UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-Q

(Mark One)

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

For the quarterly period ended June 30, 2012

OR

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

For the transition period from to

Commission file number 001-33497

Amicus Therapeutics, Inc.

(Exact Name of Registrant as Specified in Its Charter)

Delaware	**71-0869350**
(State or Other Jurisdiction of Incorporation or Organization)	(I.R.S. Employer Identification Number)

1 Cedar Brook Drive, Cranbury, NJ 08512
(Address of Principal Executive Offices and Zip Code)

Registrant's Telephone Number, Including Area Code: **(609) 662-2000**

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 x 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 x No o

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a 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. (Check one):

Large accelerated filer o	Accelerated filer o
Non-accelerated filer o	Smaller Reporting Company x

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

The number of shares outstanding of the registrant's common stock, $.01 par value per share, as of August 2, 2012 was 49,339,581 shares.

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AMICUS THERAPEUTICS, INC

Form 10-Q for the Quarterly Period Ended June 30, 2012

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We have filed applications to register certain trademarks in the United States and abroad, including AMICUS™ and AMICUS THERAPEUTICS™ (and design).

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SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

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

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

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

We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make. We have included important factors in the cautionary statements included in Part II Item 1A — "Risk Factors" of the Quarterly Report on Form 10-Q for the quarter ended June 30, 2012 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 quarterly report on Form 10-Q in conjunction with the documents that we reference herein. We do not assume any obligation to update any forward-looking statements.

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PART I. FINANCIAL INFORMATION

Item 1. Financial Statements (unaudited)

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

	December 31, 2011	June 30, 2012
Assets:		
Current assets:		
Cash and cash equivalents	$ 25,668	$ 28,858
Investments in marketable securities	30,034	66,914
Receivable due from GSK	5,043	7,237
Prepaid expenses and other current assets	5,903	2,658
Total current assets	66,648	105,667
Property and equipment, less accumulated depreciation and amortization of $9,507 and $7,658 at December 31, 2011 and June 30, 2012, respectively	2,438	5,489
Other non-current assets	709	442
Total Assets	$ 69,795	$ 111,598
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable and accrued expenses	$ 9,708	$ 9,067
Current portion of deferred revenue	8,504	7,929
Current portion of secured loan	1,044	816
Total current liabilities	19,256	17,812
Deferred revenue, less current portion	18,999	15,679
Warrant liability	1,948	4,442
Secured loan, less current portion	—	498
Commitments and contingencies		
Stockholders' equity:		
Common stock, $.01 par value, 125,000,000 shares authorized, 34,654,206 shares issued and outstanding at December 31, 2011, 125,000,000 shares authorized, 46,377,897 shares issued and outstanding at June 30, 2012	407	524
Additional paid-in capital	299,285	365,210
Accumulated other comprehensive income	4	17
Deficit accumulated during the development stage	(270,104)	(292,584)
Total stockholders' equity	29,592	73,167
Total Liabilities and Stockholders' Equity	$ 69,795	$ 111,598

See accompanying notes to consolidated financial statements

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

	Three Months Ended June 30,		Six Months Ended June 30,		Period from February 4, 2002 (inception) to June 30, 2012
	2011	2012	2011	2012	
Revenue:					

Research revenue	$ 2,380	$ 5,477	$ 6,686	$ 11,591	$ 57,493
Collaboration and milestone revenue	1,660	5,160	3,320	6,820	64,382
Total revenue	$ 4,040	$ 10,637	$ 10,006	$ 18,411	$ 121,875
Operating Expenses:					
Research and development	$ 11,618	$ 13,723	$ 22,743	$ 27,727	$ 293,347
General and administrative	6,720	5,819	11,122	9,914	123,163
Restructuring charges	—	—	—	—	1,522
Impairment of leasehold improvements	—	—	—	—	1,030
Depreciation and amortization	426	442	864	862	10,925
In-process research and development	—	—	—	—	418
Total operating expenses	18,764	19,984	34,729	38,503	430,405
Loss from operations	(14,724)	(9,347)	(24,723)	(20,092)	(308,530)
Other income (expenses):					
Interest income	46	116	105	143	14,216
Interest expense	(41)	(15)	(89)	(58)	(2,391)
Change in fair value of warrant liability	2,078	(118)	(1,354)	(2,494)	(1,594)
Other income	—	21	70	21	252
Loss before tax benefit	(12,641)	(9,343)	(25,991)	(22,480)	(298,047)
Benefit from income taxes	—	—	—	—	5,463
Net loss	(12,641)	(9,343)	(25,991)	(22,480)	(292,584)
Deemed dividend	—	—	—	—	(19,424)
Preferred stock accretion	—	—	—	—	(802)
Net loss attributable to common stockholders	$ (12,641)	$ (9,343)	$ (25,991)	$ (22,480)	$ (312,810)
Net loss attributable to common stockholders per common shares — basic and diluted	$ (0.37)	$ (0.20)	$ (0.75)	$ (0.53)	
Weighted-average common shares outstanding — basic and diluted	34,530,693	46,870,067	34,514,947	42,103,642	

See accompanying notes to consolidated financial statements

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

	Three Months Ended June 30,		Six Months Ended June 30,		Period from February 4, 2002 (inception) to June 30, 2012
	2011	2012	2011	2012	
Net loss	$ (12,641)	$ (9,343)	$ (25,991)	$ (22,480)	$ (292,584)
Other comprehensive income/(loss):					
Unrealized gain (loss) on available-for-sale securities	29	(20)	29	13	17
Other comprehensive income/(loss), before income taxes	29	(20)	29	13	17
Provision for income taxes related to other comprehensive income/(loss) items (Note 1)	—	—	—	—	—
Other comprehensive income/(loss)	$ 29	$ (20)	$ 29	$ 13	$ 17
Comprehensive loss	$ (12,612)	$ (9,363)	$ (25,962)	$ (22,467)	$ (292,567)

Note 1 — Taxes have not been accrued on unrealized gain on securities as the Company is in a loss position for all periods presented.

See accompanying notes to consolidated financial statements

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

(in thousands)

	Six Months Ended June 30,		Period from February 4, 2002 (inception) to June 30,
	2011	2012	2012
Operating activities			
Net loss	$ (25,991)	$ (22,480)	$ (292,584)
Adjustments to reconcile net loss to net cash used in operating activities:			
Non-cash interest expense	—	—	525
Depreciation and amortization	864	862	10,925
Amortization of non-cash compensation	—	—	522
Stock-based compensation - employees	5,302	3,145	38,883
Stock-based compensation - non-employees	—	—	853
Stock-based license payments	—	—	1,220
Change in fair value of warrant liability	1,354	2,494	1,594
Loss on disposal of asset	—	27	387
Impairment of leasehold improvements	—	—	1,030
Non-cash charge for in-process research and development	—	—	418
Debt instrument convertible beneficial conversion feature	—	—	135
Changes in operating assets and liabilities:			
Receivable due from GSK	(1,977)	(2,194)	(7,237)
Prepaid expenses and other current assets	(840)	3,245	(2,658)
Other non-current assets	—	267	(463)
Accounts payable and accrued expenses	(1,271)	(641)	9,067
Deferred revenue	(1,354)	(3,895)	23,608
Net cash used in operating activities	(23,913)	(19,170)	(213,775)
Investing activities			
Sale and redemption of marketable securities	57,224	34,839	706,927
Purchases of marketable securities	(36,594)	(71,706)	(773,942)
Purchases of property and equipment	(182)	(3,939)	(17,829)
Net cash provided by/(used in) investing activities	20,448	(40,806)	(84,844)
Financing activities			
Proceeds from the issuance of preferred stock, net of issuance costs	—	—	143,022
Proceeds from the issuance of common stock and warrants, net of issuance costs	—	62,057	175,303
Proceeds from the issuance of convertible notes	—	—	5,000
Payments of capital lease obligations	(40)	—	(5,587)
Payments of secured loan agreement	(626)	(726)	(3,440)
Proceeds from exercise of stock options	348	840	2,551
Proceeds from exercise of warrants (common and preferred)	—	—	264
Proceeds from capital asset financing arrangement	—	—	5,611
Proceeds from secured loan agreement	—	995	4,753
Net cash (used in) /provided by financing activities	(318)	63,166	327,477
Net (decrease) increase in cash and cash equivalents	(3,783)	3,190	28,858
Cash and cash equivalents at beginning of period	29,572	25,668	—
Cash and cash equivalents at end of period	$ 25,789	$ 28,858	$ 28,858

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

	Six Months Ended June 30,		Period from February 4, 2002 (inception) to June 30,
	2011	2012	2012
Supplemental disclosures of cash flow information			
Cash paid during the period for interest	$ 89	$ 50	$ 2,083
Non-cash activities			
Conversion of notes payable to preferred stock	$ —	$ —	$ 5,000
Conversion of preferred stock to common stock	$ —	$ —	$ 148,951
Accretion of redeemable convertible preferred stock	$ —	$ —	$ 802
Beneficial conversion feature related to the issuance of Series C redeemable convertible preferred stock	$ —	$ —	$ 19,424

See accompanying notes to consolidated financial statements

Note 1. Description of Business and Significant Accounting Policies

Corporate Information, Status of Operations and Management Plans

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

On July 17, 2012, the Company entered into an Amended and Restated License and Expanded Collaboration Agreement (the "Expanded Collaboration Agreement") with GlaxoSmithKline PLC (GSK) pursuant to which the Company and GSK will continue to develop and commercialize migalastat HCI, currently in Phase 3 development for the treatment of Fabry disease. The Expanded Collaboration Agreement amends and replaces in its entirety the License and Collaboration Agreement entered into between the Company and GSK on October 28, 2010 (the "Original Collaboration Agreement") for the development and commercialization of migalastat HCl. Under the terms of the Expanded Collaboration Agreement, the Company and GSK will 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") in collaboration with another GSK collaborator JCR Pharmaceutical Co., Ltd. The Company will commercialize all migalastat HCl products for Fabry disease in the United States while GSK will commercialize all such products in the rest of the world.

