agreement which will 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.

In June 2011, we entered into a new employment agreement with our chairman and chief executive officer, John F. Crowley, that 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 $1.8 million for out-of-pocket medical expenses and the corresponding tax gross-up payments. We entered into the employment agreement upon Mr. Crowley's return to the chief executive officer position following a brief term as executive chairman of the Company from April 2011 through August 2011 during which time he did not serve as chief executive officer. The terms of this current employment agreement are substantially similar to Mr. Crowley's prior employment agreement pursuant to which he served as chief executive officer. Notably, Mr. Crowley's base salary, bonus, severance and benefits under the current employment agreement are the same as provided under the previous agreement The agreement will continue for successive one-year terms until either party provides written notice of termination to the other in accordance with the terms of the agreement.

In December 2013, we entered into a credit and security agreement (the "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 may draw up to an additional $10 million through the end of the fourth quarter of 2014 (the "Second Tranche"). The principal outstanding balance of the First Tranche bears interest at a rate per annum fixed at 8.5%. If the Company draws from the Second Tranche, the principal outstanding balance of the Second Tranche will also have a fixed interest rate, which will be determined by reference to the applicable index rate at the time of the draw. The Company will make interest-only payments on the Term Loan beginning January 1, 2014 and continuing 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. We also recorded payments made and a contingent payable to the lenders at December 31, 2013. 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 MMA 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.

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 HCl. 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.

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Off-Balance Sheet Arrangements

We had no off-balance sheet arrangements as of December 31, 2012 and 2013.

Recent Accounting Pronouncements

In July 2013, the Financial Accounting Standards Board ("FASB") issued an update that clarified existing guidance on the presentation of unrecognized tax benefits when various qualifying tax benefit carryforwards exist, including when the unrecognized tax benefit should be presented as a reduction to deferred tax assets or as a liability. This update is required to be adopted for all annual periods and interim reporting periods beginning after December 15, 2013, with early adoption permitted. The Company is evaluating the impact of this new provision on the consolidated results of operations or financial position.

In February 2013, the FASB amended its guidance to require an entity to present the effect of certain significant reclassifications out of accumulated other comprehensive income on the respective line items in net income. The new accounting guidance does not change the items that must be reported in other comprehensive income or when an item of other comprehensive income must be reclassified to net income. The guidance is effective prospectively for fiscal years beginning after December 15, 2012 and we will be required to adopt these new provisions no later than the quarter beginning January 1, 2013. As the guidance requires additional presentation only, there will be no impact to our consolidated results of operations or financial position.

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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, 2013, we held $82.0 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, 2013, 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.

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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, 2013. In making this assessment, we used the criteria set forth by the Committee of Sponsoring Organizations of the Treadway Commission (1992 framework) (COSO) in Internal Control Integrated Framework. Based on our assessment we believe that, as of December 31, 2013, 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, 2013 has been audited by Ernst & Young LLP, an independent registered public accounting firm, as stated in their report. This report appears on page 85.

Dated March 3, 2014

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

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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, 2013, based on criteria established in Internal Control Integrated Framework issued by the Committee of Sponsoring Organizations of the Treadway Commission (1992 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, 2013, 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. (a development stage company) as of December 31, 2013 and 2012, and the related consolidated statements of operations, comprehensive loss, changes in stockholders' (deficiency) equity and cash flows for each of the three years in the period ended December 31, 2013 and the period from February 4, 2002 (inception) to December 31, 2013 of Amicus Therapeutics, Inc., and our reported dated March 3, 2014 expressed an unqualified opinion thereon.

/s/ Ernst & Young LLP

MetroPark, New Jersey
March 3, 2014

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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. (a development stage company) as of December 31, 2013 and 2012, and the related consolidated statements of operations, comprehensive loss, changes in stockholders' (deficiency) equity and cash flows for each of the three years in the period ended December 31, 2013 and the period from February 4, 2002 (inception) to December 31, 2013. 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 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, 2013 and 2012, and the consolidated results of its operations and its cash flows for each of the three years in the period ended December 31, 2013 and the period from February 4, 2002 (inception) to December 31, 2013, 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, 2013, based on criteria established in Internal Control Integrated Framework issued by the Committee of Sponsoring Organizations of the Treadway Commission (1992 framework) and our report dated March 3, 2014 expressed an unqualified opinion thereon.

/s/ ERNST & YOUNG LLP

MetroPark, New Jersey
March 3, 2014

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Amicus Therapeutics, Inc.
(a development stage company)

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

	December 31,	
	2012	**2013**
Assets:		
Current assets:		
Cash and cash equivalents	$ 33,971	$ 43,640
Investments in marketable securities	65,151	38,360
Receivable due from GSK	3,225	759
Prepaid expenses and other current assets	2,270	5,519
Total current assets	104,617	88,278
Property and equipment, less accumulated depreciation and amortization of $8,501 and $9,973 at December 31, 2012 and 2013, respectively	5,029	4,120
In-process research & development		23,000
Goodwill		11,613
Other non-current assets	442	552
Total Assets	$ 110,088	$ 127,563
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable and accrued expenses	$ 8,845	$ 10,162
Current portion of secured loan	398	299
Total current liabilities	9,243	10,461
Deferred reimbursements	30,418	36,677
Warrant liability	908	
Secured loan, less current portion	299	14,174
Contingent consideration payable		10,600
Deferred tax liability		9,186
Other non-current liability		714
Commitments and contingencies		
Stockholders' equity:		
Common stock, $.01 par value, 125,000,000 shares authorized, 49,631,672 shares issued and outstanding at December 31, 2012, 61,975,416 shares issued and outstanding at December 31, 2013	556	679
Additional paid-in capital	387,539	423,593
Accumulated other comprehensive income	14	1
Deficit accumulated during the development stage	(318,889)	(378,522)
Total stockholders' equity	69,220	45,751
Total Liabilities and Stockholders' Equity	$ 110,088	$ 127,563

See accompanying notes to consolidated financial statements

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Amicus Therapeutics, Inc.
(a development stage company)

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

	Years Ended December 31,			Period from February 4, 2002 (Inception) to December 31, 2013
	2011	2012	2013	
Revenue:				
Research revenue	$ 14,794	$ 11,591	$ 363	$ 57,856
Collaboration and milestone revenue	6,640	6,820		64,382
Total revenue	21,434	18,411	363	122,238
Operating Expenses:				
Research and development	50,856	50,273	41,944	357,837
General and administrative	19,880	19,364	18,893	151,506
Restructuring charges			1,988	3,510
Impairment of leasehold improvements				1,030
Depreciation and amortization	1,585	1,705	1,719	13,487
In-process research and development				418
Total operating expenses	72,321	71,342	64,544	527,788
Loss from operations	(50,887)	(52,931)	(64,181)	(405,550)
Other income (expenses):				
Interest income	160	316	174	14,563
Interest expense	(148)	(89)	(46)	(2,468)
Change in fair value of warrant liability	2,764	653	908	2,461
Other income	70	21		252
Loss before income tax benefit	(48,041)	(52,030)	(63,145)	(390,742)
Income tax benefit	3,629	3,245	3,512	12,220
Net loss	(44,412)	(48,785)	(59,633)	(378,522)
Deemed dividend				(19,424)
Preferred stock accretion				(802)
Net loss attributable to common stockholders	$ (44,412)	$ (48,785)	$ (59,633)	$ (398,748)
	$ (1.28)	$ (1.07)	$ (1.16)	

Net loss attributable to common stockholders per common share basic and diluted				
Weighted-average common shares outstanding	basic and diluted	34,569,642	45,565,217	51,286,059

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Amicus Therapeutics, Inc.
(a development stage company)

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

	Years Ended December 31,			Period from February 4, 2002 (inception) to December 31,
	2011	2012	2013	2013
Net loss	$ (44,412)	$ (48,785)	$ (59,633)	$ (378,522)
Other comprehensive income/(loss):				
Unrealized gain/(loss) on available-for-sale securities	32	10	(13)	1
Other comprehensive (loss)/income before income taxes	32	10	(13)	1
Provision for income taxes related to other comprehensive (loss)/income items [(a)]				
Other comprehensive (loss)/income	$ 32	$ 10	$ (13)	1
Comprehensive loss	$ (44,380)	$ (48,775)	$ (59,646)	$ (378,521)

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

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Amicus Therapeutics, Inc.
(a development stage company)

