

AMICUS THERAPEUTICS INC  
Form 10-Q  
November 12, 2013  
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**UNITED STATES**  
**SECURITIES AND EXCHANGE COMMISSION**

Washington, D.C. 20549

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**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 September 30, 2013

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 October 31, 2013 was 49,631,672 shares.



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We have registered or filed applications to register certain trademarks in the United States and abroad, including AMICUS , AMICUS THERAPEUTICS (and design) and CHART (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,” “should,” “would” and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.

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

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

We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make. We have included important factors in the cautionary statements included in Part I Item 1A “Risk Factors” of the Annual Report on Form 10-K for the year ended December 31, 2012 that we believe could cause actual results or events to differ materially from the forward-looking statements that we make. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, joint ventures, collaborations or investments we may make.

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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)**

	December 31, 2012	September 30, 2013
<b>Assets:</b>		
Current assets:		
Cash and cash equivalents	\$ 33,971	\$ 30,047
Investments in marketable securities	65,151	30,448
Receivable due from GSK	3,225	2,121
Prepaid expenses and other current assets	2,270	1,692
Total current assets	104,617	64,308
Property and equipment, less accumulated depreciation and amortization of \$8,501 and \$9,751 at December 31, 2012 and September 30, 2013, respectively	5,029	4,356
Other non-current assets	442	442
<b>Total Assets</b>	<b>\$ 110,088</b>	<b>\$ 69,106</b>
<b>Liabilities and Stockholders' Equity</b>		
Current liabilities:		
Accounts payable and accrued expenses	\$ 8,845	\$ 8,166
Current portion of secured loan	398	398
Warrant liability		34
Total current liabilities	9,243	8,598
Deferred reimbursements	30,418	34,019
Warrant liability, non-current	908	
Secured loan, less current portion	299	
Commitments and contingencies		
Stockholders' equity:		
Common stock, \$.01 par value, 125,000,000 shares authorized, 49,631,672 shares issued and outstanding at December 31, 2012, 125,000,000 shares authorized, 49,631,672 shares issued and outstanding at September 30, 2013	556	556
Additional paid-in capital	387,539	392,213
Accumulated other comprehensive income	14	5



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Deficit accumulated during the development stage		(318,889)		(366,285)
Total stockholders' equity		69,220		26,489
<b>Total Liabilities and Stockholders' Equity</b>	<b>\$</b>	<b>110,088</b>	<b>\$</b>	<b>69,106</b>

*See accompanying notes to consolidated financial statements*

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

	Three Months Ended September 30,		Nine Months Ended September 30,		Period from February 4, 2002 (inception) to September 30,
	2012	2013	2012	2013	2013
Revenue:					
Research revenue	\$	\$ 39	\$ 11,591	\$ 39	\$ 57,532
Collaboration and milestone revenue			6,820		64,382
Total revenue	\$	\$ 39	\$ 18,411	\$ 39	\$ 121,914
Operating Expenses:					
Research and development	\$ 11,499	\$ 10,110	\$ 39,226	\$ 32,824	\$ 348,717
General and administrative	4,995	4,635	14,909	14,288	146,901
Restructuring charges					1,522
Impairment of leasehold improvements					1,030
Depreciation and amortization	422	429	1,284	1,318	13,086
In-process research and development					418
Total operating expenses	16,916	15,174	55,419	48,430	511,674
Loss from operations	(16,916)	(15,135)	(37,008)	(48,391)	(389,760)
Other income (expenses):					
Interest income	92	36	235	147	14,536
Interest expense	(19)	(7)	(77)	(26)	(2,448)
Change in fair value of warrant liability	553	517	(1,941)	874	2,427
Other income			21		252
Loss before tax benefit	(16,290)	(14,589)	(38,770)	(47,396)	(374,993)
Income tax benefit					8,708
Net loss	(16,290)	(14,589)	(38,770)	(47,396)	(366,285)
Deemed dividend					(19,424)
Preferred stock accretion					(802)
Net loss attributable to common stockholders	\$ (16,290)	\$ (14,589)	\$ (38,770)	\$ (47,396)	\$ (386,511)
Net loss attributable to common stockholders per common shares basic and diluted	\$ (0.34)	\$ (0.29)	\$ (0.88)	\$ (0.96)	
Weighted-average common shares outstanding basic and diluted	48,513,647	49,621,188	44,255,885	49,621,188	