GSK is eligible to receive U.S. regulatory approval milestones totaling $20 million for migalastat HCl monotherapy and migalastat HCl for co-administration with ERT, and additional regulatory approval and product launch milestone payments totaling up to $35 million within seven years following the launch of the Co-formulated Product. The Company will also be responsible for certain pass-through milestone payments and single-digit royalties on the net U.S. sales of the Co-formulated Product that GSK must pay to a third party. In addition, the Company is no longer eligible to receive any milestones or royalties it would have been eligible to receive under the Original Collaboration Agreement other than a $3.5 million clinical development milestone achieved in the second quarter of 2012 and expected to be paid by GSK to Amicus in the third quarter of 2012.

The Company and GSK will continue to jointly fund development costs for all formulations of migalastat HCl in accordance with agreed upon development plans pursuant to which the Company and GSK will fund 25% and 75% of such costs, respectively, for the monotherapy and co-administration development of migalastat HCl for the remainder of 2012 and 40% and 60%, respectively, thereafter. Effective upon entry into the Expanded Collaboration Agreement, costs for the development of the Co-formulated Product are also split 40% and 60% between Amicus and GSK, respectively.

Additionally, simultaneous with entry into the Expanded Collaboration Agreement, the Company and GSK entered into a Stock Purchase Agreement (the "SPA") pursuant to which GSK will purchase approximately 2.9 million shares of Amicus common stock at a price of $6.30 per share. The total value of this equity investment to the Company is approximately $18.6 million and increases GSK's ownership position in the Company to 19.9%. GSK purchased approximately 6.9 million shares for an aggregate investment of approximately $31 million in connection with entry into the Original Collaboration Agreement in 2010.

For further information, see "— Note 7. Collaborative Agreements" and "— Note 9. Subsequent Events."

The Company had an accumulated deficit of approximately $292.6 million at June 30, 2012 and anticipates incurring losses through the year 2012 and beyond. The Company has not yet generated commercial sales revenue and has been able to fund its operating losses to date through the sale of its redeemable convertible preferred stock, issuance of convertible notes, net proceeds from our initial public offering (IPO) and subsequent stock offerings, payments from partners during the terms of collaboration agreements and other financing arrangements. In March 2010, the Company sold 4.95 million shares of its common stock and warrants to purchase 1.85 million shares of common stock in a registered direct offering to a select group of institutional investors for net proceeds of $17.1

million. In October 2010, the Company sold 6.87 million shares of its common stock as part of the Original Collaboration Agreement with GSK for proceeds of $31 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. The Company believes that its existing cash and cash equivalents and short-term investments will be sufficient to cover its cash flow requirements for 2012.

Basis of Presentation

The Company has prepared the accompanying unaudited consolidated financial statements in accordance with accounting principles generally accepted in the United States of America (U.S. GAAP) for interim financial information and with the instructions to Form 10-Q and Article 10-01 of Regulations S-X. Accordingly, they do not include all of the information and disclosures required by generally accepted accounting principles for complete financial statements. In the opinion of management, the accompanying unaudited financial statements reflect all adjustments, which include only normal recurring adjustments, necessary to present fairly the Company's interim financial information.

The accompanying unaudited consolidated financial statements and related notes should be read in conjunction with the Company's financial statements and related notes as contained in the Company's Annual Report on Form 10-K for the year ended December 31, 2011. For a complete description of the Company's accounting policies, please refer to the Annual Report on Form 10-K for the fiscal year ended December 31, 2011.

Revenue Recognition

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

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

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

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

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

Fair Value Measurements

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

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

New Accounting Standards

In June 2011, the Financial Accounting Standards Board (FASB) amended its guidance on the presentation of comprehensive income in financial statements to improve the comparability, consistency and transparency of financial reporting and to increase the prominence of items that are recorded in other comprehensive income. The new accounting guidance requires entities to report components of comprehensive income in either (1) a continuous statement of comprehensive income or (2) two separate but consecutive statements. The provisions of this guidance are effective for fiscal years, and interim periods within those years, beginning after December 15, 2011. Other than a change in presentation, the implementation of this accounting pronouncement did not have a material impact on our financial statements.

In May 2011, the FASB amended the FASB Accounting Standards Codification to converge the fair value measurement guidance in U.S. GAAP and International Financial Reporting Standards. Some of the amendments clarify the application of existing fair value measurement requirements, while other amendments change particular principles in fair value measurement guidance. In addition, the amendments require additional fair value disclosures. The amendments are effective for fiscal year 2012 and should be applied prospectively. The Company is currently evaluating the impact, if any, that the provisions of the amendments will have on its consolidated results of operations or financial position.

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Note 2. Cash, Cash Equivalents and Available-for-Sale Investments

As of June 30, 2012, the Company held $28.9 million in cash and cash equivalents and $66.9 million of available-for-sale investment securities which are reported at fair value on the Company's balance sheet. Unrealized holding gains and losses are reported within accumulated other comprehensive income/(loss) as a separate component of stockholders' equity. If a decline in the fair value of a marketable security below the Company's cost basis is determined to be other than temporary, such marketable security is written down to its estimated fair value as a new cost basis and the amount of the write-down is included in earnings as an impairment charge. To date, only temporary impairment adjustments have been recorded.

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

Cash and available for sale securities consisted of the following as of December 31, 2011 and June 30, 2012 (in thousands):

	As of December 31, 2011			
	Cost	Unrealized Gain	Unrealized Loss	Fair Value
Cash balances	$ 25,668	$ —	$ —	$ 25,668
U.S. government agency securities	2,000	—	—	2,000
Corporate debt securities	13,943	—	(8)	13,935
Commercial paper	13,737	12	—	13,749
Certificate of deposit	350	—	—	350
	$ 55,698	$ 12	$ (8)	$ 55,702
Included in cash and cash equivalents	$ 25,668	$ —	$ —	$ 25,668
Included in marketable securities	30,030	12	(8)	30,034
Total cash and available for sale securities	$ 55,698	$ 12	$ (8)	$ 55,702

	As of June 30, 2012			
	Cost	Unrealized Gain	Unrealized Loss	Fair Value
Cash balances	$ 28,858	$ —	$ —	$ 28,858
Corporate debt securities	41,602	1	(28)	41,575
Commercial paper	24,945	44	—	24,989
Certificate of deposit	350	—	—	350
	$ 95,755	$ 45	$ (28)	$ 95,772
Included in cash and cash equivalents	$ 28,858	$ —	$ —	$ 28,858
Included in marketable securities	66,897	45	(28)	66,914
Total cash and available for sale securities	$ 95,755	$ 45	$ (28)	$ 95,772

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Unrealized gains and losses are reported as a component of accumulated other comprehensive income/(loss) in stockholders' equity. For the year ended December 31, 2011, unrealized holding gains included in accumulated other comprehensive income was $4 thousand. For the six months ended June 30, 2012, unrealized holding gains included in accumulated other comprehensive income was $13 thousand.

For the year ended December 31, 2011 and the six months ended June 30, 2012, 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, 2011 and June 30, 2012 reflect temporary impairments that have not been recognized and have been in a loss position for less than twelve months. The fair value of these available for sale securities in unrealized loss positions was $13.2 million and $37.2 million as of December 31, 2011 and June 30, 2012, respectively.

Note 3. Basic and Diluted Net Loss Attributable to Common Stockholders per Common Share

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

	Three Months Ended June 30,		Six Months Ended June 30,	
(In thousands, except per share amounts)	2011	2012	2011	2012
Statement of Operations				
Net loss attributable to common stockholders	$ (12,641)	$ (9,343)	$ (25,991)	$ (22,480)
Net loss attributable to common stockholders per common share — basic and diluted	$ (0.37)	$ (0.20)	$ (0.75)	$ (0.53)

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 7.6 million and 9.1 million for the six months ended June 30, 2011 and 2012, respectively.

Potentially dilutive common stock equivalents were excluded from the diluted earnings per share denominator for all periods because of their anti-dilutive effect.

Note 4. Stockholders' Equity

Common Stock and Warrants

On July 17, 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 and increases GSK's ownership position in the Company to 19.9%. The Company received all proceeds from the sale of such shares on July 26, 2012.

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 on May 27, 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.

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In March 2010, the Company sold 4.9 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 on May 27, 2009. The shares of common stock and warrants were sold in units consisting of one share of common stock and one warrant to purchase 0.375 shares of common stock at a price of $3.74 per unit. The warrants have a term of four years and are exercisable any time on or after the six month anniversary of the date they were issued, at an exercise price of $4.43 per share. The aggregate offering proceeds were $18.5 million.

Stock Option Plans

During the three and six months ended June 30, 2012, the Company recorded compensation expense of approximately $1.8 million and $3.1 million, respectively. The stock-based compensation expense had no impact on the Company's cash flows from operations and financing activities. As of June 30, 2012, the total unrecognized compensation cost related to non-vested stock options granted was $9.6 million and is expected to be recognized over a weighted average period of 2.6 years.

The fair value of the options granted is estimated on the date of grant using a Black-Scholes option pricing model with the following weighted-average assumptions:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2011	2012	2011	2012
Expected stock price volatility	78.4%	77.0%	78.8%	77.5%
Risk free interest rate	2.1%	1.1%	2.2%	0.7%
Expected life of options (years)	6.25	6.25	6.25	6.25
Expected annual dividend per share	$ 0.00	$ 0.00	$ 0.00	$ 0.00

A summary of option activities related to the Company's stock options for the six months ended June 30, 2012 is as follows:

	Number of Shares (in thousands)	Weighted Average Exercise Price	Weighted Average Remaining Contractual Life	Aggregate Intrinsic Value (in millions)
Balance at December 31, 2011	6,653.5	$ 6.87		
Options granted	1,395.1	$ 6.15		
Options exercised	(223.7)	$ 3.77		
Options forfeited	(589.8)	$ 7.97		
Balance at June 30, 2012	7,235.1	$ 6.74	7.5 years	$ 3.5
Vested and unvested expected to vest, June 30, 2012	6,837.3	$ 6.79	7.4 years	$ 3.4
Exercisable at June 30, 2012	4,001.0	$ 7.59	6.3 years	$ 2.0

Note 5. Short-Term Borrowings and Long-Term Debt

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

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conditions, including a financial covenant whereby the Company 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 SVB.