Consolidated Statements of Changes in Stockholders' (Deficiency)/Equity
Period from February 4, 2002 (inception) to December 31, 2013,
and the eleven year period ended December 31, 2013
(in thousands, except share amounts)

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

Stock issued from exercise of warrants	133,332	10	65				75
Deferred compensation			2,778		(2,778)		
Amortization of deferred compensation					365		365
Non-cash charge for stock options to consultants			112				112
Accretion of redeemable convertible preferred stock			(139)				(139)
Comprehensive Loss:							
Unrealized holding loss on available-for-sale securities				(7)			(7)
Net loss						(19,972)	(19,972)
Balance at December 31, 2005	538,025	$ 40	$ 4,016	$ (16)	$ (2,547)	$ (37,322)	$ (35,829)

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Amicus Therapeutics, Inc.
(a development stage company)

Consolidated Statements of Changes in Stockholders' (Deficiency) Equity
Period from February 4, 2002 (inception) to December 31, 2003,
and the eleven year period ended December 31, 2013
(in thousands, except share amounts)

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

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Amicus Therapeutics, Inc.
(a development stage company)

Consolidated Statements of Changes in Stockholders' (Deficiency) Equity
Period from February 4, 2002 (inception) to December 31, 2003,
and the eleven year period ended December 31, 2013
(in thousands, except share amounts)

	Common Stock		Additional Paid-In Capital	Other Comprehensive Gain/ (Loss)	Deferred Compensation	Deficit Accumulated During the Development Stage	Total Stockholders' (Deficiency) Equity
	Shares	Amount					
Balance at December 31, 2008	22,634,711	$ 287	$ 234,412	$ 533	$	$ (164,189)	$ 71,043
Stock issued from exercise of stock options, net	37,716		60				60
Stock based compensation			7,787				7,787
Comprehensive (Loss)/ Income:							
Unrealized holding loss on available-for-sale securities				(490)			(490)
Net loss						(6,567)	(6,567)
Balance at December 31, 2009	22,672,427	$ 287	$ 242,259	$ 43	$	$ (170,756)	$ 71,833
Stock issued from secondary offering	4,946,525	50	13,780				13,830
Stock issued from collaboration agreement	6,866,245	69	28,014				28,083
Stock issued from exercise of stock options, net	23,735		9				9
Stock based compensation			6,186				6,186
Comprehensive (Loss)/ Income:							
Unrealized holding loss on available-for-sale securities				(71)			(71)
Net loss						(54,936)	(54,936)
Balance at December 31, 2010	34,508,932	$ 406	$ 290,248	$ (28)	$	$ (225,692)	$ 64,934
Stock issued from exercise of stock options, net	145,274	1	359				360
Stock based compensation			8,678				8,678
Comprehensive (Loss)/ Income:							
Unrealized holding gain on available-for-sale securities				32			32
Net loss						(44,412)	(44,412)
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

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Amicus Therapeutics, Inc.
(a development stage company)

Consolidated Statements of Changes in Stockholders' (Deficiency) Equity
Period from February 4, 2002 (inception) to December 31, 2003,
and the eleven year period ended December 31, 2013
(in thousands, except share amounts)

	Common Stock		Additional Paid-In Capital	Other Comprehensive Gain/ (Loss)	Deferred Compensation	Deficit Accumulated During the Development Stage	Total Stockholders' (Deficiency) Equity
	Shares	Amount					
Balance at December 31, 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

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Amicus Therapeutics, Inc.
(a development stage company)

Consolidated Statements of Cash Flows
(in thousands)

	Years Ended December 31,			Period from February 4, 2002 (Inception) to December 31, 2013
	2011	2012	2013	
Operating activities				
Net loss	$ (44,412)	$ (48,785)	$ (59,633)	$ (378,522)
Adjustments to reconcile net loss to net cash used in operating activities:				
Non-cash interest expense				525
Depreciation and amortization	1,585	1,705	1,719	13,487
Amortization of non-cash compensation				522
Stock-based compensation	8,679	6,191	6,177	48,106
Non-cash charge for stock based compensation issued to consultants				853
Restructuring charges			1,988	3,510
Change in fair value of warrant liability	(2,764)	(653)	(908)	(2,461)
Loss on disposal of asset		28		388
Stock-based license payment				1,220
Impairment of leasehold improvements				1,030
Non-cash charge for in process research and development				418
Beneficial conversion feature related to bridge financing				135
Changes in operating assets and liabilities:				
Receivable due from GSK	(5,043)	1,818	2,466	(759)
Prepaid expenses and other current assets	(3,667)	3,633	(3,249)	(5,519)
Other non-current assets	(442)	267		(466)
Account payable and accrued expenses	1,418	(863)	(613)	6,710
Deferred reimbursements	(4,776)	2,915	6,259	36,677
Net cash used in operating activities	(49,422)	(33,744)	(45,794)	(274,146)
Investing activities				
Sale and redemption of marketable securities	98,474	83,352	83,337	838,779
Purchases of marketable securities	(50,602)	(118,459)	(56,559)	(877,254)
Purchases of property and equipment	(1,420)	(4,324)	(695)	(18,908)
Net cash provided by/ (used in) investing activities	46,452	(39,431)	26,083	(57,383)
Financing activities				
Proceeds from the issuance of preferred stock, net of issuance costs				143,022
Proceeds from issuance of common stock and warrants, net of issuance costs		80,195	15,000	208,441
Proceeds from the issuance of convertible notes				5,000
Payments of capital lease obligations	(40)			(5,587)
Payments of secured loan agreement	(1,253)	(1,342)	(398)	(4,454)
Payments related to deferred financing			(110)	(110)
Proceeds from exercise of stock options	359	1,630		3,341
Proceeds from exercise of warrants (common and preferred)				264
Proceeds from capital asset financing arrangement				5,611
Proceeds from secured loan agreement		995	14,888	19,641

Net cash (used in)/ provided by financing activities	(934)	81,478	29,380	375,169
Net (decrease)/ increase in cash and cash equivalents	(3,904)	8,303	9,669	43,640
Cash and cash equivalents at beginning of year/ period	29,572	25,668	33,971	
Cash and cash equivalents at end of year/period	$ 25,668	$ 33,971	$ 43,640	$ 43,640
Supplemental disclosures of cash flow information				
Cash paid during the period for interest	$ 149	$ 84	$ 30	$ 2,147
Non-cash activities				
Conversion of warrants to common stock	$	$ 386	$	$ 386
Conversion of notes payable to Series B redeemable convertible	$	$	$	$ 5,000
Conversion of preferred stock to common stock	$	$	$	$ 148,951
Accretion of redeemable convertible preferred stock	$	$	$	$ 802
Beneficial conversion feature related to issuance of the additional issuance of Series C redeemable convertible preferred stock	$	$	$	$ 19,424

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Amicus Therapeutics, Inc.
(a development stage company)

Notes To Consolidated Financial Statements

1. Description of Business

Corporate Information, Status of Operations, and Management Plans

Amicus Therapeutics, Inc. ("the Company") was incorporated on February 4, 2002 in Delaware and is a biopharmaceutical company focused on the discovery, development and commercialization of 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 program is migalastat HCl for Fabry disease. Migalastat HCl is a novel, small molecule pharmacological chaperone in development as a monotherapy and in combination with enzyme replacement therapy "(ERT") for Fabry disease. The Company is leveraging its Chaperone-Advanced Replacement Therapy, or CHART platform to develop next-generation therapies that combine pharmacological chaperones with enzyme therapies for Pompe, Mucopolysaccharidosis Type I ("MPS I") and Gaucher diseases. Current CHART programs for Pompe disease include the pharmacological chaperone AT2220 (duvoglustat HCl) co-administered with currently marketed Pompe ERTs (Myozyme®/Lumizyme®), as well as AT2220 co-formulated with a proprietary Pompe ERT. The Company's activities since inception have consisted principally of raising capital, establishing facilities, and performing research and development. Accordingly, the Company is considered to be in the development stage.

