

AMICUS THERAPEUTICS INC

Form 10-Q

May 11, 2012

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**UNITED STATES**  
**SECURITIES AND EXCHANGE COMMISSION**

Washington, D.C. 20549

**FORM 10-Q**

(Mark One)

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

For the quarterly period ended March 31, 2012

OR

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

For the transition period from \_\_\_\_\_ to \_\_\_\_\_

Commission file number 001-33497

## Amicus Therapeutics, Inc.

(Exact Name of Registrant as Specified in Its Charter)

**Delaware**  
(State or Other Jurisdiction of  
Incorporation or Organization)

**71-0869350**  
(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  No

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

Indicate by check mark 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

Accelerated filer

Non-accelerated filer

Smaller Reporting Company

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

The number of shares outstanding of the registrant's common stock, \$.01 par value per share, as of April 26, 2012 was 46,376,169 shares.



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AMICUS THERAPEUTICS, INC

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We have filed applications to register certain trademarks in the United States and abroad, including AMICUSTM and AMICUS THERAPEUTICSTM (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;
- our ability to achieve development and commercialization milestone payments and sales royalties under our collaboration with GlaxoSmithKline plc;
- the scope, progress, results and costs of preclinical development, laboratory testing and clinical trials for our product candidates including those testing the use of pharmacological chaperones co-administered with enzyme replacement therapy 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 I Item 1A Risk Factors of the Annual Report on Form 10-K for the year ended December 31, 2011 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.

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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.

Table of Contents**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)**

	<b>December 31, 2011</b>	<b>March 31, 2012</b>
<b>Assets:</b>		
Current assets:		
Cash and cash equivalents	\$ 25,668	\$ 24,523
Investments in marketable securities	30,034	83,673
Receivable due from GSK	5,043	4,301
Prepaid expenses and other current assets	5,903	2,789
<b>Total current assets</b>	<b>66,648</b>	<b>115,286</b>
Property and equipment, less accumulated depreciation and amortization of \$9,507 and \$7,216 at December 31, 2011 and March 31, 2012, respectively	2,438	5,073
Other non-current assets	709	442
<b>Total Assets</b>	<b>\$ 69,795</b>	<b>\$ 120,801</b>
<b>Liabilities and Stockholders Equity</b>		
Current liabilities:		
Accounts payable and accrued expenses	\$ 9,708	\$ 8,449
Current portion of deferred revenue	8,504	8,187
Current portion of secured loan	1,044	1,129
<b>Total current liabilities</b>	<b>19,256</b>	<b>17,765</b>
Deferred revenue, less current portion	18,999	17,339
Warrant liability	1,948	4,324
Secured loan, less current portion		597
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, 46,376,169 shares issued and outstanding at March 31, 2012	407	524
Additional paid-in capital	299,285	363,456
Accumulated other comprehensive income	4	37



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Deficit accumulated during the development stage		(270,104)		(283,241)
Total stockholders' equity		29,592		80,776
<b>Total Liabilities and Stockholders' Equity</b>	\$	69,795	\$	120,801

*See accompanying notes to consolidated financial statements*

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

	2011	Three Months Ended March 31, 2012	2012	Period from February 4, 2002 (inception) to March 31, 2012
<b>Revenue:</b>				
Research revenue	\$ 4,306	\$ 6,114	\$ 52,016	
Collaboration revenue	1,660	1,660	59,222	
Total revenue	5,966	7,774	111,238	
<b>Operating Expenses:</b>				
Research and development	\$ 11,125	\$ 14,004	\$ 279,624	
General and administrative	4,402	4,095	117,344	
Restructuring charges			1,522	
Impairment of leasehold improvements			1,030	
Depreciation and amortization	438	420	10,483	
In-process research and development			418	
Total operating expenses	15,965	18,519	410,421	
Loss from operations	(9,999)	(10,745)	(299,183)	
<b>Other income (expenses):</b>				
Interest income	59	27	14,100	
Interest expense	(48)	(43)	(2,376)	
Change in fair value of warrant liability	(3,432)	(2,376)	(1,476)	
Other income	70		231	
Loss before tax benefit	(13,350)	(13,137)	(288,704)	
Benefit from income taxes			5,463	
Net loss	(13,350)	(13,137)	(283,241)	
Deemed dividend			(19,424)	
Preferred stock accretion			(802)	
Net loss attributable to common stockholders	\$ (13,350)	\$ (13,137)	\$ (303,467)	
Net loss attributable to common stockholders per common share basic and diluted	\$ (0.39)	\$ (0.35)		
Weighted-average common shares outstanding basic and diluted	34,498,926	37,887,520		