In addition, the Company committed to a second loan and security agreement with SVB in August 2011 (the "2011 Loan Agreement") in order to finance certain capital expenditures 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 provides for up to $3 million of equipment financing through January 2014. Borrowings under the 2011 Loan Agreement are 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 the same financial covenants as the 2009 Loan Agreement. The Company has at all times been in compliance with these covenants during the term of both agreements.

At June 30, 2012, the total amount due under the 2009 Loan Agreement and the 2011 Loan Agreement was $1.3 million. The carrying amount of the Company's borrowings approximates fair value at June 30, 2012.

Note 6. 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 three months ended June 30, 2012. No transfers of assets between Level 1 and Level 2 of the fair value measurement hierarchy occurred during the six months ended June 30, 2012.

Secured Debt

As disclosed in Note 5, the Company has loan and security agreements with Silicon Valley Bank. The carrying amount of the Company's borrowings approximates fair value at June 30, 2012. 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.

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

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the basis for the fair value classification. There are six inputs: closing price of Amicus stock on the day of evaluation; the exercise price of the warrants; the remaining term of the warrants; the volatility of Amicus' stock over that term; annual rate of dividends; and the riskless rate of return. Of those inputs, the exercise price of the warrants and the remaining term are readily observable in the warrant agreements. The annual rate of dividends is based on the Company's historical practice of not granting dividends. The closing price of Amicus stock would fall under Level 1 of the fair value hierarchy as it is a quoted price in an active market. The riskless rate of return is a Level 2 input, while the historical volatility is a Level 3 input in accordance with the fair value accounting guidance. Since the lowest level input is a Level 3, the Company determined the warrant liability is most appropriately classified within Level 3 of the fair value hierarchy. This liability is subject to fair value mark-to-market adjustment each period. As a result, the Company recognized the change in the fair value of the warrant liability as non-operating expense of $0.1 million and $2.5 million for the three and six months ended June 30, 2012, respectively. The resulting fair value of the warrant liability at June 30, 2012 was $4.4 million. The weighted average assumptions used in the Black-Scholes valuation model for the warrants as of December 31, 2011 and June 30, 2012 are as follows:

	December 31, 2011	June 30, 2012
Expected stock price volatility	67.3%	73.27%
Risk free interest rate	0.28%	0.29%
Expected life of warrants (years)	2.17	1.67
Expected annual dividend per share	$ 0.00	$ 0.00

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 June 30, 2012, are identified in the following table (in thousands):

	Balance as of December 31, 2011		
	Level 1	Level 2	Total
Assets:			
Cash/Money market funds	$ 25,668	$ —	$ 25,668
Corporate debt securities	—	2,000	2,000
Commercial paper	—	13,749	13,749
Corporate debt securities	—	13,935	13,935
Certificate of deposit	—	350	350

$ 25,668	$ 30,034	$ 55,702	

	Level 1	Level 2	Level 3	Total
Liabilities:				
Secured debt	$ —	$ 1,044	$ —	$ 1,044
Warrants liability	—	—	1,948	1,948
	$ —	$ 1,044	$ 1,948	$ 2,992

	Balance as of June 30, 2012		
	Level 1	Level 2	Total
Assets:			
Cash/Money market funds	$ 28,858	$ —	$ 28,858
Corporate debt securities	—	41,575	41,575
Commercial paper	—	24,989	24,989
Certificate of deposit	—	350	350
	$ 28,858	$ 66,914	$ 95,772

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	Level 1	Level 2	Level 3	Total
Liabilities:				
Secured debt	$ —	$ 1,314	$ —	$ 1,314
Warrants liability	—	—	4,442	4,442
	$ —	$ 1,314	$ 4,442	$ 5,756

The change in the fair value of the Level 3 liability was an increase of $0.1 million and a decrease of $2.1 million for the three months ended June 30, 2012, and 2011, respectively. The change in fair value for the Level 3 liability was an increase of $2.5 million and $1.4 million for the six months ended June 30, 2012 and 2011, respectively.

Note 7. Collaborative Agreements

GSK

On October 28, 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. 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 and represented a 14.8% ownership position in the Company as of June 30, 2012.

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

The total arrangement consideration which was allocated to the single unit of accounting identified above was $33.2 million which consists of the upfront license payment of $30 million and the premium over the closing market price of the common stock transaction of $3.2 million. The Company will recognize this consideration as Collaboration Revenue on a straight-line basis over the development period of 5.2 years as included in the detailed development plan that was included in the Original Collaboration Agreement. The Company determined that the overall level of activity over the development period approximates a straight-line approach. At June 30, 2012, the Company had recognized approximately $10.9 million of the total arrangement consideration as Collaboration Revenue since the inception of the agreement.

The Company evaluated the contingent milestones included in the collaboration agreement at the inception of the Original Collaboration Agreement and determined that the contingent milestones are substantive milestones and will be recognized as revenue in the period that the milestone is achieved. The Company determined that the research based milestones were commensurate with the enhanced value of each delivered item as a result of the

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Company's specific performance to achieve the milestones. There was considerable effort underway to meet the specified milestones and efforts continue to complete the development of migalastat HCl. Additionally, there is considerable time and effort involved in evaluating the data from the clinical trials that are planned and underway and if acceptable, in preparing the documentation required for filing for approval with the applicable regulatory authorities. The

research based milestones would have related to past performances when achieved and are reasonable relative to the other payment terms within the collaboration agreement, including the $30 million upfront payment and the cost sharing arrangement. In June 2012, the Company achieved a clinical development 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.

On July 17, 2012, the Company entered into the Expanded Collaboration Agreement with GSK pursuant to which the Company and GSK will continue to develop and commercialize migalastat HCl. The Expanded Collaboration Agreement amends and replaces in its entirety the Original Collaboration Agreement. For further information, see "— Note 9. Subsequent Events."

Note 8. Restructuring Charges

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

The following table summarizes the restructuring charges and utilization for the six months ended June 30, 2012 (in thousands):

	Balance as of December 31, 2011	Charges	Cash Payments	Adjustments	Balance as of June 30, 2012
Facilities consolidation	$ 38	—	$ (38)	—	$ —

Note 9. Subsequent Events

The Company evaluated events that occurred subsequent to June 30, 2012 through the date of issuance of these financial statements. The following events are noted:

Expanded GSK Collaboration Agreement

On July 17, 2012, the Company entered into the Expanded Collaboration Agreement with GSK pursuant to which the Company and GSK will continue to develop and commercialize migalastat HCl, currently in Phase 3 development for the treatment of Fabry disease. The Expanded Collaboration Agreement amends and replaces in its entirety the Original Collaboration Agreement entered into between the Company and GSK on October 28, 2010 for the development and commercialization of migalastat HCl. Under the terms of the Expanded Collaboration Agreement, the Company and GSK will co-develop all formulations of migalastat HCl for Fabry disease, including the development of the Co-formulated Product in collaboration with another GSK collaborator, JCR Pharmaceutical Co., Ltd. The Company will commercialize all migalastat HCl products for Fabry disease in the United States while GSK will commercialize all such products in the rest of the world. The exclusive license granted to GSK under the Original Collaboration Agreement to commercialize migalastat HCl worldwide is therefore replaced under the Expanded Collaboration Agreement with two exclusive licenses: (i) an exclusive license from GSK to Amicus to commercialize migalastat HCl in the United States, and (ii) an exclusive license from Amicus to GSK to commercialize migalastat HCl in the rest of world. GSK and Amicus each have a license to manufacture migalastat HCl for commercialization of monotherapy and chaperone-ERT co-administration migalastat HCl products while GSK maintains an exclusive license to manufacture such products for development purposes (subject to limited exceptions) and to manufacture the Co-formulated Product.

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GSK is eligible to receive U.S. regulatory approval milestones totaling $20 million for migalastat HCl monotherapy and chaperone-ERT co-administration, and additional regulatory approval and product launch milestone payments totaling up to $35 million within seven years following the launch of the Co-formulated Product. Amicus will also be responsible for certain pass-through milestone payments and single-digit royalties on the net U.S. sales of the Co-formulated Product that GSK must pay to a third party. In addition, Amicus is no longer eligible to receive any milestones or royalties it would have been eligible to receive under the Original Collaboration Agreement other than a $3.5 million clinical development milestone achieved in the second quarter of 2012 and expected to be paid by GSK to Amicus in the third quarter of 2012.

The Company and GSK will continue to jointly fund development costs for all formulations of migalastat HCl in accordance with agreed upon development plans pursuant to which Amicus and GSK will fund 25% and 75% of such costs, respectively, for the monotherapy and co-administration development of migalastat HCl for the remainder of 2012 and 40% and 60%, respectively, thereafter. Effective upon entry into the Expanded Collaboration Agreement, costs for the development of the Co-formulated Product are also split 40% and 60% between Amicus and GSK, respectively.

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. The SPA provides GSK with customary registration rights for the shares purchased and includes a six-month lock-up provision. The total purchase price was $18.6 million and the Company received all proceeds from the sale of such shares on July 26, 2012.

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ITEM 2. MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

Overview

Amicus Therapeutics, Inc. (Amicus) is a biopharmaceutical company focused on the discovery, development and commercialization of orally-administered, small molecule drugs known as pharmacological chaperones, a novel, first-in-class approach to treating a broad range of diseases including

lysosomal storage diseases and diseases of neurodegeneration. We believe that our pharmacological chaperone technology, our advanced product pipeline, especially our lead product candidate, migalastat HCl, and our strategic collaboration with GSK uniquely position us as a leader in the development of treatments for rare and orphan diseases.