In November 2013, the Company completed the acquisition of Callidus Biopharma, Inc. ("Callidus") through an Agreement and Plan of Merger ("the Merger Agreement") between the Company's wholly owned subsidiary, CB Acquisition Corp ("CB") and Callidus whereby CB merged with and into Callidus with Callidus becoming the surviving corporation of the merger. As a result of the merger, Callidus became a wholly owned subsidiary of Amicus. For further information, see " Note 3. Acquisition of Callidus Biopharma,Inc. Callidus was a privately-held biologics company focused on developing best-in-class enzyme replacement therapies (ERTs) for lysosomal storage diseases (LSDs). Callidus' lead ERT is a recombinant human acid-alpha glucosidase (rhGAA, called "AT-B200") for Pompe disease in late preclinical development

In November 2013, Amicus 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 HCl 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 is no upfront payment from Amicus to GSK. For the next-generation Fabry ERT (migalastat HCl co-formulated with ERT), GSK is eligible to receive single-digit royalties on net sales in eight major markets outside the U.S. For migalastat HCl 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.

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) shares of the Company's common stock and b) a 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 and GSK's resulting equity stake in the Company was 17.6% at December 31, 2013.

In September 2013, the Company entered into a collaboration agreement with Biogen Idec ("Biogen") to discover, develop and commercialize novel small molecules for the treatment of

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Amicus Therapeutics, Inc.
(a development stage company)

Notes To Consolidated Financial Statements (Continued)

Parkinson's disease. The collaboration will build upon preclinical studies at the Company and independent published research that suggest increasing activity of the lysosomal enzyme glucocerobrosidase ("GCase") in the brain may correct alpha-synuclein pathology and other deficits associated with Parkinson's disease. Under terms of the multi-year agreement, the Company and Biogen 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 will be responsible for funding all discovery, development, and commercialization activities. In addition the Company will be reimbursed for all full-time employees working on the project. The Company is also eligible to receive development and regulatory milestones, as well as modest royalties on global net sales.

For further information, see " Note 15. Collaborative Agreements"

The Company had an accumulated deficit of approximately $378.5 million at December 31, 2013 and anticipates incurring losses through the fiscal year ending December 31, 2014 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. In March 2010, the Company sold 4.95 million shares of its common stock and also sold warrants to purchase 1.9 million shares of common stock in a registered direct offering to a select group of institutional investors for net proceeds of $17.1 million. In October 2010, the Company sold 6.87 million shares of its common stock as part of the Original Collaboration Agreement with GSK for proceeds of $31.0 million. In March 2012, the Company sold 11.5 million shares of its common stock in a stock offering for net proceeds of $62.0 million. In July 2012, the Company sold 2.9 million shares of its common stock as part of the Expanded Collaboration Agreement with GSK for proceeds of $18.6 million. In November 2013, the Company sold 7.5 million shares of its common stock and also sold warrants to purchase 1.6 million shares of its common stock in a private placement for proceeds of $15 million. The Company believes that its existing cash and cash equivalents and short-term investments will be sufficient to cover its cash flow requirements for 2014.

2. Summary of Significant Accounting Policies

Basis of Presentation

The accompanying consolidated financial statements have been prepared in accordance with 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

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Amicus Therapeutics, Inc.
(a development stage company)

Notes To Consolidated Financial Statements (Continued)

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 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, 2013 and 2012.

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

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Amicus Therapeutics, Inc.
(a development stage company)

Notes To Consolidated Financial Statements (Continued)

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, which were applied in fiscal 2010, provide that, when a collaboration arrangement contains multiple deliverables, such as license and research and development services, the Company allocates revenue to each separate unit of accounting based on a selling price hierarchy. The selling price hierarchy for a deliverable is based on (i) its vendor specific objective evidence ("VSOE") if available, (ii) third party evidence ("TPE") if VSOE is not available, or (iii) 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

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

Fair Value Measurements

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

Level 1 inputs utilize quoted prices (unadjusted) in active markets for identical assets or liabilities that the Company has the ability to access at measurement date. Level 2 inputs are inputs other than quoted prices included in Level 1 that are observable for the asset or liability, either directly or indirectly. Level 2 inputs may include quoted prices for similar assets and liabilities in active markets, as well as inputs that are observable for the asset or liability (other than quoted prices), such as interest rates, foreign exchange rates, and yield curves that are observable at commonly quoted intervals. Level 3 inputs are unobservable inputs for the asset or liability, which 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.

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Other Income and Expenses

Other income includes funds received from the U.S. Treasury Department in 2011 for the Qualified Therapeutic Discovery Projects tax credit and grant program and the gain on sale of certain fixed assets in 2012. Other expenses include costs directly attributable to a planned offering of the Company's securities that were subsequently withdrawn during 2006 and the losses on the disposal of certain fixed assets.

Income Taxes

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

Other Comprehensive Income/ (Loss)

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

Leases

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

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

Stock-Based Compensation

At December 31, 2013, 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.

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

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

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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,		
		2011	2012	2013
Historical				
Numerator:				
Net loss attributable to common stockholders		$ (44,412)	$ (48,785)	$ (59,633)
Denominator:				
Weighted average common shares outstanding	basic and diluted	34,569,642	45,565,217	51,286,059

Dilutive common stock equivalents would include the dilutive effect of common stock options and warrants for common stock equivalents. Potentially dilutive common stock equivalents totaled approximately 8.5 million, 9.4 million and 12.0 million for the years ended December 31, 2011, 2012 and 2013, respectively. Potentially dilutive common stock equivalents were excluded from the diluted earnings per share denominator for all periods because of their anti-dilutive effect.

Dividends

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

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.

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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 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 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.

Recent Accounting Pronouncements

In July 2013, the FASB issued an update that clarified existing guidance on the presentation of unrecognized tax benefits when various qualifying tax benefit carryforwards exist, including when the unrecognized tax benefit should be presented as a reduction to deferred tax assets or as a liability. This update is required to be adopted for all annual periods and interim reporting periods beginning after December 15, 2013, with early adoption permitted. The Company is evaluating the impact of this new provision on the consolidated results of operations or financial position.

In February 2013, the FASB amended its guidance to require an entity to present the effect of certain significant reclassifications out of accumulated other comprehensive income on the respective line items in net income. The new accounting guidance does not change the items that must be reported in other comprehensive income or when an item of other comprehensive income must be reclassified as net income. The guidance is effective prospectively for fiscal years beginning after December 15, 2012. The Company adopted these new provisions for the quarterly period beginning January 1, 2013. As the guidance requires additional presentation only, there was no impact on the Company's consolidated results of operations or financial position.

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.

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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 AT-B200 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, 2013, 4.8 million shares were issued and 2.4 million shares remain issuable to former Callidus shareholders. 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 13.5% and various probability factors. As of December 31, 2013, the range of outcomes and assumptions used to develop these estimates has not changed (see " Note 10. Assets and Liabilities Measured at Fair Value" for additional discussion regarding fair value measurements of the contingent acquisition consideration payable).

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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 Company is still in the process of valuing the assets acquired and liabilities assumed; therefore the allocation of the acquisition consideration is still subject to change.

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, 2012	2013
Revenue	$ 18,411	$ 363
Net income (loss)	$ (49,807)	$ (61,804)

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. Management determined that the carrying value did not exceed the fair value due to the short time period from the date of acquisition until the end of the year, and as a result no impairment of goodwill existed at December 31, 2013. The following table represents the changes in goodwill for the year ended December 31, 2013 (in thousands):

Balance at December 31, 2012	$
Goodwill related to the acquisition of Callidus (See Note 3)	11,613
Balance at December 31, 2013	$ 11,613

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. Management determined that the carrying value did not exceed the fair value due to the short time period from the date of acquisition until the end of the year and as a result, no impairment of IPR&D existed at December 31, 2013. The following table represents the changes in IPR&D for the year ended December 31, 2013 (in thousands):

Balance at December 31, 2012	$
IPR&D related to the acquisition of Callidus (See Note 3)	23,000
Balance at December 31, 2013	$ 23,000

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6. Cash, Money Market Funds and Marketable Securities

As of December 31, 2013, the Company held $43.6 million in cash and cash equivalents and $38.4 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. Cash and available for sale securities consisted of the following as of December 31, 2012 and December 31, 2013 (in thousands):

	As of December 31, 2012			
	Cost	Unrealized Gain	Unrealized Loss	Fair Value
Cash balances	$ 33,971	$	$	$ 33,971
Corporate debt securities	42,503	5	(11)	42,497
Commercial paper	19,725	19		19,744
Certificate of deposit	2,909	1		2,910
	$ 99,108	$ 25	$ (11)	$ 99,122
Included in cash and cash equivalents	$ 33,971	$	$	$ 33,971
Included in marketable securities	65,137	25	(11)	65,151
Total cash and marketable securities	$ 99,108	$ 25	$ (11)	$ 99,122

	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

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All of the Company's available for sale investments as of December 31, 2012 and December 31, 2013 are due in one year or less.