*See accompanying notes to consolidated financial statements*



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

	<b>Three Months Ended September 30,</b>		<b>Nine Months Ended September 30,</b>		<b>Period from February 4, 2002 (inception) to September 30, 2013</b>
	<b>2012</b>	<b>2013</b>	<b>2012</b>	<b>2013</b>	
Net loss	\$ (16,290)	\$ (14,589)	\$ (38,770)	\$ (47,396)	\$ (366,285)
Other comprehensive (loss)/income:					
Unrealized gain/(loss) on available-for-sale securities	20	2	33	(9)	5
Other comprehensive income/(loss), before income taxes	20	2	33	(9)	5
Provision for income taxes related to other (loss)/comprehensive income items (a)					
Other comprehensive income/(loss)	\$ 20	\$ 2	\$ 33	\$ (9)	\$ 5
Comprehensive loss	\$ (16,270)	\$ (14,587)	\$ (38,737)	\$ (47,405)	\$ (366,280)

(a) 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)**

	Nine Months Ended September 30,		Period from February 4, 2002 (inception) to September 30, 2013
	2012	2013	
<b>Operating activities</b>			
Net loss	\$ (38,770)	\$ (47,396)	\$ (366,285)
Adjustments to reconcile net loss to net cash used in operating activities:			
Non-cash interest expense			525
Depreciation and amortization	1,284	1,318	13,086
Amortization of non-cash compensation			522
Stock-based compensation - employees	4,743	4,674	46,603
Stock-based compensation - non-employees			853
Stock-based license payments			1,220
Change in fair value of warrant liability	1,941	(874)	(2,427)
Loss on disposal of asset	28		388
Impairment of leasehold improvements			1,030
Non-cash charge for in-process research and development			418
Beneficial conversion feature related to bridge financing			135
Changes in operating assets and liabilities:			
Receivable due from GSK	1,859	1,104	(2,121)
Prepaid expenses and other current assets	2,826	578	(1,692)
Other non-current assets	267		(466)
Accounts payable and accrued expenses	(280)	(679)	8,166
Deferred reimbursements	(268)	3,601	34,019
Net cash used in operating activities	(26,370)	(37,674)	(266,026)
<b>Investing activities</b>			
Redemption of marketable securities	47,445	68,348	823,790
Purchases of marketable securities	(99,320)	(33,654)	(854,349)
Purchases of property and equipment	(4,167)	(645)	(18,858)
Net cash (used in)/ provided by investing activities	(56,042)	34,049	(49,417)
<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	80,195		193,441
Proceeds from the issuance of convertible notes			5,000
Payments of capital lease obligations			(5,587)
Payments of secured loan agreement	(1,139)	(299)	(4,355)
Proceeds from exercise of stock options	966		3,341
Proceeds from exercise of warrants (common and preferred)			264
Proceeds from capital asset financing arrangement			5,611

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Proceeds from secured loan arrangement	995			4,753
Net cash provided by/ (used in) financing activities	81,017	(299)		345,490
Net (decrease)/ increase in cash and cash equivalents	(1,395)	(3,924)		30,047
Cash and cash equivalents at beginning of period	25,668	33,971		
Cash and cash equivalents at end of period	\$ 24,273	\$ 30,047	\$	30,047

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**Amicus Therapeutics, Inc.**

**(a development stage company)**

**Consolidated Statements of Cash Flows (continued)**

*(Unaudited)*

**(in thousands)**

	Nine Months Ended September 30,		Period from February 4, 2002 (inception) to September 30, 2013
	2012	2013	
<b>Supplemental disclosures of cash flow information</b>			
Cash paid during the period for interest	\$ 72	\$ 24	\$ 2,141
<b>Non-cash activities</b>			
Conversion of warrants to common stock	\$	\$	\$ 386
Conversion of notes payable to Series B redeemable convertible 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. ( we, us, our, Amicus or the Company ) was incorporated on February 4, 2002 in Delaware and is a biopharmaceutical company focused on the discovery, development and commercialization of next-generation medicines for a range of rare and orphan diseases, with a focus on improved therapies for lysosomal storage diseases ( LSDs ). The Company's lead program is migalastat HCl for Fabry disease. Migalastat HCl is a novel, small molecule pharmacological chaperone in development as a monotherapy and in combination with ERT for Fabry disease. The Company is leveraging its Chaperone-Advanced Replacement Therapy, or CHART platform to develop next-generation therapies that combine pharmacological chaperones with enzyme therapies for Pompe, Mucopolysaccharidosis Type I ( MPS I ) and Gaucher diseases. Current CHART programs for Pompe disease include the pharmacological chaperone AT2220 (duvoglustat HCl) co-administered with currently marketed Pompe ERTs (Myozyme®/Lumizyme®), as well as AT2220 co-formulated with a proprietary Pompe ERT. The Company's activities since inception have consisted principally of raising capital, establishing facilities, and performing research and development. Accordingly, the Company is considered to be in the development stage.