We are focused on the development of pharmacological chaperone monotherapy programs and pharmacological chaperones in combination with enzyme replacement therapy (ERT), the current standard of treatment for Fabry and other lysosomal storage disease. In 2012, we are advancing two pharmacological chaperone monotherapy programs for genetic diseases:

- Migalastat HCl for patients with Fabry disease identified as having alpha-galactosidase A (alpha-Gal A) mutations amenable to chaperone therapy, and
- AT3375 for Parkinson's disease in Gaucher disease carriers and potentially the broader Parkinson's population.

Our pharmacological chaperone-ERT combination programs for 2012 include:

- Migalastat HCl co-administered with ERT for patients with Fabry disease receiving ERT treatment with any genetic mutation,
- Migalasat HCl co-formulated with a proprietary preclinical ERT,
- AT2220 (duvoglustat HCl) co-administered with ERT for Pompe disease,
- AT3375 and afegostat tartrate co-administered with ERT for Gaucher disease, and
- Several new, undisclosed pharmacological chaperone programs focused on the combination of chaperones with ERTs for additional lysosomal storage diseases.

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

Our lead product candidate, migalastat HCl for Fabry disease, is in late Phase 3 development. We are developing and commercializing migalastat HCl with an affiliate of GSK pursuant to the Expanded Collaboration Agreement entered into in July 2012. Our partnership with GSK allows us to utilize GSK's significant expertise in clinical, regulatory, commercial and manufacturing matters in the development in migalastat HCl. In addition, the cost-sharing arrangements under the Expanded Collaboration Agreement provide us with financial strength and allow us to continue the development of migalastat HCl while also advancing our other programs. We also believe this collaboration is important in validating our status as a leader in the development of treatments for rare diseases given the increasing focus placed on the rare disease field.

Our Phase 3 clinical development program for the use of migalastat HCl as monotherapy in Fabry disease includes two global registration studies for patients with Fabry disease identified as having alpha-Gal A mutations amenable to migalastat HCl: Study 011 and Study 012. We completed enrollment of 67 total patients in Study 011, our placebo-controlled Phase 3 study, in December 2011 and expect results in the fourth quarter of 2012. We

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plan to use the data from Study 011 to support marketing applications for the U.S. Food and Drug Administration (FDA) and other regulatory agencies. Study 012 is our second phase 3 study for migalastat HCl intended to support the worldwide registration of migalastat HCl for Fabry disease. We dosed the first patient in Study 012 in September 2011 to compare the safety and efficacy of migalastat HCl and ERT (agalsidase beta or agalsidase alfa) and expect to complete enrollment of approximately 50 patients by the end of 2012.

In addition to potential benefits pharmacological chaperones may provide as a monotherapy, we also believe the use of pharmacological chaperones co-administered and co-formulated with ERT may address certain key limitations of ERT. The use of pharmacological chaperones co-administered with ERT may significantly enhance the safety and efficacy of ERT by, among other effects, prolonging the half-life of infused enzymes in the circulation, increasing uptake of the active enzymes into cells and tissues, and increasing enzyme activity and substrate reduction in target tissues compared to that observed with ERT alone. We are evaluating the use of pharmacological chaperones co-administered with ERT in two Phase 2 clinical studies, one evaluating the use of migalastat HCl co-administered with ERT for Fabry disease (Study 013) and another evaluating the use of AT2220 co-administered with ERT for Pompe disease (Study 010).

We are also conducting preclinical studies with JCR Pharmaceutical Co., Ltd (JCR) evaluating migalastat HCl co-formulated with a proprietary recombinant human alpha-Gal A enzyme (JR-051). Preclinical studies conducted by Amicus, GSK and JCR suggest that this co-formulated chaperone-ERT product may provide greater alpha-Gal A enzyme uptake into tissue and markedly reduced levels of GL-3 in Fabry disease-relevant tissues compared to recombinant enzyme alone. Amicus and GSK believe that this co-formulated chaperone-ERT product for Fabry disease has the potential to enter clinical studies in 2013.

Amicus is also investigating chaperone-ERT combinations as potential next-generation treatments for Gaucher and other undisclosed lysosomal storage diseases where there are significant opportunities to improve treatment outcomes. In Gaucher disease, Amicus is continuing preclinical studies to evaluate two pharmacological chaperones, AT2101 (afegostat tartrate) and AT3375, in combination with ERT (beta-glucosidase). Both of these chaperones target the enzyme deficient in Gaucher disease.

Gaucher disease is caused by inherited genetic mutations in the GBA gene, and mutations in this gene that encodes for the GCase enzyme are the most common genetic risk factor for Parkinson's. By targeting GCase in the brain, AT3375 could potentially treat Gaucher, Parkinson's disease in Gaucher carriers, and possibly the general Parkinson's population.

We have generated significant losses to date and expect to continue to generate losses as we continue the clinical development of our drug candidates, including migalastat HCl, and conduct preclinical studies on other programs. These activities are budgeted to expand over time and will require further resources if we are to be successful. From our inception in February 2002 through June 30, 2012, we have accumulated a deficit of $292.6 million. As we have not yet generated commercial sales revenue from any of our product candidates, our losses will continue and are likely to be substantial in the near term.

Program Status

Migalastat HCl for Fabry Disease: Phase 3 Global Registration Program

We and our partner GSK are conducting two Phase 3 global registration studies (Study 011 and Study 012) to support the global approval of migalastat HCl monotherapy for the treatment of Fabry disease. Study 011 and Study 012 are investigating migalastat HCl at an oral dose of 150 mg, administered every-other-day (QOD) to Fabry patients identified as having alpha-Gal A mutations amenable to migalastat HCl as a monotherapy. Study 011 is a randomized, placebo-controlled study with a six-month, double-blind primary treatment period and a six-month, open-label follow-up period. The primary endpoint is based on interstitial capillary globotriaosylceramide (GL-3) as measured in kidney biopsy. The six-month primary treatment period was completed in a total of 63 patients during the second quarter 2012. These patients received kidney biopsies at baseline and month six. All 63 of these patients are continuing in the six-month follow-up period, and all of these patients are expected to have 12-month kidney biopsies by year-end 2012.

We and GSK have recently engaged in encouraging interactions with the FDA regarding the planned NDA for migalastat HCl. The agency indicated it would consider safety and efficacy data from both the six- and 12-month kidney biopsies to support conditional approval under subpart H. In order to preserve the integrity and availability of clinical data for the open-label follow-up period, we and GSK have jointly determined that the unblinding and analysis of the data from the primary six-month treatment arm will not occur prior to fourth quarter 2012. We and GSK remain blinded to the results at this time.

Study 012 is a randomized, open-label, 18-month Phase 3 study investigating the safety and efficacy of migalastat HCI compared to current standard-of-care ERTs Fabrazyme (agalsidase beta) or Replagal (agalsidase

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alfa) for Fabry disease. A majority of patients have been enrolled in this study, which is targeting approximately 50 total patients (30 to switch to migalastat HCl and 20 to remain on ERT). Study 012 is currently underway at 25 clinical sites worldwide, including U.S. sites that are now able to enroll patients who have resumed full-dose Fabrazyme. Amicus and GSK continue to anticipate that enrollment in this study will be completed by year-end 2012.

Phase 2 and Phase 3 extension studies continue to evaluate long-term safety with migalastat HCl monotherapy in Fabry patients. As of June 30, 2012, all patients who have completed the six-month treatment and six-month follow-up periods in Study 011 are currently enrolled in a Phase 3 extension study. An additional 17 subjects continue in the ongoing Phase 2 extension study and have been receiving migalastat HCl for up to six years.

We will lead all U.S. commercial activities for migalastat HCl upon approval, including pricing, matching, patient access and reimbursement.

Pharmacological Chaperone-ERT (PC-ERT) Co-Administration for Lysosomal Storage Diseases

Fabry Disease

Study 013 is an ongoing open-label Phase 2 study to investigate a single oral dose of migalastat HCl (150 mg or 450 mg) co-administered two hours prior to ERT (Fabrazyme or Replagal) in males diagnosed with Fabry disease. When co-administered with ERT, migalastat HCl is designed to bind to and stabilize the enzyme in the circulation, independent of alpha-Gal A mutation type.

Positive preliminary results from Study 013 were announced in the first quarter 2012 in patients who received migalastat HCl 150 mg co-administered with Fabrazyme (0.5 mg/kg or 1.0 mg/kg). We expect to present results at a Fall 2012 scientific conference. Both Amicus and GSK are committed to working together to advance this Fabry co-administration program, which has been recognized as having significant medical interest and importance. A repeat-dose global study of migalastat HCl co-administered with ERT is currently being designed as the next step in U.S. and global development. Following the withdrawal of the U.S. marketing application for Replagal, Fabrazyme remains the only ERT with conditional approval in the U.S.

Pompe Disease

Amicus is investigating four ascending doses of AT2220 co-administered with the ERT alglucosidase alfa in a Phase 2 open-label study (Study 010) for Pompe disease. Approximately 22 patients will receive one infusion of ERT alone, and a single oral dose of AT2220 prior to the next ERT infusion. In addition to safety and pharmacokinetic effects, Study 010 will measure uptake of active enzyme in muscle tissue with and without the chaperone, three or seven days following each infusion.

Positive preliminary results from Study 010 were announced in the second quarter 2012 in patients enrolled in the first two cohorts of the study at the lowest dose groups of AT2220. Previous preclinical studies using acid alpha-glucosidase (GAA) knock-out mouse models of Pompe disease demonstrated that AT2220 co-administered with ERT increased the ERT uptake in key tissues of disease, including skeletal muscle and heart. This increased ERT uptake into muscle following AT2220-ERT co-administration corresponded in preclinical studies with greater reductions in muscle glycogen compared to ERT alone. Glycogen is the substrate that accumulates in the lysosomes of muscles in patients with Pompe disease. We expect to present additional results at a Fall 2012 scientific conference.

In parallel with Study 010, Amicus is evaluating ERT-related immunogenicity in Pompe disease. Immune responses occur in a majority of Pompe patients receiving alglucosidase alfa infusions (Lacana E, Yao LP, Pariser AR, Rosenberg AS. 2012. The role of immune tolerance induction in restoration of the efficacy of ERT in Pompe disease., Am J Med Genet C Semin Med Genet. 160C:30-39) which have the potential to limit treatment outcomes with ERT. Preclinical results to date suggest that AT2220 when co-administered with Myozyme may mitigate immunogenicity induced by this ERT by stabilizing the enzyme in its properly folded and active form.