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, 2012 unrealized holding gains of $10 thousand and for the year ended December 31, 2013, unrealized holding loss of $13 thousand respectively, were included in the statements of comprehensive loss.

For the years ended December 31, 2012 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, 2012 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 $33.1 million and $23.6 million as of December 31, 2012 and December 31, 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 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, 2012 and 2013, were as follows (in thousands):

	Year Ended December 31,	
	2012	2013
Balance, beginning	$ 4	$ 14
Current period changes in fair value,[(a)]	10	(13)
Reclassification of earnings,[(a)]		
Balance, ending	$ 14	$ 1

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

7. Property and Equipment

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

	December 31,	
	2012	2013
Property and equipment consist of the following:		
Computer equipment	$ 3,399	$ 3,537
Computer software	849	1,064
Research equipment	5,807	5,918
Furniture and fixtures	1,544	1,527
Leasehold improvements	1,931	2,047

	13,530	14,093
Less accumulated depreciation and amortization	(8,501)	(9,973)
	$ 5,029	$ 4,120

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Depreciation and amortization expense was $1.7 million each for the years ended December 31, 2013 and 2012 and $13.5 million for the Period February 4, 2002 (inception) to December 31, 2013, respectively. There were no capital lease obligations outstanding as of December 31, 2013.

8. Accounts Payable and Accrued Expenses

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

	December 31,	
	2012	2013
Accounts payable	$ 2,115	$ 2,146
Accrued professional fees	409	498
Accrued contract manufacturing & contract research costs	1,743	1,499
Accrued compensation and benefits	4,229	4,781
Accrued facility costs	167	963
Accrued other	182	275
	$ 8,845	$ 10,162

9. Stockholders' Equity

Common Stock and Warrants

As of December 31, 2013, the Company was authorized to issue 125,000,000 shares of common stock. Dividends on common stock will be paid when, and if declared by the board of directors. Each holder of common stock is entitled to vote on all matters that are appropriate for shareholder voting and is entitled to one vote for each share held.

The fair value of the warrant liability issued in connection with the March 2010 registered direct offering de minimis as of December 31, 2013. These warrants expired on March 2, 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) shares of the Company's common stock, par value $0.01 (the "Common Stock") and b) 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 shareholders prior to consummation of these transactions. The Shares and the Units sold to the investors were offered and 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 (b) 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. At December 31, 2013, GSK's resulting equity stake in the Company was 17.6%.

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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 tern 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, 2013, 4.8 million of these shares were issued and balance of the shares is expected to be issued by March 31, 2014.

In July 2012, Amicus and GSK entered into the SPA pursuant to which GSK purchased 2.9 million unregistered shares of Amicus common stock at a price of $6.30 per share. The total purchase price for these shares was $18.6 million. In March 2012, the Company sold 11.5 million shares of its common stock at a public offering price of $5.70 through a Registration Statement on Form S-3 that was declared effective by the SEC in May 2009. The aggregate offering proceeds were $65.6 million.

In October 2010, GSK purchased approximately 6.9 million shares of the Company's common stock at $4.56 per share, in connection with the Original Collaboration Agreement. The total value of this equity investment was approximately $31 million.

In March 2010, the Company sold 4.95 million shares of its common stock and warrants to purchase 1.9 million shares of common stock in a registered direct offering to a selected group of institutional investors through a Registration Statement on Form S-3 that was declared effective by the SEC in May 2009. The shares of common stock and warrants were sold in units consisting of one share of common stock and one warrant to purchase 0.375 shares of common stock at a price of $3.74 per unit. The warrants have a term of four years and are exercisable any time on or after the six month anniversary of the date they were issued, at an exercise price of $4.43 per share. The aggregate offering proceeds were $18.5 million. There were approximately 1.4 million warrants outstanding at December 31, 2013. These warrants expired on March 2, 2014

Stock Option Plans

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

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The 2007 Director Plan is intended to promote the recruiting and retention of highly qualified eligible directors and strengthen the commonality of interest between directors and stockholders by encouraging ownership of common stock of the Company. The options granted under the 2007 Director Plan are NSOs and under the provisions of this plan, no option will have a term in excess of 10 years.

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

As of December 31, 2013, there were no shares reserved for issuance under the 2002 Plan. The Company has reserved up to 2,508,411 shares for issuance under the 2007 Plan and the 2007 Director Plan.

The Company recognized stock-based compensation expense of $8.7 million, $6.2 million and $6.2 million in 2011, 2012 and 2013, respectively. The following table summarizes the stock compensation expense recognized in the statements of operations (in thousands):

	Years Ended December 31,		
	2011	2012	2013
Stock compensation expense recognized in:			
Research and development expense	$ 2,928	$ 3,603	$ 3,583
General and administrative expense	5,751	2,588	2,594
Total stock compensation expense	$ 8,679	$ 6,191	$ 6,177

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

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75% and it has experienced significant business transactions (Shire and GSK collaborations), 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.

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

	Years Ended December 31,		
	2011	2012	2013
Expected stock price volatility	78.8%	77.2%	82.0%
Risk free interest rate	2.0%	0.8%	1.3%
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 2011, 2012 and 2013 were $4.11, $3.31 and $2.14, 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 thousands)
Options outstanding, December 31, 2010	5,104.1	$ 7.27		
Granted	2,217.0	$ 5.92		
Exercised	(108.5)	$ 3.88		
Forfeited	(559.1)	$ 7.34		
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	7.0 years	$ 44.4
Vested and unvested expected to vest, December 31, 2013	8,637.7	$ 5.73	6.9 years	$ 43.3
Exercisable at December 31, 2013	5,502.7	$ 6.68	5.8 years	$ 38.5

The aggregate intrinsic value of options exercised during the years ended December 31, 2011 and 2012 was $0.3 million and $0.9 million, respectively. There were no options exercised during the year ended December 31, 2013. As of December 31, 2013, the total unrecognized

compensation cost related to non-vested stock options granted was $7.3 million and is expected to be recognized over a weighted

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average period of 2.3 years. Cash proceeds from stock options exercised during the years ended December 31, 2011 and 2012 were $0.4 million and $1.6 million respectively.

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

Upon vesting in 2011, there were 13,225 shares surrendered to fund minimum statutory tax withholding requirements. There were no restricted stock awards in 2011, 2012 or 2013. As of December 31, 2013, there was no unrecognized compensation cost related to unvested restricted stock awards. The total fair value of restricted stock awards which vested during 2011 was $0.4 million.

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, 2013. No transfers of assets between Level 1 and Level 2 of the fair value measurement hierarchy occurred during the year ended December 31, 2013.

Secured Debt

As disclosed in Note 16, 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, 2013. 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, 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 MMA submission. This is effective 5 years from the closing of

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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 arises from acquisition of Callidus, as discussed in Note 3. The valuation of the contingent consideration payable is estimated using a probability-based income approach utilizing an appropriate discount rate. Subsequent changes in the fair value of the contingent consideration payable will be recorded in intangible asset amortization and contingent consideration in the Company's consolidated statements of operations.