*See accompanying notes to consolidated financial statements*

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

	2011	Three Months Ended March 31,	2012	Period from February 4, 2002 (inception) to March 31, 2012
Net loss	\$ (13,350)	\$	(13,137)	\$ (283,241)
Other comprehensive income/(loss):				
Unrealized gain on available-for- sale securities	29		33	37
Other comprehensive income/(loss), before income taxes	29		33	37
Provision for income taxes related to other comprehensive income/(loss) Items (Note 1)				
Other comprehensive income/(loss)	\$ 29	\$	33	\$ 37
Comprehensive loss	\$ (13,321)	\$	(13,104)	\$ (283,204)

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*

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

	2011	Three Months Ended March 31, 2012	Period from February 4, 2002 (inception) to March 31, 2012
<b>Operating activities</b>			
Net loss	\$ (13,350)	\$ (13,137)	\$ (283,241)
Adjustments to reconcile net loss to net cash used in operating activities:			
Non-cash interest expense			525
Depreciation and amortization	438	420	10,483
Amortization of non-cash compensation			522
Stock-based compensation - employees	1,418	1,388	37,125
Stock-based compensation - non-employees			853
Stock-based license payments			1,220
Change in fair value of warrant liability	3,432	2,376	1,476
Loss on disposal of asset		27	387
Impairment of leasehold improvements			1,030
Non-cash charge for in-process research and development			418
Beneficial conversion feature related to bridge financing			135
Changes in operating assets and liabilities:			
Receivable from GSK	(3,969)	742	(4,301)
Prepaid expenses and other current assets	(68)	3,114	(2,789)
Other non-current assets		267	(442)
Accounts payable and accrued expenses	(872)	(1,259)	8,449
Deferred revenue	(335)	(1,977)	25,526
Net cash used in operating activities	(13,306)	(8,039)	(202,624)
<b>Investing activities</b>			
Sale and redemption of marketable securities	30,889	14,300	686,390
Purchases of marketable securities	(19,099)	(67,906)	(770,142)
Purchases of property and equipment	(71)	(3,082)	(16,970)
Net cash provided by/(used in) investing activities	11,719	(56,688)	(100,722)
<b>Financing activities</b>			
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		62,063	175,309
Proceeds from the issuance of convertible notes			5,000
Payments of capital lease obligations	(40)		(5,611)
Payments of secured loan agreement	(313)	(313)	(3,027)
Proceeds from exercise of stock options	30	837	2,548
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

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Net cash (used in)/provided by financing activities	(323)	63,582	327,869
Net (decrease)/increase in cash and cash equivalents	(1,910)	(1,145)	24,523
Cash and cash equivalents at beginning of period	29,572	25,668	
Cash and cash equivalents at end of period	\$ 27,662	\$ 24,523	\$ 24,523

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

	2011	Three Months Ended March 31, 2012	Period from February 4, 2002 (inception) to March 31, 2012
<b>Supplemental disclosures of cash flow information</b>			
Cash paid during the period for interest	\$ 48	\$ 20	\$ 2,052
<b>Non-cash activities</b>			
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*

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**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 disorders and diseases of neurodegeneration. The Company's activities since inception have consisted principally of raising capital, establishing facilities and performing research and development. Accordingly, the Company is considered to be in the development stage.

In October 2010, the Company entered into the License and Collaboration Agreement with Glaxo Group Limited, an affiliate of GlaxoSmithKline plc (GSK), to develop and commercialize migalastat HCl. Under the terms of the License and 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 is eligible to receive further payments of \$173.5 million upon the successful achievement of development, regulatory and commercialization milestones, as well as tiered double-digit royalties on global sales of migalastat HCl. Potential payments include up to (i) \$13.5 million related to the attainment of certain clinical development objectives and the acceptance of regulatory filings in select worldwide markets, (ii) \$80 million related to market approvals for migalastat HCl in selected territories throughout the world, and (iii) \$80 million associated with the achievement of certain sales thresholds. GSK and the Company are jointly funding development costs in accordance with an agreed upon development plan. For further information, see Note 7. Collaborative Agreements.