As part of a grant from the Muscular Dystrophy Association, Amicus is using blood samples from healthy volunteers and from Pompe patients in Study 010 to determine if particular human leukocyte antigen (HLA) types

are predictive of clinical immunogenicity to ERT. These results may help guide further investigation of the effects of AT2220 on immune response to ERT in future clinical studies.

Gaucher Disease and Other Lysosomal Storage Diseases

We are also investigating chaperone-ERT combinations as potential next-generation treatments for Gaucher and other undisclosed lysosomal storage diseases where we believe there are significant opportunities to improve treatment outcomes. In Gaucher disease, we are continuing preclinical studies to evaluate two pharmacological chaperones, AT2101 (afegostat tartrate) and AT3375, in combination with ERT (beta-glucosidase). Both of these chaperones target the enzyme deficient in Gaucher disease.

Collaboration with GSK

On July 17, 2012, the Company entered into the Expanded Collaboration Agreement (the "Expanded Collaboration Agreement") with GSK pursuant to which the Company and GSK will continue to develop and commercialize migalastat HCI, currently in Phase 3 development for the treatment of Fabry disease. The Expanded Collaboration Agreement amends and replaces in its entirety the Original Collaboration Agreement for the development and commercialization of migalastat HCl. Under the terms of the Expanded Collaboration Agreement, the Company and GSK will co-develop all formulations of migalastat HCl for Fabry disease, including the development of migalastat HCl co-formulated with JR-051. The Company will commercialize all migalastat HCl products for Fabry disease in the United States while GSK will commercialize all such products in the rest of the world. The exclusive license granted to GSK under the Original Collaboration Agreement to commercialize migalastat HCl worldwide is therefore replaced under the Expanded Collaboration Agreement with two exclusive licenses: (i) an exclusive license from GSK to Amicus to commercialize migalastat HCl in the United States, and (ii) an exclusive license from Amicus to GSK to commercialize migalastat HCl in the rest of world. GSK and Amicus each have a license to manufacture migalastat HCl for commercialization of monotherapy and chaperone-ERT co-administration migalastat HCl products while GSK maintains an exclusive license to manufacture such products for development purposes (subject to limited exceptions) and to manufacture the Co-formulated Product. In the event of a change of control of Amicus during the term of the Expanded Collaboration Agreement, GSK has the option to purchase an exclusive license to develop, manufacture and commercialize migalastat HCl in the United States.

GSK is eligible to receive U.S. regulatory approval milestones totaling $20 million for migalastat HCl monotherapy and chaperone-ERT co-administration, and additional regulatory approval and product launch milestone payments totaling up to $35 million within seven years following the launch of the Co-formulated Product. Amicus will also be responsible for certain pass-through milestone payments and single-digit royalties on the net U.S. sales of the Co-formulated Product that GSK must pay to a third party. In addition, Amicus is no longer eligible to receive any milestones or royalties it would have been eligible to receive under the Original Collaboration Agreement other than a $3.5 million clinical development milestone achieved in the second quarter of 2012 and expected to be paid by GSK to Amicus in the third quarter of 2012.

The Company and GSK will continue to jointly fund development costs for all formulations of migalastat HCl in accordance with agreed upon development plans pursuant to which Amicus and GSK will fund 25% and 75% of such costs, respectively, for the monotherapy and co-administration development of migalastat HCl for the remainder of 2012 and 40% and 60%, respectively, thereafter. Beginning immediately, costs for the development of the Co-formulated Product are also split 40% and 60% between Amicus and GSK, respectively.

Additionally, simultaneous with entry into the Expanded Collaboration Agreement, the Company and GSK entered into a Stock Purchase Agreement (the "SPA") pursuant to which GSK will purchase approximately 2.9 million shares of Amicus common stock at a price of $6.30 per share. The total value of this equity investment to the Company is approximately $18.6 million and increases GSK's ownership position in the Company to 19.9%.

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

In November 2010, GSK paid us an initial, non-refundable license fee of $30 million and a premium of $3.2 million related to GSK's purchase of an equity investment in Amicus. The total upfront consideration received of $33.2 million will be recognized as Collaboration and Milestone Revenue on a straight-line basis over the development period of the collaboration agreement which is approximately 5.2 years. In June 2012, we recognized $3.5 million as milestone revenue due to the completion of the last patient visit in the Fabry Phase 3 011 study. For the three and six months ended June 30, 2012, we recognized approximately $5.2 million and $6.8 million, respectively, of the total upfront consideration and milestone event as Collaboration and Milestone Revenue, and approximately $5.5 million and $11.6 million, respectively, of Research Revenue for reimbursed research and development costs..

Research and Development Expenses

We expect to continue to incur substantial research and development expenses as we continue to develop our product candidates and explore new uses for our pharmacological chaperone technology. However, we will share future research and development costs related to migalastat HCl with GSK in accordance with the Expanded Collaboration Agreement. Research and development expense consists of:

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

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

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

We expense research and development costs as incurred, including payments made to date under our license agreements. We believe that significant investment in product development is a competitive necessity and plan to continue these investments in order to realize the potential of our product candidates. From our inception in February 2002 through June 30, 2012, we have incurred research and development expense in the aggregate of $293.3 million.

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

Projects	Three Months Ended June 30, 2011	Three Months Ended June 30, 2012	Six Months Ended June 30, 2011	Six Months Ended June 30, 2012	Period from February 4, 2002 (inception) to June 30, 2012
Third party direct project expenses					
Migalastat HCl (Fabry Disease — Phase 3)	$ 4,166	$ 4,560	$ 8,228	$ 10,269	$ 75,604
Afegostat tartrate (Gaucher Disease — Phase 2*)	(1)	15	(201)	42	26,157
AT2220 (Pompe Disease — Phase 2)	41	3	45	—	13,243
Neurodegenerative Diseases (Preclinical)	498	124	949	341	8,949
Co-Administration studies (Fabry & Pompe - Phase 2; Gaucher - Preclinical)	48	1,320	169	2,188	5,138
Total third party direct project expenses	4,752	6,022	9,190	12,840	129,091
Other project costs (1)					
Personnel costs	4,801	5,203	9,724	10,645	103,896
Other costs (2)	2,065	2,498	3,829	4,242	60,360
Total other project costs	6,866	7,701	13,553	14,887	164,256
Total research and development costs	$ 11,618	$ 13,723	$ 22,743	$ 27,727	$ 293,347

(1) Other project costs are leveraged across multiple projects.
(2) Other costs include facility, supply, overhead, and licensing costs that support multiple clinical and preclinical projects.
* We do not plan to advance afegostat tartrate into Phase 3 development at this time.

The successful development of our product candidates is highly uncertain. At this time, we cannot reasonably estimate or know the nature, timing and costs of the efforts that will be necessary to complete the remainder of the development of our product candidates. As a result, we are not able to reasonably estimate the period, if any, in which material net cash inflows may commence from our product candidates, including migalastat 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. In addition, GSK has considerable influence over and decision-making authority related to our migalastat HCl program. A change in the outcome of any of the foregoing variables with respect to the development of a product candidate could mean a significant change in the costs and timing associated with the development, regulatory approval and commercialization of that product candidate. For example, if the FDA or other regulatory authorities were to require us to conduct clinical trials beyond those which

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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 June 30, 2012, we spent $123.2 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 equipment financing agreement.

Critical Accounting Policies and Significant Judgments and Estimates

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

While there were no significant changes during the quarter ended June 30, 2012 to the items that we disclosed as our significant accounting policies and estimates described in Note 2 to the Company's financial statements as contained in the Company's Annual Report on Form 10-K for the year ended December 31, 2011, we believe that the following accounting policies are the most critical to aid you in fully understanding and evaluating our financial condition and results of operations.

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.

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

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

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

Accrued Expenses

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

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

Stock-Based Compensation

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

We 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.

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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), we believe that we do 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:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2011	2012	2011	2012
Expected stock price volatility	78.4%	77.0%	78.8%	77.5%
Risk free interest rate	2.1%	1.1%	2.2%	0.7%
Expected life of options (years)	6.25	6.25	6.25	6.25
Expected annual dividend per share	$ 0.00	$ 0.00	$ 0.00	$ 0.00

Warrants

The warrants issued in connection with the March 2010 registered direct offering are classified as a liability. The fair value of the warrants 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. The weighted average assumptions used in the Black-Scholes valuation model for the warrants December 31, 2011 and June 30, 2012 are as follows:

	December 31, 2011	June 30, 2012
Expected stock price volatility	67.3%	73.27%
Risk free interest rate	0.28%	0.29%
Expected life of warrants (years)	2.17	1.67
Expected annual dividend per share	$ 0.00	$ 0.00

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

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

The following table provides a reconciliation of the numerator and denominator used in computing basic and diluted net loss attributable to common stockholders per common share and pro forma net loss attributable to common stockholders per common share:

(In thousands, except per share amount)	Three Months Ended June 30, 2011	Three Months Ended June 30, 2012	Six Months Ended June 30, 2011	Six Months Ended June 30, 2012
Historical				
Numerator:				
Net loss attributable to common stockholders	$ (12,641)	$ (9,343)	$ (25,991)	$ (22,480)
Denominator:				
Weighted average common shares outstanding - basic and diluted	34,530,693	46,870,067	34,514,947	42,103,642

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 7.6 million and 9.1 million for the six months ended June 30, 2011 and 2012, 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

Three Months Ended June 30, 2012 Compared to Three Months Ended June 30, 2011

Revenue. For the three months ended June 30, 2012 we recognized $3.5 million as milestone revenue upon achieving clinical development milestone and $1.7 million of the total upfront consideration received from GSK upon entry into the License and Collaboration Agreement as Collaboration Revenue. The total upfront consideration received of $33.2 million will be recognized as Collaboration and Milestone Revenue on a straight-line basis over the development period of the collaboration agreement which is approximately 5.2 years.