Warrants

The Company allocated $3.3 million of proceeds from its March 2010 registered direct offering to warrants issued in connection with the offering that was classified as a liability. The valuation of the warrants is determined using the Black-Scholes model. This model uses inputs such as the underlying price of the shares issued when the warrant is exercised, volatility, risk free interest rate and expected life of the instrument. The Company has determined that the warrant liability should be classified within Level 3 of the fair value hierarchy by evaluating each input for the Black-Scholes model against the fair value hierarchy criteria and using the lowest level of input as the basis for the fair value classification. There are six inputs: closing price of Amicus stock on the day of evaluation; the exercise price of the warrants; the remaining term of the warrants; the volatility of Amicus' stock over that term; annual rate of dividends; and the riskless rate of return. Of those inputs, the exercise price of the warrants and the remaining term are readily observable in the warrant agreements. The annual rate of dividends is based on the Company's historical practice of not granting dividends. The closing price of Amicus stock would fall under Level 1 of the fair value hierarchy as it is a quoted price in an active market. The riskless rate of return is a Level 2 input, while the historical volatility is a Level 3 input in accordance with the fair value accounting guidance. Since the lowest level input is a Level 3, the Company determined the warrant liability is most appropriately classified within Level 3 of the fair value hierarchy. This liability is subject to fair value mark-to-market adjustment each period. The Company recognized the change in the fair value of the warrant liability as non-operating income of $0.9 million for the year ended December 31, 2013. There was no resulting fair value of the warrant liability at December 31, 2013. The weighted average assumptions used in the Black-Scholes valuation model for the warrants are as follows:

	December 31,	
	2012	**2013**
Expected stock price volatility	93.2%	45.9%
Risk free interest rate	0.17%	0.07%
Expected life of warrants (years)	1.17	0.17
Expected annual dividend per share	$ 0.00	$ 0.00

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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, 2012 are identified in the following table (in thousands):

	Level 1	Level 2	Total
Assets:			
Cash/money market funds	$ 33,971	$	$ 33,971
Commercial paper		19,744	19,744
Corporate debt securities		42,497	42,497
Certificate of deposit		2,910	2,910
	$ 33,971	$ 65,151	$ 99,122

	Level 1	Level 2	Level 3	Total
Liabilities:				
Secured debt	$	$ 697	$	$ 697
Warrant liability			908	908
	$	$ 697	$ 908	$ 1,605

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
Warrant liability				
Contingent consideration payable			10,600	10,600
	$	$ 14,473	$ 10,864	$ 25,337

The change in the fair value of the Level 3 liability was a decrease of $0.7 million at December 31, 2012, after settlement of $0.3 million upon the exercise of 0.5 million warrants. 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 the 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.

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11. 401(k) Plan

The Company has a 401(k) plan (the Plan) covering all eligible employees. During 2007, the Board of Directors approved a company matching program that began on January 1, 2008. The matching program allows for a company match of up to 5% of salary and bonus paid during the year. In 2013, the Company changed the vesting policy whereby the match vests immediately upon enrollment. The Company's total contribution to the Plan was $0.6 million, $0.7 million and $0.7 million for the years ended December 31, 2011, 2012 and 2013, respectively.

12. Leases

Operating Leases

In 2011, the Company entered into a lease agreement to lease approximately 73,646 square feet of laboratory and office space in Cranbury, New Jersey. The initial term of the lease, which commenced in March 2012, is for seven years and may be extended by the Company for two additional five-year periods. In 2008, the Company entered into a lease agreement for its laboratory and office space in San Diego, CA, which will expire in September 2016. As part of the restructuring efforts, this 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, 2013, aggregate annual future minimum lease payments under these leases are as follows (in thousands):

Operating Leases	
Years ending December 31:	
2014	$ 1,930
2015	2,033
2016	2,039
2017	1,769
2018 and beyond	2,074
	$ 9,845

Rent expense for the years ended December 31, 2011, 2012 and 2013 were $2.3 million, $2.6 million and $2.6 million respectively.

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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, 2013 and did not accrue for interest or penalties as of December 31, 2013. The Company does not have an accrual for uncertain tax positions as of December 31, 2013. Tax returns for all years 2006 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,	
	2012	2013
Current deferred tax asset		
Non-cash stock issue	$ 7,100	$ 8,172
Others	1,257	1,343
	8,357	9,515
Non-current deferred tax assets		
Amortization/depreciation	3,176	3,068
Research tax credit	9,072	13,680
Net operating loss carry forwards	62,060	79,984
Deferred revenue	12,149	14,649
Others	520	682
Gross deferred tax assets	95,334	121,578
Deferred tax liability related to business acquisition		(9,186)
Total net deferred tax asset	95,334	112,392
Less valuation allowance	(95,334)	(121,578)
Net deferred tax assets (liability)	$	$ (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, 2012, and 2013, the Company recorded valuation allowances of $95.3 million and $121.6 million, respectively, representing an increase in the valuation allowance of $20.1 million in 2012 and an increase of $26.3 million in 2013, due to the uncertainty regarding the realization of such deferred tax assets, to offset the benefits of net operating losses generated during those years.

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As of December 31, 2013, the Company had federal and state net operating loss carry forwards (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 in 2009 through 2012 will expire in 2029 through 2032 due to a change in the New Jersey state law regarding the net operating loss carry forward period. Utilization of NOLs may be subject to a substantial annual limitation in the event of an ownership change that has occurred previously or could occur in the future pursuant to Section 382 of the Internal Revenue Code of 1986, as amended, as well as similar state provisions. An ownership change may limit the amount of NOLs that can be utilized annually to offset future taxable income and tax, and may, in turn, result in the expiration of a portion of those carry forwards before utilization. In general, an ownership change, as defined by Section 382, results from transactions that increase the ownership of certain shareholders or public groups in the stock of a corporation by more than 50 percentage points over a three year period. The Company completed a detailed study of its NOLs and determined that in 2013, there was no ownership change in excess of 50%; therefore there was no write-down to net realizable value of the federal NOLs and research and development credits subject to the 382 limitations. A tax benefit of $2.0 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, 2011, 2012 and 2013 are as follows:

	Years Ended December 31,		
	2011	2012	2013
Statutory rate	(34)%	(34)%	(34)%
State taxes, net of federal benefit	(13)	(3)	(5)
Permanent adjustments	3		(1)
R&D credit		(8)	(3)
Other	2	1	
Valuation allowance	34	38	37
Net	(8)%	(6)%	(6)%

The Company recognized a tax benefit of $3.6 million, $3.2 million and $3.5 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, 2011, 2012 and 2013, 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:

Mt. Sinai School of Medicine of New York University (MSSM) The Company acquired exclusive worldwide patent rights to develop and commercialize migalastat HCl, afegostat and AT2220 and other pharmacological chaperones for the prevention or treatment of human diseases or clinical conditions by increasing the activity of wild-type and mutant enzymes pursuant to a license agreement with MSSM. In connection with this agreement, the Company issued 232,266 shares of common stock to MSSM in

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April 2002. In 2006, the Company amended its license agreement with MSSM to expand its exclusive worldwide patent rights to develop and commercialize pharmacological chaperones. In connection with the amendment, the Company paid $1.0 million and issued 133,333 shares of its common stock with an estimated fair value of $1.2 million to MSSM. In total, the Company recorded $2.2 million of research and development expense in connection with the amendment in 2006. This agreement expires upon expiration of the last of the licensed patent rights, which will be in 2019, subject to any patent term extension that may be granted, or 2024 if 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. For further information see " Note 15. Collaborative Agreements." Under the terms of the amended and restated license agreement, the Company agreed to pay $2.6 million to MSSM in connection with the $50 million upfront payment that the Company received from a collaboration agreement in November 2007 and an additional $2.6 million for the sole right to and control over the prosecution of patent rights. In accordance with the Company's license agreement with MSSM, the Company paid $3 million of the $30 million upfront payment received from GSK to MSSM in December 2010 and $0.35 million of the $3.5 million milestone payment received from GSK in August 2012, pursuant to the Original Collaboration Agreement. These payments to MSSM are classified as research and development expenses in the Company's financial statements.

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

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

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be $7.8 million. The Company is also required to pay royalties on net sales. This license will terminate in 2016.

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

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

15. Collaborative Agreements

GSK

In October 2010, the Company entered into the Original Collaboration Agreement with Glaxo Group Limited, an affiliate of GSK, to develop and commercialize migalastat HCl. Under the terms of the Original Collaboration Agreement, GSK received an exclusive worldwide license to develop, manufacture and commercialize migalastat HCl. In consideration of the license grant, the Company received an upfront, license payment of $30 million from GSK and was eligible to receive further payments of approximately $173.5 million upon the successful achievement of development, regulatory and commercialization milestones, as well as tiered double-digit royalties on global sales of migalastat HCl. Potential payments included up to (i) $13.5 million related to the attainment of certain clinical development objectives and the acceptance of regulatory filings in select worldwide markets, (ii) $80 million related to market approvals for migalastat HCl in selected territories throughout the world, and (iii) $80 million associated with the achievement of certain sales thresholds. GSK and the Company were jointly funding development costs in accordance with an agreed upon development plan. Additionally, GSK purchased approximately 6.9 million shares of the Company's common stock at $4.56 per share, a 30% premium on the average price per share of the Company's stock over a 60 day period preceding the closing date of the transaction. The total value of this equity investment to the Company was approximately $31 million.