The Company had an accumulated deficit of approximately \$283.2 million at March 31, 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 the 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 License and 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.

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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)



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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.

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

***Fair Value Measurements***

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

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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).

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

*New Accounting Standards*

In June 2011, the 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 years beginning after December 15, 2011 and should be applied prospectively. The adoption of this accounting pronouncement did not have a material impact on our financial statements.

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

As of March 31, 2012, the Company held \$24.5 million in cash and cash equivalents and \$83.7 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.



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Cash and available for sale securities consisted of the following as of December 31, 2011 and March 31, 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 March 31, 2012				
	Cost	Unrealized Gain	Unrealized Loss	Fair Value	
Cash balances	\$ 24,523	\$	\$	\$	\$ 24,523
Corporate debt securities	46,629	6	(46)		46,589
Commercial paper	36,657	77			36,734
Certificate of deposit	350				350
	\$ 108,159	\$ 83	\$ (46)	\$	\$ 108,196
Included in cash and cash equivalents	\$ 24,523	\$	\$	\$	\$ 24,523
Included in marketable securities	83,636	83	(46)		83,673
Total cash and available for sale securities	\$ 108,159	\$ 83	\$ (46)	\$	\$ 108,196

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 three months ended March 31, 2012, unrealized holding gains included in accumulated other comprehensive income was \$33 thousand.

For the year ended December 31, 2011 and the three months ended March 31, 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 March 31, 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 \$26.4 million as of December 31, 2011 and March 31, 2012, respectively.

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

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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-

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dilutive. Therefore, the weighted average shares used to calculate both basic and diluted earnings per share are the same.

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

(In thousands, except per share amounts)	Three Months Ended	
	2011	2012
<b>Statement of Operations</b>		
Net loss attributable to common stockholders	\$ (13,350)	\$ (13,137)
Net loss attributable to common stockholders per common share basic and diluted	\$ (0.39)	\$ (0.35)

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 7.7 million and 9.2 million for the three months ended March 31, 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*

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 in connection with the License and Collaboration Agreement, GSK purchased approximately 6.9 million shares of the Company's common stock at \$4.56 per share. The total value of this equity investment was approximately \$31 million and currently represents a 14.8% ownership position in the Company.

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. 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 months ended March 31, 2012, the Company recorded stock-based compensation expense of approximately \$1.4 million. The stock-based compensation expense had no impact on the Company's cash flows from operations and financing activities. As of March 31, 2012, the total unrecognized compensation cost related to non-vested stock options granted was \$11.3 million and is expected to be recognized over a weighted average period of 2.7 years.



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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 March 31,			
	2011		2012	
Expected stock price volatility		78.8%		78.4%
Risk free interest rate		2.3%		1.1%
Expected life of options (years)		6.25		6.25
Expected annual dividend per share	\$	0.00	\$	0.00

A summary of option activities related to the Company's stock options for the three months ended March 31, 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	1,172.1	\$ 6.38		
Options exercised	(222.0)	\$ 3.77		
Options forfeited	(235.2)	\$ 10.26		
Balance at March 31, 2012	7,368.4	\$ 6.78	7.7 years	\$ 3.0
Vested and unvested expected to vest, March 31, 2012	6,886.3	\$ 6.84	7.6 years	\$ 2.7
Exercisable at March 31, 2012	3,481.8	\$ 7.86	6.2 years	\$ 1.5

### **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 contains customary terms and conditions, including a financial covenant whereby the Company must maintain a minimum amount of liquidity measured at the end of each month 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 covenant as the 2009 Loan Agreement. The Company has at all times been in compliance with these covenants during the term of both agreements.

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At March 31, 2012, the total amount due under the 2009 Loan Agreement and the 2011 Loan Agreement was \$1.7 million. The carrying amount of the Company's borrowings approximates fair value at March 31, 2012.