Research revenue was $5.5 million for the three months ended June 30, 2012, representing an increase of $3.1 million or 129% from the $2.4 million for the three months ended June 30, 2011. The increase was due to an increase in GSK's reimbursement rate from 50% to 75% on Jan 1, 2012 and increased overall expenditures in the development of migalastat HCl.

Research and Development Expense. Research and development expense was $13.7 million for the three months ended June 30, 2012, representing an increase of $2.1 million or 18% from $11.6 million for the three months ended June 30, 2011. The variance was primarily attributable to higher personnel costs, an increase in consulting costs and an increase in contract research and manufacturing costs due to the increased activity within the Fabry program.

General and Administrative Expense. General and administrative expense was $5.8 million for the three months ended June 30, 2012, representing a decrease of $0.9 million or 13% from $6.7 million for the three months ended June 30, 2011. The decrease was primarily due to additional stock option compensation expense recognized in 2011 as a result of the change in the terms of the Chief Executive Officer's stock options resulting from his resignation

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and subsequent reappointment to the Chief Executive Officer position partially offset by an increase in personnel costs associated with a severance charge of $0.7 million in 2012.

Interest Income and Interest Expense. Interest income was $0.12 million for the three months ended June 30, 2012, representing an increase of $ 0.07 million or 140% increase from $0.05 million for the three months ended June 30, 2011. The increase was due to overall higher average cash and investment balances, due to cash raised in the March 2012 stock offering. Interest expense was approximately $0.02 million for the three months ended June 30, 2012 compared to $0.04 for the three months ended June 30, 2011. The decrease was due to less outstanding debt during the period on the secured loan.

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 exercised or expired. Changes in the fair value of the warrants are reported in the statements of operations as non-operating income or expense. For the three months ended June 30, 2012, we reported an expense of $0.1 million related to the increase in fair value of these warrants as compared to a gain of $2.1 million for the three months ended June 30, 2011, representing a decrease of $2.2 million or 106%. The decrease was due to the fluctuations in the price of our common stock.

Other Income. Other income was $0.02 million for the three months ended June 30, 2012 and represents cash received from the sale of property, plant and equipment.

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Results of Operations

Six Months Ended June 30, 2012 Compared to Six Months Ended June 30, 2011

Revenue. For the six months ended we recognized $3.5 million as milestone revenue upon achieving clinical development milestone and $3.3 million of the total upfront consideration received from GSK upon entry into the License and Collaboration Agreement as Collaboration Revenue. The total upfront consideration received of $33.2 million will be recognized as Collaboration and Milestone Revenue on a straight-line basis over the development period of the collaboration agreement which is approximately 5.2 years.

Research revenue was $11.6 million for the six months ended June 30, 2012, representing an increase of $4.9 million or 73% from the $6.7 million for the six months ended June 30, 2011. The increase was due to an increase in GSK's reimbursement rate from 50% to 75% on Jan 1, 2012.

Research and Development Expense. Research and development expense was $27.7 million for the six months ended June 30, 2012, representing an increase of $5.0 million or 22% from $22.7 million for the six months ended June 30, 2011. The variance was primarily attributable to higher personnel costs, an increase in consulting costs and an increase in contract research and manufacturing costs due to the increased activity within the Fabry program.

General and Administrative Expense. General and administrative expense was $9.9 million for the six months ended June 30, 2012, representing a decrease of $1.2 million or 11% from $11.1 million for the six months ended June 30, 2011. The decrease was primarily due to additional stock option compensation expense recognized in 2011 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 partially offset by an increase in personnel costs associated with a severance charge of $0.7 million in 2012.

Interest Income and Interest Expense. Interest income was $0.1 million for the six months ended June 30, 2012, and 2011. Interest expense was approximately $0.06 million for the six months ended June 30, 2012 compared to $0.09 for the three months ended June 30, 2011. The decrease was due to less outstanding debt during the period on the secured loan.

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 exercised or expired. Changes in the fair value of the warrants are reported in the statements of operations as non-operating income or expense. For the six months ended June 30, 2012, we reported an expense of $2.5 million related to the increase in fair value of these warrants as compared to an expense of $1.4 million for the six months ended June 30, 2011, representing an increase of $1.1 million or 79%. The increase was due to the fluctuations in the price of our common stock.

Other Income/Expense. Other income for the six months ended June 30, 2011 represents funds received from the U.S. Treasury Department in February 2011 of $0.07 million under the Qualified Therapeutic Discovery Projects tax credit and grant program. Other income for the six months ended June 30, 2012 was $0.02 million and represents cash received from the sale of property, plant and equipment.

Liquidity and Capital Resources

Source of Liquidity

As a result of our significant research and development expenditures and the lack of any approved products to generate product sales revenue, we have not been profitable and have generated operating losses since we were incorporated in 2002. We have funded our operations principally with $148.7 million of proceeds from redeemable convertible preferred stock offerings, $75.0 million of gross proceeds from our IPO in June 2007, $18.5 million of gross proceeds from our Registered Direct Offering in March 2010, $65.6 million of gross proceeds from our stock offering in March 2012, $80.0 million from the non-refundable license fees from the collaboration agreements and $31.0 million from GSK's investment in the Company. In the future, we expect to fund our operations, in part,

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through the receipt of cost-sharing payments from GSK. The following table summarizes our significant funding sources as of June 30, 2012:

Funding	Year	No. Shares	Approximate Amount (1) (in thousands)
Series A Redeemable Convertible Preferred Stock	2002	444,443	$ 2,500
Series B Redeemable Convertible Preferred Stock	2004, 2005, 2006, 2007	4,917,853	31,189
Series C Redeemable Convertible Preferred Stock	2005, 2006	5,820,020	54,999
Series D Redeemable Convertible Preferred Stock	2006, 2007	4,930,405	60,000
Common Stock	2007	5,000,000	75,000
Upfront License Fee from Shire	2007	—	50,000
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
		44,425,491	$ 419,023

(1) Represents gross proceeds

On July 17, 2012, we entered into an SPA with GSK, pursuant to which GSK purchased 2.9 million unregistered shares of our common stock at a price of $6.30 per share. The total purchase price for these shares was $18.6 million and increases GSK's ownership position to 19.9%. We received all proceeds from the sale of such shares on July 26, 2012.

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 March 31, 2012 of $10.9 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.

As of June 30, 2012, we had cash, cash equivalents and marketable securities of $95.8 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 six months ended June 30, 2011 was $23.9 million due primarily to the net loss for the six months ended June 30, 2011 of $26.0 million and the change in operating assets and liabilities of $5.4 million. The change in operating assets and liabilities consisted of an increase in receivables from GSK related to the collaboration agreement of $2.0 million; a decrease in deferred revenue of $1.4 million related to the recognition of the upfront payment from GSK for the collaboration agreement; and a decrease in accounts payable and accrued expenses of $1.3 million related to program expenses.

Net cash used in operations for the six months ended June 30, 2012 was $19.2 million, due primarily to the net loss for the six months ended June 30, 2012 of $22.5 million and the change in operating assets and liabilities of $3.2 million. The change in operating assets and liabilities consisted of a increase in

receivables from GSK related to the collaboration agreement of $2.2 million; a decrease of $3.2 million in prepaid assets primarily related to a receivable from the sale of state net operating loss carry forwards, or NOLs; a decrease of $0.3 in non-current assets related to the return of the security deposit on the terminated lease; a decrease in deferred revenue of $3.9 million related to the recognition of the upfront payment from GSK for the collaboration agreement and a decrease in accounts payable and accrued expenses of $0.6 million related to program expenses.

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Net Cash Provided By/ (Used in) Investing Activities

Net cash provided by investing activities for the six months ended June 30, 2011 was $20.4 million. Net cash provided by investing activities reflects $57.2 million for the sale and redemption of marketable securities partially offset by $36.6 million for the purchase of marketable securities and $0.2 million for the acquisition of property and equipment.

Net cash used in investing activities for the six months ended June, 2012 was $40.8 million. Net cash used by investing activities reflects $71.7 million for the purchase of marketable securities and $3.9 million for the acquisition of property and equipment partially offset by $34.8 million for the sale and redemption of marketable securities.

Net Cash (Used in)/Provided by Financing Activities

Net cash used in financing activities for the six months ended June 30, 2011 was $0.3 million, consisting primarily of $0.6 million of payments on our secured loan agreement and capital lease obligations. The payments were partially offset by $0.3 million of cash proceeds from the exercise of stock options.

Net cash provided by financing activities for the three months ended June 30, 2012 was $63.2 million, consisting of $62.1 million from the issuance of common stock, $1.0 million as proceeds from the new secured loan agreement with SVB and $0.8 million from the exercise of stock options. This was partially offset by the payments of our secured loan agreement of $0.7 million.

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

We do not anticipate that we will generate revenue from commercial sales until at least 2013, 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 for 2012.

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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 tartrate 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 tartrate, we may be obligated, under the agreements that we have in place, to make future milestone payments aggregating up to approximately $7.9 million. In addition, under the Expanded Collaboration Agreement, GSK is eligible to receive U.S. regulatory approval milestones totaling $20 million for migalastat HCl monotherapy and migalastat HCl for co-administration with ERT, and additional regulatory approval and product launch milestone payments totaling up to $35 million within seven years following the launch of the Co-formulated Product. However, such potential milestone payments are subject to many uncertain variables that would cause such payments, if any, to vary in size.

Royalties

Under our license agreements, if we owe royalties on net sales for one of our products to more than one licensor, then we have the right to reduce the royalties owed to one licensor for royalties paid to another. The amount of royalties to be offset is generally limited in each license and can vary under each agreement. For 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 tartrate should we advance afegostat tartrate to commercialization. To date, we have not made any royalty payments on sales of our.

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

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ITEM 3. 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 June 30, 2012, we held $95.8 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. 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 outside the U.S. While most expenses are paid in U.S. dollars, there are minimal payments made in local foreign currency. If exchange rates undergo a change of 10%, we do not believe that it would have a material impact on our results of operations or cash flows.