In July 2012, the Company entered into the Expanded Collaboration Agreement with GSK pursuant to which the Company and GSK continue to develop and commercialize migalastat HCl, currently in Phase 3 development for the treatment of Fabry disease. The Expanded Collaboration Agreement amended and replaced in its entirety the Original Collaboration Agreement. Under the terms of the Expanded Collaboration Agreement, the Company and GSK were to co-develop all formulations of migalastat HCl for Fabry disease, including the development of migalastat HCl co-formulated with an investigational enzyme replacement therapy (ERT) for Fabry disease (the "Co-formulated Product").

Additionally, simultaneous with entry into the Expanded Collaboration Agreement, Amicus and GSK entered into an SPA pursuant to which GSK purchased approximately 2.9 million shares of Amicus common stock at a price of $6.30 per share for proceeds of $18.6 million.

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In November 2013, Amicus entered into the Revised Agreement with GSK, pursuant to which Amicus has obtained global rights to develop and commercialize migalastat HCl 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 the next-generation Fabry ERT (migalastat HCl co-formulated with ERT), GSK is eligible to receive single-digit royalties on net sales in eight major markets outside the U.S. For migalastat HCl 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 HCl.

Under the Original Collaboration Agreement, the upfront license fee, together with the premium received on the stock purchase, was being recognized as Collaboration Revenue over the original development period. In addition, the Company was receiving reimbursements of research expenditures under the cost sharing arrangement which was being accounted for as Research Revenue on the statement of operations. Under the Expanded Collaboration Agreement, the Company will continue to receive research expense reimbursements for the development of migalastat HCl but may be required to pay contingent milestones to GSK in the future related to the U.S. commercial rights to migalastat HCl.

In accordance with the revenue recognition guidance related to multiple-element arrangements, the Company identified all of the deliverables at the inception of the Expanded Collaboration Agreement. The significant deliverables were determined to be the rest of world licensing rights to migalastat HCl, the research services to continue and complete the development of migalastat HCl and the delivery of the Company's common stock. The Company determined that the rest of world licensing rights and the research services represent one unit of accounting as none of these deliverables on its own has standalone value separate from the other. The Company also determined that the delivery of the Company's common stock does have standalone value separate from the rest of world licensing rights and the research services. As a result, the Company's common stock was considered a separate unit of accounting and was accounted for as an issuance of common stock. However, as the Company's common stock was sold at a premium to the market closing price, the premium amount paid over the market closing price was determined to be additional consideration paid to the Company for the collaboration agreement and was included as consideration for the single unit of accounting (rest of world licensing rights and research services) identified above.

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 (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

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,

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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 does not believe that, for accounting purposes, the new U.S. licensing rights to migalastat HCl obtained from GSK under the Expanded Collaboration Agreement, nor the ex U.S. licensing rights to migalastat HCl obtained from GSK under the Revised Agreement, represent a separate, identifiable benefit from the licenses in the Original Collaboration Agreement. The contingent amounts payable to GSK are 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 HCl.

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 recognizes 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 is also affected by the determination that the overall total arrangement consideration is no longer fixed and determinable, despite the fact that the research activities will continue and that the research expense reimbursements by GSK to Amicus will be received as the research activities related to the reimbursement would have already been completed. Therefore any research reimbursements from GSK are recorded as deferred reimbursements on the balance sheet and not recognized until the total arrangement consideration becomes fixed and determinable.

As a result, all revenue recognition was suspended until the total arrangement consideration becomes 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 is 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.

Under the Original Collaboration Agreement, the Company evaluated the contingent milestones and determined that they were substantive milestones and would be recognized as revenue in the period that the milestone is achieved. The Company determined that the research based milestones were 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 have related to past performances when achieved and were reasonable relative to the other payment terms within the Original Collaboration Agreement. In June 2012, the Company achieved a clinical development

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milestone and recognized $3.5 million of milestone revenue. Under the terms of the Expanded Collaboration Agreement, the Company is no longer entitled to receive any milestone payments from GSK.

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 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 will be responsible for funding all discovery, development, and commercialization activities. In addition the Company will be reimbursed for all full-time employees working on the project as part of a cost sharing arrangement. The Company is 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. The Company has not commenced its planned principal operations (i.e. selling commercial products) and is therefore a development stage enterprise. 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, 2013, the Company recognized $0.4 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 will be recognized as revenue in the period that the milestone is achieved. The Company determined that the research based milestones are commensurate with the enhanced value of each delivered item as a result of the Company's specific performance to achieve the milestones. 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.

16. Short-Term Borrowings and Long-Term Debt

In August 2011, the Company entered into a loan and security agreement (the "2011 Loan Agreement") with Silicon Valley Bank ("SVB") in order to finance certain capital expenditures to be made by the Company in connection with its move in March 2012 to new office and laboratory space in Cranbury, New Jersey. The 2011 Loan Agreement provided for up to $3 million of equipment financing through January 2014. Borrowings under the 2011 Loan Agreement were collateralized by equipment purchased with the proceeds of the loan and bear interest at a variable rate of SVB prime + 2.5%. The current SVB prime rate is 4.0%. In February 2012, the Company borrowed approximately

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Amicus Therapeutics, Inc.
(a development stage company)

Notes To Consolidated Financial Statements (Continued)

$1.0 million from the 2011 Loan Agreement which will 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. At December 31, 2013, the total amount due under the 2011 Loan Agreement was $0.3 million.

In December 2013, the Company entered into a credit and security agreement (the "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"). The Company drew $15 million of the aggregate principal amount of the Term Loan at the end of December 2013 (the "First Tranche") and may draw up to an additional $10 million through the end of the fourth quarter of 2014 (the "Second Tranche"). The principal outstanding balance of the First Tranche bears interest at a rate per annum fixed at 8.5%. If the Company draws from the Second Tranche, the principal outstanding balance of the Second Tranche will also have a fixed interest rate, which will be determined by reference to the applicable index rate at the time of the draw. The Company will make interest-only payments on the Term Loan beginning January 1, 2014 and continuing 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, 2013, the total principal amount due under the Term Loan was $15 million.

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 MMA 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 non-current liability on the Company's consolidated balance sheet.

The carrying amount of the Company's borrowings approximates fair value at December 31, 2013.

The remaining future minimum payments of principal due as of December 31, 2013 are as follows (in thousands):

Years ending December 31:	
2014	$ 299
2015	4,035
2016	5,443
2017	5,522
2018 and beyond	
Total principal obligation	15,299
Less short-term portion	(299)
Long-term portion, gross of debt discount	15,000
Less debt discount	(826)
Long term portion, net of debt discount	$ 14,174

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Amicus Therapeutics, Inc.
(a development stage company)

Notes To Consolidated Financial Statements (Continued)

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 subleased locations in San Diego, CA. The Company recorded a total charge of $1.9 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 fixed assets in the vacated building of $0.1 million. At December 31, 2013, $1.1 million of the restructuring charges related to employment termination costs were unpaid and classified under accrued expenses on the balance sheet.

The following table summarizes the restructuring charges and utilization for the year ended December 31, 2013 (in thousands):

	Balance as of December 31, 2012	Charges	Cash Payments	Adjustments	Balance as of December 31, 2013
Employment termination costs	$	$ 1,227	$ (88)	$	$ 1,139
Facilities consolidation		703			703
Property and equipment disposal		58			
Total	$	$ 1,988	$ (88)	$	$ 1,842

Employment termination costs will be paid within one year and 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, 2013 and there were no material recognized or non-recognized subsequent events during this period.

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

	Quarters Ended			
	March 31	June 30	September 30	December 31
2012				
Net loss	(13,137)	(9,343)	(16,290)	(10,015)
Basic and diluted net loss per common share [1]	(0.35)	(0.20)	(0.34)	(0.20)
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.

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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, 2013. 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, 2013, 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, 2013 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.

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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 "Executive Officers."

In 2007, we adopted a Code of Business Ethics and Conduct for Employees, Executive Officers and Directors that applies to our employees, officers and directors and incorporate guidelines designed to deter wrongdoing and to promote the honest and ethical conduct and compliance with applicable laws and regulations. In addition, the code of ethics incorporates our guidelines pertaining to topics such as conflicts of interest and workplace behavior. We have posted the text of our code on our website at *www.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 "Executive Compensation Compensation Discussion and Analysis."