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**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 March 31, 2012. No transfers of assets between Level 1 and Level 2 of the fair value measurement hierarchy occurred during the three months ended March 31, 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 March 31, 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

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each input for the Black-Scholes model against the fair value hierarchy criteria and using the lowest level of input as the basis for the fair value classification. There are six inputs: closing price of Amicus stock on the day of evaluation; the exercise price of the warrants; the remaining term of the warrants; the volatility of Amicus stock over that term; annual rate of dividends; and the riskless rate of return. Of those inputs, the exercise price of the warrants and the remaining term are readily observable in the warrant agreements. The annual rate of dividends is based on the Company's historical practice of not granting dividends. The closing price of Amicus stock would fall under Level 1 of the fair value hierarchy as it is a quoted price in an active market. The riskless rate of return is a Level 2 input, while the historical volatility is a Level 3 input in accordance with the fair value accounting guidance. Since the lowest level input is a Level 3, the Company determined the warrant liability is most appropriately classified within Level 3 of the fair value hierarchy. This liability is subject to fair value mark-to-market adjustment each period. As a result, the Company recognized the change in the fair value of the warrant liability as non-operating expense of \$2.4 million for the three months ended March 31, 2012. The resulting fair value of the warrant liability at March 31, 2012 was \$4.3 million.

A summary of the fair value of the Company's assets and liabilities aggregated by the level in the fair value hierarchy within which those measurements fall as of March 31, 2012, are identified in the following table (in thousands):

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	Level 1	Level 2	Total
<b>Assets:</b>			
Cash/Money market funds	\$ 24,523	\$	\$ 24,523
Corporate debt securities		46,589	46,589
Commercial paper		36,734	36,734
Certificate of deposit		350	350
	\$ 24,523	\$ 83,673	\$ 108,196

	Level 1	Level 2	Level 3	Total
<b>Liabilities:</b>				
Secured debt	\$	\$ 1,726	\$	\$ 1,726
Warrants liability			4,324	4,324
	\$	\$ 1,726	\$ 4,324	\$ 6,050

The following table summarizes the changes in Level 3 liability for the three months ended March 31, 2012 (in thousands):

	Balance as of December 31, 2011	Increase in fair value	Balance as of March 31, 2012
Warrants liability	\$ 1,948	\$ 2,376	\$ 4,324

**Note 7. Collaborative Agreements***GSK*

On October 28, 2010, the Company entered into the License and Collaboration Agreement with Glaxo Group Limited, an affiliate of GSK, to develop and commercialize migalastat HCl. Under the terms of the License and 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 is eligible to receive further payments of approximately \$173.5 million upon the successful achievement of development, regulatory and commercialization milestones, as well as tiered double-digit royalties on global sales of migalastat HCl. Potential payments include up to (i) \$13.5 million related to the attainment of certain clinical development objectives and the acceptance of regulatory filings in select worldwide markets, (ii) \$80 million related to market approvals for migalastat HCl in selected territories throughout the world, and (iii) \$80 million associated with the achievement of certain sales thresholds. GSK and the Company are jointly funding development costs in accordance with an agreed upon development plan. The Company funded 50% of the development costs in 2011 and will fund only 25% of the development costs in 2012 and beyond, subject to annual and aggregate caps. Additionally, GSK purchased approximately 6.9 million shares of the Company's common stock at \$4.56 per share, a 30% premium on the average price per share of the Company's stock over a 60 day period preceding the closing date of the transaction. The total value of this equity investment to the Company was approximately \$31 million and represents a 14.8% ownership position in the Company as of March 31, 2012. Under the terms of the collaboration agreement, while the Company will collaborate with GSK, GSK has decision-making authority over clinical, regulatory and commercial matters. Additionally, GSK has primary responsibility for interactions with regulatory agencies and prosecuting applications for marketing and reimbursement approvals worldwide.

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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

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rights, the technology and know how transfer and the research services. As a result, the Company's common stock is considered a separate unit of accounting and was accounted for as an issuance of common stock. However, as the Company's common stock was sold at a premium to the market closing price, the premium amount paid over the market closing price was considered as additional consideration paid to the Company for the collaboration agreement and was included as consideration for the single unit of accounting identified above.