ITEM 4. CONTROLS AND PROCEDURES

As of the end of the period covered by this Quarterly Report on Form 10-Q, an evaluation of the effectiveness of our disclosure controls and procedures (pursuant to Rule 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended (the Exchange Act)) was carried out under the supervision of our Principal Executive Officer and Principal Financial Officer, with the participation of our management. Based on that evaluation, the Principal Executive Officer and the Principal Financial Officer concluded that, as of the end of such period, our disclosure controls and procedures are effective in recording, processing, summarizing and reporting, on a timely basis, information required to be disclosed by us in the reports that we file or submit under the Exchange Act and are effective in ensuring that information required to be disclosed by us in the reports that we file or submit under the Exchange Act is accumulated and communicated to our management, including our Principal Executive Officer and Principal Financial Officer, as appropriate to allow timely decisions regarding required disclosure.

During the fiscal quarter covered by this report, there has been no change in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) that occurred during the fiscal quarter that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

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PART II. OTHER INFORMATION

ITEM 1. LEGAL PROCEEDINGS

We are not a party to any material legal proceedings.

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 $312.8 million and we had an accumulated deficit of $292.6 million as of June 30, 2012. To date, we have financed our operations primarily through private placements of our redeemable convertible preferred stock, proceeds from our initial public offering and subsequent stock offerings, payments from partners during the terms of collaboration agreements and other financing arrangements. 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 (Study 011) and worldwide (Study 012);

- continue our ongoing Phase 2 clinical trial of migalastat HCl co-administered with ERT for Fabry disease and our Phase 2 clinical trial of AT2220 co-administered with ERT for Pompe disease;

- continue our preclinical studies on the use of pharmacological chaperones for the treatment of Parkinson's Disease;
- continue our preclinical studies on the use of pharmacological chaperones co-administered with ERT for 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

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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. While research and development costs associated with our migalastat HCl program will be shared with GSK so long as our collaboration continues, we remain responsible for all costs related to our other programs.

Should GSK terminate our collaboration agreement, we would likely need to seek additional funding in order to complete any clinical trials related to migalastat HCl, seek regulatory approvals of migalastat HCl, and launch the product candidate outside of the United States and continue our other clinical and preclinical programs. Capital may not be available when needed on terms that are acceptable to us, or at all, especially in light of the current challenging economic environment. If adequate funds are not available to us on a timely basis, we may be required to reduce or eliminate research development programs or commercial efforts.

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

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

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

Until such time, if ever, as we generate product revenue to finance our operations, we expect to finance our cash needs through public or private equity offerings and debt financings, corporate collaboration and licensing arrangements and grants from patient advocacy groups, foundations and government agencies. If we are able to raise capital by issuing equity securities, as we did in March 2012, our stockholders will experience dilution. In addition,

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stockholders may experience dilution if the holders of the warrants issued in connection with our March 2010 offering exercise their warrants. Debt financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends and may include rights that are senior to the holders of our common stock. Each of our current loan and security agreements 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. Any debt financing or additional equity that we raise may contain terms, such as liquidation and other preferences, which are not favorable to us or our stockholders. If we raise capital through additional collaboration and licensing arrangements with third parties, it may be necessary to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or to grant licenses on terms that may not be favorable to us or our stockholders.

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 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 successfully complete large-scale, clinical trials, obtain regulatory approvals, manufacture a commercial-scale product or arrange for a third party to do so on our behalf, or conduct sales and marketing activities necessary for successful product commercialization. Consequently, any predictions about our future success or viability may not be as accurate as they could be if we had a longer operating history.

In addition, 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.

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Risks Related to the Development and Commercialization of Our Product Candidates

We depend heavily on the success of our most advanced product candidate, migalastat HCl. 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 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, in particular our collaboration with GSK. The successful commercialization of our product candidates will depend on several factors, including the following:

- successful enrollment of patients in our clinical trials on a timely basis;
- obtaining supplies of our product candidates and, where required, third party marketed products including ERTs, for completion of our clinical trials on a timely basis;
- successful completion of preclinical studies and clinical trials;
- obtaining regulatory agreement in the structure and design of our clinical programs;
- obtaining marketing approvals from the 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

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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.

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

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

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

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

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

We have limited experience in conducting and managing the preclinical development activities and clinical trials necessary to obtain regulatory approvals, including approval by the FDA. We have not obtained regulatory approval nor commercialized any of our product candidates. Although we completed enrollment in our first Phase 3 study of migalastat HCl, 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

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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;
- 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;

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- 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. In addition, GSK has significant influence on the conduct of our migalastat HCl program, and could compel us to perform unanticipated clinical trials of migalastat HCl or delay the approval process for a variety of reasons.

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 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.

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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 against companies for alleged improper promotion and has enjoined several companies from engaging in off-label promotion. The FDA has also requested that companies enter into consent decrees or permanent injunctions under which specified promotional conduct is changed or curtailed.

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;

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- willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- strength of marketing and distribution support and timing of market introduction of competitive products;

- publicity concerning our products or competing products and treatments; and
- sufficient third party insurance coverage or reimbursement.

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

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

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

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

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

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

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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. 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.

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;

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- unforeseen costs associated with creating our own sales and marketing team or with entering into a partnering agreement with an independent sales and marketing organization; and
- efforts by our competitors to commercialize products at or about the time when our product candidates would be coming to market.

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

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

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

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

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

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

- decreased demand for any product candidates or products;
- damage to our reputation;
- regulatory investigations, prosecutions or enforcement actions that could require costly recalls or product modifications;
- withdrawal of clinical trial participants;
- costs to defend the related litigation;

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- substantial monetary awards to trial participants or patients, including awards that substantially exceed our product liability insurance, which we would then be required to pay from other sources, if available, and would damage our ability to obtain liability insurance at reasonable costs, or at all, in the future;

- loss of revenue;

- the diversion of management's attention from managing our business; and

- the inability to commercialize any such product candidates or products.

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

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

The development and commercialization of new drugs is highly competitive and competition is expected to increase. We face competition with respect to our current product candidates and any products we may seek to develop, acquire or commercialize in the future from major pharmaceutical companies, specialty pharmaceutical companies and biotechnology companies worldwide. For example, several large pharmaceutical and biotechnology companies currently market and sell products for the treatment of lysosomal storage diseases, including Fabry disease. These products include sanofi aventis' Fabrazyme® and Shire plc's Replagal®. In addition, sanofi aventis, Shire and Actelion, Ltd. market and sell Cerezyme® , VPRIV and Zavesca®, respectively, for the treatment of Gaucher disease, and sanofi aventis markets and sells Myozyme® and Lumizyme® for the treatment of Pompe disease. In addition, taliglucerase alfa, a new enzyme replacement therapy for the treatment of Gaucher disease which is being developed by Protalix BioTherapeutics and Pfizer, Inc., was approved by the FDA for Type 1 Gaucher disease in May. We are also aware of other enzyme replacement and substrate reduction therapies in development by third parties, including eliglustat tartrate, an oral treatment developed by sanofi aventis and in Phase 3 development for the treatment of Gaucher disease, and taliglucerase alfa, a new enzyme replacement therapy for the treatment of Gaucher disease which is being developed by Protalix BioTherapeutics.

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

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advantageous to our business. In addition, if we obtain regulatory approvals for our products, manufacturing efficiency and marketing capabilities are likely to be significant competitive factors. We currently have no commercial manufacturing capability, sales force or marketing infrastructure. Further, many of our competitors have substantial resources and expertise in conducting collaborative arrangements, sourcing in-licensing arrangements and acquiring new business lines or businesses that are greater than our own.

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

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

Risks Related to Our Dependence on Third Parties

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

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

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

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

- reliance on the third party for regulatory compliance and quality assurance;

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

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

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

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

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

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

We acquired the US commercial rights to all formulations of migalastat HCl under the Expanded Collaboration Agreement with GSK entered into in July 2012. We therefore 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

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our investment would be lost if we cannot retain or reposition our sales and marketing personnel. Factors that may inhibit our efforts to commercialize our products include:

- our inability to recruit and retain adequate numbers of effective sales and marketing personnel;
- the inability of sales personnel to obtain access to or persuade adequate numbers of physicians to prescribe any future products;
- the lack of complementary products to be offered by sales personnel, which may put us at a competitive disadvantage relative to companies with more extensive product lines; and
- unforeseen costs and expenses associated with creating an independent sales and marketing organization.

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

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

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

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

We do not independently conduct clinical trials for our product candidates or certain preclinical development activities of our product candidates, such as long-term safety studies in animals. We rely on, or work in conjunction with, third parties, such as contract research organizations, medical institutions and clinical investigators, to perform these functions. For example, we rely heavily on a contract research organization to help us conduct our ongoing Phase 3 clinical trials in migalastat 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

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

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

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

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

For each of our product candidates, we are collaborating with physicians, patient advocacy groups, foundations and government agencies in order to assist with the development of our products. We plan to pursue similar activities in future programs and plan to evaluate the merits of retaining commercialization rights for ourselves or entering into selective collaboration arrangements with leading pharmaceutical or biotechnology companies, such as our collaboration for migalastat HCl with GSK for commercialization rights outside of the United States. 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.

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

We expect that a substantial amount of the funding for our operations will come from our collaboration with GSK. We and GSK are jointly developing migalastat HCl and sharing costs associated with the development program in accordance with agreed upon development plans. Under the plans, while we currently pay 25% of joint development costs for development of all formulations of migalastat HCl other than migalastat HCl co-formulated with ERT (for which we will pay 40% beginning with entry into the Expanded Collaboration Agreement in July 2012), we will be responsible for 40% of joint development costs for all formulations of migalastat HCl beginning in 2013. Our business plan and financial guidance currently include assumptions regarding GSK's cost-sharing obligations. However, GSK may elect to terminate this collaboration at its discretion. If this collaboration is unsuccessful, or if it is terminated in whole or in part, our business could be adversely affected. As a result, we could require additional financing earlier than we currently expect, or need to take additional steps to manage the financial risk associated with such termination, including actions that may affect our other programs. In addition, while we are collaborating with GSK on the development of migalastat HCl, GSK has ultimate decision making authority with respect to clinical development.