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

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

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

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

Item 14. *PRINCIPAL ACCOUNTING FEES AND SERVICES.*

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

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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*

		Incorporated by Reference to SEC Filing			
Exhibit No.	**Filed Exhibit Description**	**Form**	**Date**	**Exhibit No.**	**Filed with this Form 10-K**
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	
4.3	Form of Warrant	Form 8-K	2/25/10	4.1	
10.1	2002 Equity Incentive Plan, as amended, and forms of option agreements thereunder	S-1/A (333-141700)	4/27/07	10.1	
+10.2	Amended and Restated License Agreement, dated October, 31, 2008, by and between the Registrant and Mount Sinai School of Medicine of New York University	Form 10-K	2/6/09	10.3	
+10.3	License Agreement, dated as of June 26, 2003, by and between the Registrant and University of Maryland, Baltimore County, as amended	S-1 (333-141700)	3/30/07	10.4	

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Exhibit No.	Filed Exhibit Description	Incorporated by Reference to SEC Filing			Filed with this Form 10-K
		Form	Date	Exhibit No.	
+10.4	Exclusive License Agreement, dated as of June 8, 2005, by and between the Registrant and Novo Nordisk, A/S	S-1 (333-141700)	3/30/07	10.5	
10.6	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	
10.7	Form of Director and Officer Indemnification Agreement	S-1 (333-141700)	3/30/07	10.17	
10.8	Restricted Stock Agreement, dated as of March 8, 2007, by and between the Registrant and Glenn P. Sblendorio	S-1/A (333-141700)	4/27/07	10.21	
10.9	Lease Agreement, dated as of July 31, 2006, by and between the Registrant and Cedar Brook II Corporate Center, L.P.	S-1/A (333-141700)	4/27/07	10.22	
10.10	Amended and Restated 2007 Director Option Plan and form of option agreement	Form 8-K Current Report	6/18/10	10.2	
10.11	2007 Employee Stock Purchase Plan	S-1/A (333-141700)	5/17/07	10.24	
10.12	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	
+10.13	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	
10.14	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	
10.15	Letter Agreement, dated as of December 30, 2008, by and between the Registrant and Bradley L. Campbell	Form 10-K	2/6/09	10.26	
10.16	Letter Agreement, dated as of December 30, 2008, by and between the Registrant and S. Nicole Schaeffer	Form 10-K	2/6/09	10.28	

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Exhibit No.	Filed Exhibit Description	Incorporated by Reference to SEC Filing			Filed with this Form 10-K
		Form	Date	Exhibit No.	
10.17	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	
10.18	Letter Agreement, dated as of December 30, 2008, by and between the Registrant and Geoffrey P. Gilmore	Form 10-K	2/6/09	10.31	
10.19	Summary Management Bonus Program				X
10.20	First Amendment to Lease Agreement dated June 11, 2009 between the Registrant and Cedar Brook 5 Corporate Center, L.P.	Form 10-Q	8/6/09	10.1	
+10.21	License and Collaboration Agreement dated as of October 28, 2010 by and between the Registrant and Glaxo Group Limited	Form 10-K	3/4/11	10.30	
+10.22	Stock Purchase Agreement dated as of October 28, 2010 by and between the Registrant and Glaxo Group Limited	Form 10-K	3/4/11	10.31	
10.23	Letter Agreement, dated as of May 10, 2010 by and between the Registrant and Ken Valenzano	Form 10-K	3/4/11	10.32	
10.24	Letter Agreement, dated as of January 3, 2011 by and between the Registrant and Kenneth Peist	Form 10-K	3/4/11	10.33	
10.25	Letter Agreement, dated as of January 3, 2011 by and between the Registrant and Enrique Dilone	Form 10-K	3/4/11	10.34	
10.26	Letter Agreement dated April 18, 2011 between the Registrant. and Matthew R. Patterson	Form 8-K	4/18/11	10.1	
10.27	Restricted Stock Award Agreement dated April 18, 2011 between the Registrant and Matthew R. Patterson	Form 8-K	4/18/11	10.2	
10.28	Amicus Therapeutics, Inc. 2007 Amended and Restated Equity Incentive Plan	Form 8-K	5/25/11	10.1	

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Exhibit No.	Filed Exhibit Description	Incorporated by Reference to SEC Filing			Filed with this Form 10-K
		Form	Date	Exhibit No.	
10.29	Employment Agreement, dated as of June 28, 2011, by and between the Registrant and John F. Crowley	Form 8-K	6/30/11	10.1	
10.30	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	
10.31	Letter Agreement dated March 5, 2012 between the Registrant and William D. Baird, III	Form 8-K	4/16/12	10.1	
+10.32	Amended and Restated License and Expanded Collaboration Agreement dated as of July 17, 2012 by and between the Registrant and Glaxo Group Limited	Form 10-Q	11/5/12	10.1	
+10.32	Stock Purchase Agreement dated as of July 17, 2012 by and between the Registrant and Glaxo Group Limited	Form 10-Q	11/5/12	10.1	
10.33	Amendment to Employment Agreement dated April 18, 2013 between Amicus Therapeutics, Inc and John F. Crowley	Form 8-K	4/24/13	10.1	
10.34	Letter Agreement dated April 18, 2013 between Amicus Therapeutics, Inc. and William D. Baird, III	Form 8-K	4/24/13	10.2	
10.35	Letter Agreement dated April 18, 2013 between Amicus Therapeutics, Inc. and Bradley L. Campbell	Form 8-K	4/24/13	10.3	
10.36	Letter Agreement dated April 18, 2013 between Amicus Therapeutics, Inc. and David J. Lockhart	Form 8-K	4/24/13	10.4	
10.37	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	
10.38	Letter Agreement, dated as of April 18, 2013 by and between the Registrant and Joan C. Winterbottom	Form 10-Q	8/7/13	10.5	

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Exhibit No.	Filed Exhibit Description	Incorporated by Reference to SEC Filing			Filed with this Form 10-K
		Form	Date	Exhibit No.	
10.39	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	
10.40	Letter Agreement, dated as of June 5, 2013 by and between the Registrant and Jayne Gershkowitz	Form 10-Q	8/7/13	10.7	
10.41	Letter Agreement, dated as of June 5, 2013 by and between the Registrant and Peter M. Macaluso	Form 10-Q	8/7/13	10.8	
10.42	Letter Agreement, dated November 20, 2013 by and among the Company and the purchasers identified therein	Form 8-K	11/20/13	10.1	
10.43	Form of Warrant issued on November 20, 2013	Form 8-K	11/20/13	10.2	
10.44	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	
10.45	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	
+10.46	Second Restated Agreement, dated November 19, 2013 by and between Amicus Therapeutics, Inc. and Glaxo Group Limited				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

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Exhibit No.	Filed Exhibit Description	Incorporated by Reference to SEC Filing			Filed with this Form 10-K
		Form	Date	Exhibit No.	
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, 2013, formatted in XBRL (Extensible Business Reporting Language) and filed electronically herewith: (i) the Consolidated Balance Sheets as of December 31, 2013 and December 31, 2012; (ii) the Consolidated Statements of Operations for the years ended December 31, 2011, 2012 and 2013; (iii) the Consolidated Statements of Comprehensive Loss for the years ended December 31, 2011, 2012 and 2013; (iv) the Consolidated Statements of Cash Flows for the years ended December 31, 2011, 2012 and 2013; (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.1

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SIGNATURES

Pursuant to the requirements of Section 13 or 15 (d) of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized on March 3, 2014.

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	Chairman and Chief Executive Officer (Principal Executive Officer)	March 3, 2014
(John F. Crowley)		
/s/ William D. Baird III	Chief Financial Officer (Principal Financial Officer)	March 3, 2014
(William D. Baird III)		
/s/ Daphne Quimi	Vice President, Finance and Controller (Principal Accounting Officer)	March 3, 2014
(Daphne Quimi)		
/s/ Sol J. Barer, Ph.D.	Director	March 3, 2014
(Sol J. Barer, Ph.D.)		
/s/ James Barrett	Director	March 3, 2014
(James Barrett)		
/s/ Robert Essner	Director	March 3, 2014
(Robert Essner)		
/s/ Donald J. Hayden	Director	March 3, 2014
(Donald J. Hayden)		

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Signature	Title	Date
/s/ Ted W. Love, M.D.	Director	March 3, 2014
(Ted W. Love, M.D.)		
/s/ Margaret G. McGlynn, R.Ph.	Director	March 3, 2014
(Margaret G. McGlynn, R.Ph.)		
/s/ Michael G. Raab	Director	March 3, 2014
(Michael G. Raab)		
/s/ Glenn Sblendorio	Director	March 3, 2014
(Glenn Sblendorio)		
/s/ James N. Topper, M.D., Ph.D.	Director	March 3, 2014
(James N. Topper, M.D., Ph.D.)		