The total arrangement consideration which was allocated to the single unit of accounting identified above was \$33.2 million which consists of the upfront license payment of \$30 million and the premium over the closing market price of the common stock transaction of \$3.2 million. The Company will recognize this consideration as Collaboration Revenue on a straight-line basis over the development period of 5.2 years as included in the detailed development plan that was included in the collaboration agreement. The Company determined that the overall level of activity over the development period approximates a straight-line approach. At March 31, 2012, the Company recognized approximately \$9.2 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 collaboration agreement and determined that the contingent milestones are substantive milestones and will be recognized as revenue in the period that the milestone is achieved. The Company determined that the research based milestones are commensurate with the enhanced value of each delivered item as a result of the Company's specific performance to achieve the milestones. There is considerable effort underway to meet the specified milestones and complete the development of 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 relate to past performances when achieved and are reasonable relative to the other payment terms within the collaboration agreement, including the \$30 million upfront payment and the cost sharing arrangement.

**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 three months ended March 31, 2012 (in thousands):

	Balance as of December 31, 2011	Charges	Cash Payments	Adjustments	Balance as of March 31, 2012
Facilities consolidation	\$ 38		\$ (38)		\$

**Note 9. Subsequent Events**

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On April 16, 2012, the Company appointed William D. Chip Baird, III as Chief Financial Officer. Mr. Baird will be responsible for all Finance, Accounting and Investor Relations activities and will report directly to John F. Crowley, Chairman and Chief Executive Officer. Mr. Baird joins Amicus from PTC Therapeutics, Inc., where he worked for over 10 years in positions of increasing responsibility. He served as Chief Financial Officer for the last seven years, responsible for all areas of finance, investor and public relations, human resources, facilities and project management. During his tenure at PTC, Mr. Baird was instrumental in raising over \$500 million through a combination of venture capital, non-dilutive grant funding, collaborations with industry-leading pharmaceutical and biotech companies, and debt financings.



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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,
- 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 a License and Collaboration Agreement entered into in October 2010. Our partnership with GSK allows us to utilize GSK's significant expertise in clinical, regulatory, commercial and manufacturing matters in the development in migalastat HCl. In addition, the cost-sharing arrangements and potential milestone and royalty payments under the License and Collaboration Agreement provide us with financial strength and allow us to continue the development of 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 third quarter of 2012. We plan to use the data from Study 011 to support subsequent marketing applications for the FDA and other regulatory

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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, although timelines may be influenced by the ERT shortage.

In addition to potential benefits pharmacological chaperones may provide as a monotherapy, we also believe the use of pharmacological chaperones co-administered with ERT may address certain key limitations of ERT. The use of pharmacological chaperones co-administered with ERT may significantly enhance the safety and efficacy of ERT by, among other effects, prolonging the half-life of infused enzymes in the circulation, increasing uptake of the 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).

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 GCCase enzyme are the most common genetic risk factor for Parkinson's. By targeting GCCase in the brain, AT3375 could potentially treat Gaucher, Parkinson's disease in Gaucher carriers, and possibly the general Parkinson's population. By year-end 2012, Amicus expects to complete preclinical and IND-enabling studies of AT3375, which are supported in part by a grant from the Michael J. Fox Foundation.

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 March 31, 2012, we have accumulated a deficit of \$283.2 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 six-month, placebo-controlled Phase 3 study of migalastat HCl that completed enrollment of 67 patients with Fabry disease. Results from this study are anticipated in the third quarter of 2012 to support subsequent marketing applications for the U.S. Food and Drug Administration (FDA) and other regulatory agencies.

Study 012 is a randomized, open-label, 18-month Phase 3 study investigating the safety and efficacy of migalastat HCl compared to current standard-of-care ERTs Fabrazyme (agalsidase beta) or Replagal (agalsidase 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 April 30, 2012, 38 of 40 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. There are also more than 150 patient-years of experience from the Phase 2 and Phase 2 extension studies, and ongoing Phase 3 and Phase 3 extension studies of migalastat HCl for Fabry disease.

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

*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 provide an update from this study in the third quarter 2012 and to present final 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. Enrollment has been completed for the first two dose groups and results from Study 010 are anticipated in the first half of 2012.