Risks Related to Our Intellectual Property

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

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

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

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

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

The patents and patent applications that we have licensed from Mt. Sinai School of Medicine relating to use of migalastat HCl to treat Fabry disease expire in 2018 in the U.S., 2019 in Europe and Japan and 2019 in Canada. The patent applications covering migalastat HCl to treat Fabry disease have been sublicensed by us to GSK, which now controls the worldwide prosecution of said patent applications to the extent they relate to migalastat HCl. GSK also controls post-grant patent prosecution and enforcement proceedings outside of the U.S. only. 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 tartrate'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 tartrate 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.

- 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

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interference, declared by the U.S. Patent and Trademark Office to determine priority of invention in the U.S. The costs of these proceedings could be substantial and it is possible that our efforts could be unsuccessful, resulting in a loss of our U.S. patent position.

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

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

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

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

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

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

No assurance can be given that patents do not exist, have not been filed, or could not be filed or issued, which contain claims covering our 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.

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

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

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

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

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

Risks Related to Regulatory Approval of Our Product Candidates

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

Our product candidates, including migalastat 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. In the case of migalastat HCl, GSK will have primary responsibility for the preparation, submission and maintenance of applications for approval with regulatory agencies outside the United States.

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

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inspection of manufacturing facilities by, the FDA. Our future products may not be effective, may be only moderately effective or may prove to have undesirable or unintended side effects, toxicities or other characteristics that may preclude our obtaining regulatory approval or prevent or limit commercial use.

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

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

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

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

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- 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 on February 25, 2004, for the active ingredient in afegostat tartrate for the treatment of Gaucher disease on January 10, 2006 and for AT2220 for the treatment of Pompe disease on June 18, 2007. We also obtained orphan medicinal product designation in the EU for migalastat HCl on May 22, 2006and for afegostat tartrate on October 23, 2007. We anticipate filing for orphan drug designation in the EU for AT2220 for the treatment of Pompe disease. Generally, if a product with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, the product is entitled to a period of marketing exclusivity, which precludes the applicable regulatory authority from approving another marketing application for the same drug for that time period. The applicable period is 7 years in the U.S. and 10 years in Europe. For a drug composed of small molecules, the FDA defines "same drug" as a drug that contains the same active molecule and is intended for the same use. Obtaining orphan drug exclusivity for migalastat HCl and afegostat tartrate 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;

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- fines;
- suspension or withdrawal of regulatory approvals or refusal to approve pending applications or supplements to approved applications that we submit;
- refusal to permit the import or export of our products;
- product seizure or detentions;
- injunctions or the imposition of civil or criminal penalties; and
- adverse publicity.

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

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

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

Risks Related to Employee Matters

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

We are highly dependent on John F. Crowley, our Chairman and Chief Executive Officer, Bradley L. Campbell, our Chief Business Officer, David J. Lockhart, Ph.D., our Chief Scientific Officer, Pol F. Boudes, M.D., our Chief Medical 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.

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 59% of our common stock as of June 30, 2012. 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.

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

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

We completed our initial public offering of equity securities in June 2007, and prior to 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 may never develop or be sustained. If an active market for our common stock does not develop or is not sustained, it may be difficult for our stockholders to sell shares 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;

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- future sales or anticipated sales of our common stock by us or our stockholders; and
- the other factors described in this "Risk Factors" section.

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

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

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

ITEM 2. UNREGISTERED SALES OF EQUITY SECURITIES AND USE OF PROCEEDS

Recent Sales of Unregistered Securities

None.

Use of Proceeds

Initial Public Offering

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

After deducting expenses of approximately $6.9 million, we received net offering proceeds of approximately $68.1 million from our initial public offering. As of June 30, 2012, we have used the proceeds of approximately $68.1 million for clinical development of our projects, research and development activities relating to additional preclinical projects and to fund working capital and other general corporate purposes.

March 2010 Registered Direct Offering

In March 2010, we sold 4,946,524 shares of our common stock and warrants to purchase 1,854,946 shares of common stock in a registered direct offering to a select group of institutional investors through a Registration Statement on Form S-3 (File No. 333-158405) that was declared effective by the SEC on May 27, 2009. The shares of common stock and warrants were sold in units consisting of one share of common stock and one warrant to purchase 0.375 shares of common stock at a price of $3.74 per unit. The warrants have a term of four years and are exercisable any time on or after the six month

anniversary of the date they were issued, at an exercise price of $4.43 per share. The aggregate offering proceeds were $18.5 million. Leerink Swann LLC served as sole placement agent for the offering. Following the sale of the common stock and warrants, the public offering terminated.

We paid Leerink Swann a placement agency fee equal to 5.7% of the aggregate offering proceeds, approximately $1.05 million. The net proceeds of the offering were approximately $17.1 million after deducting the

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placement agency fee and all other estimated offering expenses. No offering expenses were paid directly or indirectly to any of our directors or officers (or their associates) or persons owning ten percent or more of any class of our equity securities or to any other affiliates.

As of June 30, 2012, we have used approximately $17.1 million in net proceeds to further advance the development of our lead product candidate, migalastat HCl, and the completion of certain activities required for the submission of a license application globally, as well as for general corporate matters.

March 2012 Stock Offering

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 on May 27, 2009. The aggregate offering proceeds were $65.6 million. Leerink Swann LLC and Cowen and Company served as placement agents for the offering.

We paid Leerink Swann LLC and Cowen and Company a placement agency fee equal to 5.0% of the aggregate offering proceeds, approximately $3.3 million. The net proceeds of the offering were approximately $62.0 million after deducting the placement agency fee and all other estimated offering expenses. No offering expenses were paid directly or indirectly to any of our directors or officers (or their associates) or persons owning ten percent or more of any class of our equity securities or to any other affiliates.

As of June 30, 2012, we had invested the $62.1 million in net proceeds from our registered direct offering in money market funds and in investment-grade, interest bearing instruments, pending their use. Through June 30, 2012, we have not used the net proceeds from this offering. We intend to use the proceeds from this offering to advance the clinical and preclinical development of our pharmacological chaperone monotherapy, co-formulation and co-administration programs, especially our lead program migalastat HCl for Fabry disease; to potentially enter into collaborations, alliances and other business development opportunities including the acquisition of preclinical-stage, clinical-stage and marketed products that are consistent with our strategic plan and support our continued transformation to a commercial biotechnology company, and for other general corporate purposes.

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

Issuer Purchases of Equity Securities

There were no purchases of our common stock for the three months ended June 30, 2012.

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ITEM 3. DEFAULTS UPON SENIOR SECURITIES

None.

ITEM 4. MINE SAFETY DISCLOSURES

None.

ITEM 5. OTHER INFORMATION

None.

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ITEM 6. EXHIBITS

Exhibit Number	Description
3.1 (1)	Restated Certificate of Incorporation
3.2 (2)	Amended and Restated By-laws
31.1*	Certification of Principal Executive Officer pursuant to Rules 13a-14 and 15d-14 promulgated pursuant to the Securities Exchange Act of 1934, as amended
31.2*	Certification of Principal Financial Officer pursuant to Rules 13a-14 and 15d-14 promulgated pursuant to the Securities Exchange Act of

	1934, as amended
32.1*	Certification of Principal Executive Officer and Principal Financial Officer pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
101.INS	XBRL Instance Document
101.SCH	XBRL Schema Document
101.CAL	XBRL Calculation Linkbase Document
101.DEF	XBRL Extension Definition Linkbase Document
101.LAB	XBRL Label Linkbase Document
101.PRE	XBRL Extension Presentation Linkbase Document

(1) Incorporated by reference to Exhibit 3.1 to our Annual Report on Form 10K filed on February 28, 2012

(2) Incorporated by reference to Exhibit 3.4 to our Registration Statement on Form S-1

* These certifications are being furnished solely to accompany this quarterly report pursuant to 18 U.S.C. Section 1350, and are not being filed for purposes of Section 18 of the Securities Exchange Act of 1934 and are not to be incorporated by reference into any filing of Amicus Therapeutics, Inc., whether made before or after the date hereof, regardless of any general incorporation language in such filing.

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SIGNATURES

Pursuant to the requirements of the Securities and Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned thereunto duly authorized.

AMICUS THERAPEUTICS, INC.

Date: August 7, 2012

By: /s/ JOHN F. CROWLEY

John F. Crowley
Chairman and Chief Executive Officer
(Principal Executive Officer)

Date: August 7, 2012

By: /s/ WILLIAM D. BAIRD III

William D. Baird III
Chief Financial Officer
(Principal Financial Officer)

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Exhibit 31.1

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

I, John F. Crowley, certify that:

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

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

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

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

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

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

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

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

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

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

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

Date: August 7, 2012

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

Exhibit 31.2

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

I, William D. Baird III, certify that:

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

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

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

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

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

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

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

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

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

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

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

Date: August 7, 2012

/s/ William D. Baird III
William D. Baird III
Chief Financial Officer

Exhibit 32.1

CERTIFICATION OF PRINCIPAL EXECUTIVE OFFICER AND PRINCIPAL FINANCIAL OFFICER PURSUANT TO 18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

Each of the undersigned hereby certifies, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, in his capacity as an officer of Amicus Therapeutics, Inc. (the "Company"), that, to his knowledge, the Quarterly Report of the Company on Form 10-Q for the period ended June 30, 2012, fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934 (15 U.S.C. 78m or 78o(d)) and that the information contained in such report fairly presents, in all material respects, the financial condition and results of operations of the Company. This written statement is being furnished to the Securities and Exchange Commission as an exhibit to such Form 10-Q. A signed original of this statement has been provided to the Company and will be retained by the Company and furnished to the Securities and Exchange Commission or its staff upon request.

Date: August 7, 2012 By: /s/ John F. Crowley
John F. Crowley
Chairman and Chief Executive Officer

Date: August 7, 2012 By: /s/ William D. Baird III
William D. Baird III
Chief Financial Officer