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Exhibit No.	Filed Exhibit Description	Incorporated by Reference to SEC Filing			Filed with this Form 10-K
		Form	Date	Exhibit No.	
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	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	
4.3	Form of Warrant	Form 8-K	2/25/10	4.1	
10.1	2002 Equity Incentive Plan, as amended, and forms of option agreements thereunder	S-1/A (333-141700)	4/27/07	10.1	
+10.2	Amended and Restated License Agreement, dated October, 31, 2008, by and between the Registrant and Mount Sinai School of Medicine of New York University	Form 10-K	2/6/09	10.3	
+10.3	License Agreement, dated as of June 26, 2003, by and between the Registrant and University of Maryland, Baltimore County, as amended	S-1 (333-141700)	3/30/07	10.4	
+10.4	Exclusive License Agreement, dated as of June 8, 2005, by and between the Registrant and Novo Nordisk, A/S	S-1 (333-141700)	3/30/07	10.5	
10.5	Sublease Agreement, dated as of May 12, 2005, by and between the Registrant and Purdue Pharma, L.P.	S-1 (333-141700)	3/30/07	10.6	
10.6	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	
10.7	Form of Director and Officer Indemnification Agreement	S-1 (333-141700)	3/30/07	10.17	
10.8	Restricted Stock Agreement, dated as of March 8, 2007, by and between the Registrant and Glenn P. Sblendorio	S-1/A (333-141700)	4/27/07	10.21	

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Exhibit No.	Filed Exhibit Description	Incorporated by Reference to SEC Filing			Filed with this Form 10-K
		Form	Date	Exhibit No.	
10.9	Lease Agreement, dated as of July 31, 2006, by and between the Registrant and Cedar Brook II Corporate Center, L.P.	S-1/A (333-141700)	4/27/07	10.22	
10.10	Amended and Restated 2007 Director Option Plan and form of option agreement	Form 8-K Current Report	6/8/10 5/17/07	10.2 10.24	
10.11	2007 Employee Stock Purchase Plan	S-1/A (333-141700)			
10.12	Lease Agreement dated as of September 11, 2008 by and between the Registrant and A/G Touchstone, TP, LLC.	Form 8-K Current Report	9/15/08	10.1	
+10.13	First Amendment to lease dated April 15, 2011 by and between the Registrant and AG Touchstone, TP, LLC Pharmaceuticals Ireland, Ltd.	Form 10-K	2/28/12	10.13	
10.14	Letter Agreement, dated as of December 30, 2008, by and between the Registrant and David Lockhart, Ph.D.	Form 8-K Current Report	12/31/08	10.4	
10.15	Letter Agreement, dated as of December 30, 2008, by and between the Registrant and Bradley L. Campbell	Form 10-K	2/6/09	10.26	
10.16	Letter Agreement, dated as of December 30, 2008, by and between the Registrant and S. Nicole Schaeffer	Form 10-K	2/6/09	10.28	
10.17	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	
10.18	Letter Agreement, dated as of December 30, 2008, by and between the Registrant and Geoffrey P. Gilmore	Form 10-K	2/6/09	10.31	
10.19	Summary Management Bonus Program				X
10.20	First Amendment to Lease Agreement dated June 11, 2009 between the Registrant and Cedar Brook 5 Corporate Center, L.P.	Form 10-Q	8/6/09	10.1	

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Exhibit No.	Filed Exhibit Description	Incorporated by Reference to SEC Filing			Filed with this Form 10-K
		Form	Date	Exhibit No.	
+10.21	License and Collaboration Agreement dated as of October 28, 2010 by and between the Registrant and Glaxo Group Limited	Form 10-K	3/4/11	10.30	
+10.22	Stock Purchase Agreement dated as of October 28, 2010 by and between the Registrant and Glaxo Group Limited	Form 10-K	3/4/11	10.31	
10.23	Letter Agreement, dated as of May 10, 2010 by and between the Registrant and Ken Valenzano	Form 10-K	3/4/11	10.32	
10.24	Letter Agreement, dated as of January 3, 2011 by and between the Registrant and Kenneth Peist	Form 10-K	3/4/11	10.33	
10.25	Letter Agreement, dated as of January 3, 2011 by and between the Registrant and Enrique Dilone	Form 10-K	3/4/11	10.34	
10.26	Letter Agreement, dated as of April 18, 2011, between the Registrant and Matthew R. Patterson	Form 8-K	4/18/11	10.1	
10.27	Restricted Stock Award Agreement dated as of April 18, 2011, between the Registrant and Matthew R. Patterson	Form 8-K	4/18/11	10.2	
10.28	Amicus Therapeutics, Inc. 2007 Amended and Restated Equity Incentive Plan	Form 8-K	5/25/11	10.1	
10.29	Employment Agreement, dated as of June 28, 2011, by and between the Registrant and John F. Crowley	Form 8-K	6/30/11	10.1	
10.30	Lease Agreement, dated as of August 16, 2011, between the Registrant and Cedar Brook 3 Corporate Center, L.P.	Form 8-K	8/16/11	10.1	
10.30	Letter Agreement dated March 5, 2012 between the Registrant and William D. Baird, III	Form 8-K	4/16/12	10.1	
+10.31	Amended and Restated License and Expanded Collaboration Agreement dated as of July 17, 2012 by and between the Registrant and Glaxo Group Limited	Form 10-Q	11/5/12	10.1	

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Exhibit No.	Filed Exhibit Description	Incorporated by Reference to SEC Filing			Filed with this Form 10-K
		Form	Date	Exhibit No.	
+10.32	Stock Purchase Agreement dated as of July 17, 2012 by and between the Registrant and Glaxo Group Limited	Form 10-Q	11/5/12	10.2	
10.33	Amendment to Employment Agreement dated April 18, 2013 between Amicus Therapeutics, Inc and John F. Crowley	Form 8-K	4/24/13	10.1	
10.34	Letter Agreement dated April 18, 2013 between Amicus Therapeutics, Inc. and William D. Baird, III	Form 8-K	4/24/13	10.2	
10.35	Letter Agreement dated April 18, 2013 between Amicus Therapeutics, Inc. and Bradley L. Campbell	Form 8-K	4/24/13	10.3	
10.36	Letter Agreement dated April 18, 2013 between Amicus Therapeutics, Inc. and David J. Lockhart	Form 8-K	4/24/13	10.4	
10.37	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	
10.38	Letter Agreement, dated as of April 18, 2013 by and between the Registrant and Joan C. Winterbottom	Form 10-Q	8/7/13	10.5	
10.39	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	
10.40	Letter Agreement, dated as of June 5, 2013 by and between the Registrant and Jayne Gershkowitz	Form 10-Q	8/7/13	10.7	
10.41	Letter Agreement, dated as of June 5, 2013 by and between the Registrant and Peter M. Macaluso	Form 10-Q	8/7/13	10.8	
10.42	Letter Agreement, dated November 20, 2013 by and among the Company and the purchasers identified therein	Form 8-K	11/20/13	10.1	
10.43	Form of Warrant issued on November 20, 2013	Form 8-K	11/20/13	10.2	

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Exhibit No.	Filed Exhibit Description	Incorporated by Reference to SEC Filing			Filed with this Form 10-K
		Form	Date	Exhibit No.	
10.44	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	
10.45	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	
+10.46	Second Restated Agreement, dated November 19, 2013 by and between Amicus Therapeutics, Inc. and Glaxo Group Limited				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

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Exhibit No.	Filed Exhibit Description	Incorporated by Reference to SEC Filing			Filed with this Form 10-K
		Form	Date	Exhibit No.	
101	The following financial information from this Annual Report on Form 10-K for the year ended December 31, 2013, formatted in XBRL (Extensible Business Reporting Language) and filed electronically herewith: (i) the Consolidated Balance Sheets as of December 31, 2013 and December 31, 2012; (ii) the Consolidated Statements of Operations for the years ended December 31, 2011, 2012 and 2013; (iii) the Consolidated Statements of Comprehensive Loss for the years ended December 31, 2011, 2012 and 2013; (iv) the Consolidated Statements of Cash Flows for the years ended December 31, 2011, 2012 and 2013; (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.