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*

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We are also investigating chaperone-ERT combinations as potential next-generation treatments for Gaucher and other undisclosed lysosomal storage diseases 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 October 28, 2010, we entered into the License and Collaboration Agreement with Glaxo Group Limited, an affiliate of GSK, to develop and commercialize migalastat HCl. Under the terms of the License and Collaboration Agreement, GSK received an exclusive worldwide license to develop, manufacture and commercialize migalastat HCl. In consideration of the license grant, we received an upfront, license payment of \$30 million from GSK and we are eligible to receive further payments of up to \$173.5 million upon the successful achievement of development, regulatory and commercialization milestones, as well as tiered double-digit royalties on global sales of migalastat HCl. Potential payments include up to (i) \$13.5 million related to the attainment of certain clinical development objectives and the acceptance of regulatory filings in select worldwide markets, (ii) \$80 million related to market approvals for migalastat HCl in selected territories throughout the world, and (iii) \$80 million associated with the achievement of certain sales thresholds. We and GSK are jointly funding development costs in accordance with an agreed upon development plan pursuant to which we funded 50% of the development costs in 2011 and we will fund only 25% of the development costs

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in 2012 and beyond, subject to annual and aggregate caps. Additionally, GSK purchased approximately 6.9 million shares of our common stock at a price of \$4.56 per share. The total value of this equity investment to us was approximately \$31 million and represents a 14.8% ownership position in the Company as of March 31, 2012. Under the terms of the collaboration agreement, while we will collaborate with GSK, GSK has decision-making authority over clinical, regulatory and commercial matters. Additionally, GSK has primary responsibility for interactions with regulatory agencies and prosecuting applications for marketing and reimbursement approvals worldwide.

***Other Potential Alliances and Collaborations***

We continually evaluate other potential collaborations and business development opportunities that would bolster our ability to develop and commercialize 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 Revenue on a straight-line basis over the development period of the collaboration agreement which is approximately 5.2 years. For the three months ended March 31, 2012, we recognized approximately \$1.7 million of the total upfront consideration as Collaboration Revenue and approximately \$6.1 million 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 License and 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;

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- 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



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inception in February 2002 through March 31, 2012, we have incurred research and development expense in the aggregate of \$279.6 million.

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

Projects	Three Months Ended		Period from February 4, 2002 (inception) to March 31, 2012
	2011	March 31, 2012	
Third party direct project expenses			
Migalastat HCl (Fabry Disease Phase 3)	\$ 4,062	\$ 5,709	\$ 71,044
Afegostat tartrate (Gaucher Disease Phase 2*)	(200)	27	26,142
AT2220 (Pompe Disease Phase 2)	4	(3)	13,240
Neurodegenerative Diseases (Preclinical)	451	217	8,825
Co-Administration studies (Fabry & Pompe Disease - Phase 2; Gaucher Preclinical)	55	868	3,818
Total third party direct project expenses	4,372	6,818	123,069
Other project costs (1)			
Personnel costs	4,923	5,442	98,693
Other costs (2)	1,830	1,744	57,862
Total other project costs	6,753	7,186	156,555
Total research and development costs	\$ 11,125	\$ 14,004	\$ 279,624

(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;

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- the results of our clinical trials; and
- any mandate by the U.S. Food and Drug Administration (FDA) or other regulatory authority to conduct clinical trials beyond those currently anticipated.

Our expenditures are subject to additional uncertainties, including the terms and timing of regulatory approvals, and the expense of filing, prosecuting, defending and enforcing any patent claims or other intellectual property rights. We may obtain unexpected results from our clinical trials. We may elect to discontinue, delay or modify clinical trials of some product candidates or focus on others. In addition, GSK has considerable influence over and decision-making authority related to our 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 we currently anticipate, or if we experience significant delays in enrollment in any of our clinical trials, we could be

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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 March 31, 2012, we spent \$117.3 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 March 31, 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***

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

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

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

*Accrued Expenses*

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

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

*Stock-Based Compensation*

We 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			
	2011		March 31, 2012	
Expected stock price volatility		78.8%		78.4%
Risk free interest rate		2.3%		1.1%
Expected life of options (years)		6.25		6.25
Expected annual dividend per share	\$	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 March 31, 2011 and 2012 are as follows:

	March 31, 2011		March 31, 2012	
	Expected stock price volatility		77.9%	
Risk free interest rate		1.24%		0.31%
Expected life of warrants (years)		2.92		1.92
Expected annual dividend per share	\$	0.00	\$	0.00

**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.

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

(In thousands, except per share amount)	Three Months Ended	
	2011	March 31, 2012
<b>Historical</b>		
Numerator:		
Net loss attributable to common stockholders	\$ (13,350)	\$ (13,137)
Denominator:		
Weighted average common shares outstanding - basic and diluted	34,498,926	37,887,520

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 7.7 million and 9.2 million for the three months ended March 31, 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 March 31, 2012 Compared to Three Months Ended March 31, 2011*

*Revenue.* For the three months ended March 31, 2012, we recognized \$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 Revenue on a straight-line basis over the development period of the collaboration agreement which is approximately 5.2 years. Research revenue was \$6.1 million for the three months ended March 31, 2012, representing an increase of \$1.8 million or 42% from the \$4.3 million for the three months ended March 31, 2011. The increase in research revenue was due to the increase in GSK's reimbursement rate from 50% to 75%, effective on January 1, 2012.

*Research and Development Expense.* Research and development expense was \$14.0 million for the three months ended March 31, 2012, representing an increase of \$2.9 million or 26% from \$11.1 million for the three months ended March 31, 2011. The variance was primarily attributable to increases in contract research and manufacturing costs due to the increased activity within the Fabry program program, higher personnel costs and increases in consulting costs.

*General and Administrative Expense.* General and administrative expense was \$4.1 million for the three months ended March 31, 2012, representing a decrease of \$0.3 million or 7% from \$4.4 million for the three months ended March 31, 2011. The variance was primarily due to lower personnel costs, and a decrease in consulting fees and professional fees.



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*Interest Income and Interest Expense.* Interest income was \$0.03 million for the three months ended March 31, 2012, representing a decrease of \$0.03 million or 50% from \$0.06 million for the three months ended March 31, 2011. The decrease was due to the overall lower average cash and investment balances. Interest expense was approximately \$0.04 million for the three months ended March 31, 2012 compare to \$0.05 for the three months ended March 31, 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

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value of the warrants are reported in the statements of operations as non-operating income or expense. For the three months ended March 31, 2012, we reported an expense of \$2.4 million related to the increase in fair value of these warrants as compared to an expense of \$3.4 million for the three months ended March 31, 2011, representing a decrease of \$1.0 million or 29%. The decrease was due to the fluctuations in the price of our common stock.

*Other Income/Expense.* Other income of \$0.07 million in the first three months ended March 31, 2011 represented funds received from the U.S. Treasury Department in February 2011 for the Qualified Therapeutic Discovery Projects tax credit and grant program. There was no other income/expense for the three months ended March 31, 2012.

**Liquidity and Capital Resources***Source of Liquidity*

As a result of our significant research and development expenditures and the lack of any approved products to generate product sales revenue, we have not been profitable and have generated operating losses since we were incorporated in 2002. We have funded our operations principally with \$148.7 million of proceeds from redeemable convertible preferred stock offerings, \$75.0 million of gross proceeds from our IPO in June 2007, \$18.5 million of gross proceeds from our Registered Direct Offering in March 2010, \$80.0 million from the non-refundable license fees from the collaboration agreements, \$31.0 million from GSK's investment in the Company at the time the collaboration was formed and \$65.6 million of gross proceeds from our stock offering in March 2012. In the future, we expect to fund our operations, in part, through the receipt of cost-sharing and milestone payments from GSK. The following table summarizes our significant funding sources as of March 31, 2012:

<b>Funding</b>	<b>Year</b>	<b>No. Shares</b>	<b>Approximate Amount (1) (in thousands)</b>
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 (Initial public offering)	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 Sold to GSK	2010	6,866,245	31,285
Common Stock Offering	2012	11,500,000	65,550
		44,425,490	\$ 419,023

(1) Represents gross proceeds

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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 \$9.2 million. We also received \$31.1million 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 March 31, 2012, we had cash, cash equivalents and marketable securities of \$108.2 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.

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***Net Cash Used in Operating Activities***

Net cash used in operations for the three months ended March 31, 2011 was \$13.3 million due primarily to the net loss for the three months ended March 31, 2011 of \$13.4 million and the change in operating assets and liabilities of \$5.2 million. The change in operating assets and liabilities consisted of an increase in receivables from GSK related to the collaboration agreement of \$4.0 million; a decrease in deferred revenue of \$0.3 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 \$0.9 million related to program expenses.

Net cash used in operations for the three months ended March 31, 2012 was \$8.0 million, due primarily to the net loss for the three months ended March 31, 2012 of \$13.1 million and the change in operating assets and liabilities of \$0.9 million. The change in operating assets and liabilities consisted of a decrease in receivables from GSK related to the collaboration agreement of \$0.7 million; a decrease of \$3.1 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 \$2.0 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 Provided by/ (Used in) Investing Activities***

Net cash provided by investing activities for the three months ended March 31, 2011 was \$11.7 million. Net cash provided by investing activities reflects \$30.9 million for the sale and redemption of marketable securities partially offset by \$19.1 million for the purchase of marketable securities and \$0.1 million for the acquisition of property and equipment.

Net cash used in investing activities for the three months ended March 31, 2012 was \$56.7 million. Net cash used in investing activities reflects \$67.9 million for the purchase of marketable securities and \$3.1 million for the acquisition of property and equipment in connection with our move to new office and laboratory space, partially offset by \$14.3 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 three months ended March 31, 2011 was \$0.3 million, consisting primarily of payments of our secured loan agreement and capital lease obligations.

Net cash provided by financing activities for the three months ended March 31, 2012 was \$63.6 million, consisting of \$62.1 million from March 2012 stock offering, \$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 offset by the payments of our secured loan agreement of \$0.3 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;
- our ability to achieve development and commercialization milestone payments and sales royalties under our collaboration with GSK;
- the scope, progress, results and costs of preclinical development, laboratory testing and clinical trials for our product candidates including those testing the use of pharmacological chaperones co-administered with ERT and for the treatment of diseases of neurodegeneration;
- the costs, timing and outcome of regulatory review of our product candidates;

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- 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.

***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. 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 products and believe we are at least a couple years away from selling any products that would require us to make any such royalty payments.

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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.

### **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 March 31, 2012, we held \$108.2 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

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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.

**PART II. OTHER INFORMATION**

**ITEM 1. LEGAL PROCEEDINGS**

We are not a party to any material legal proceedings.

**ITEM 1A. RISK FACTORS**

There have been no material changes with respect to the Risk Factors disclosed in our Annual Report on Form 10-K for the year ended December 31, 2011.



**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.

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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 March 31, 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 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 March 31, 2012, we have used approximately \$17.1 million of the net proceeds to further advance the development of our lead product candidate, migalastat HCl, 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 underwriters for the offering.

We paid Leerink Swann LLC and Cowen and Company an underwriting 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 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 March 31, 2012, we had invested the \$62.0 million in net proceeds in money market funds and in investment-grade, interest bearing instruments, pending their use. Through March 31, 2012, we have not used any of 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 and co-administration programs, especially our lead program migalastat HCl for Fabry disease; to potentially enter into collaborations, alliances and

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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 March 31, 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
10.1 (3)	Letter Agreement dated March 5, 2012 between Amicus Therapeutics, Inc. and William D. Baird
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

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(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

(3) Incorporated by reference to Exhibit 10.1 to our Current Report on Form 8-K filed on April 16, 2012.

\* 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: May 10, 2012

By: /s/ JOHN F. CROWLEY

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

Date: May 10, 2012

By: /s/ WILLIAM D. BAIRD III

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

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**INDEX TO EXHIBITS**

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3.2 (2)	Amended and Restated By-laws
10.1 (3)	Letter Agreement dated March 5, 2012 between Amicus Therapeutics, Inc. and William D. Baird
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

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(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

(3) Incorporated by reference to Exhibit 10.1 to our Current Report on Form 8-K filed on April 16, 2012.

\* 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.