On September 10, 2013, the Company entered into a collaboration agreement with Biogen Idec ( Biogen ) to discover, develop and commercialize novel small molecules for the treatment of Parkinson's disease. The collaboration will build upon preclinical studies at the Company and independent published research that suggest increasing activity of the lysosomal enzyme glucocerebrosidase ( GCase ) in the brain may correct alpha-synuclein pathology and other deficits associated with Parkinson's disease. Under terms of the multi-year agreement, the Company and Biogen will collaborate in the discovery of a new class of small molecules that target the GCase enzyme, for further development and commercialization by Biogen. Biogen will be responsible for funding all discovery, development, and commercialization activities. In addition the Company will be reimbursed for all full-time employees working on the project. The Company is also eligible to receive development and regulatory milestones, as well as modest royalties on global net sales.

On July 17, 2012, the Company entered into an Amended and Restated License and Expanded Collaboration Agreement (the Expanded Collaboration Agreement ) with an affiliate of GSK pursuant to which the Company and GSK continue to develop and commercialize migalastat HCl for the treatment of Fabry disease. The Expanded Collaboration Agreement amends and replaces in its entirety the License and Collaboration Agreement entered into between the Company and GSK on October 28, 2010 (the Original Collaboration Agreement ) for the development and commercialization of migalastat HCl. Under the terms of the Expanded Collaboration Agreement, the Company and GSK are co-developing all formulations of migalastat HCl for Fabry disease, including migalastat HCl monotherapy, currently in Phase 3 development, as well as migalastat HCl co-formulated with an investigational ERT for Fabry disease (the Co-formulated Product ). The Co-formulated Product is being developed by the Company and GSK in collaboration with JCR Pharmaceutical Co. Ltd. The Company will commercialize all migalastat HCl products for Fabry disease in the United States while GSK will commercialize all such products in the rest of the world.

The Company and GSK jointly fund development costs for all formulations of migalastat HCl in accordance with agreed upon development plans pursuant to which the Company and GSK fund 40% and 60% of such costs, respectively, for 2013 and through the end of the development plans. Additionally, simultaneous with entry into the Expanded Collaboration Agreement, the Company and GSK entered into a Stock Purchase Agreement (the SPA ) pursuant to which GSK purchased approximately 2.9 million shares of the Company's common stock at a price of \$6.30 per share. The total value of this equity investment to the Company is approximately \$18.6 million. As of September 30, 2013, GSK's ownership position in the Company is approximately 19.8%.

For further information, see Note 7. Collaborative Agreements



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The Company had an accumulated deficit of approximately \$366.3 million at September 30, 2013 and anticipates incurring losses through the fiscal year ending December 31, 2013 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

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redeemable convertible preferred stock, issuance of convertible notes, net proceeds from its initial public offering ( IPO ) and subsequent stock offerings, payments from partners during the terms of the collaboration agreements and other financing arrangements. In March 2010, the Company sold 4.95 million shares of its common stock and also sold warrants to purchase 1.9 million shares of common stock in a registered direct offering to a select group of institutional investors for net proceeds of \$17.1 million. In October 2010, the Company sold 6.87 million shares of its common stock as part of the Original Collaboration Agreement with GSK for proceeds of \$31.0 million. In March 2012, the Company sold 11.5 million shares of its common stock in a stock offering for net proceeds of \$62.0 million. In July 2012, the Company sold 2.9 million shares of its common stock as part of the Expanded Collaboration Agreement with GSK for proceeds of \$18.6 million. The Company believes that its existing cash and cash equivalents and short-term investments will be sufficient to cover its cash flow requirements into the fourth quarter of 2014.

***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 Regulation S-X. Accordingly, the unaudited consolidated financial statements do not include all of the information and disclosures required by U.S. GAAP for complete financial statements. In the opinion of management, the accompanying unaudited financial statements reflect all adjustments, which include only normal recurring adjustments, necessary to present fairly the Company's interim financial information.

The accompanying unaudited consolidated financial statements and related notes should be read in conjunction with the Company's financial statements and related notes as contained in the Company's Annual Report on Form 10-K for the year ended December 31, 2012. 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, 2012.

***Revenue Recognition***

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

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

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

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

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

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

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

For the reimbursements for research and development costs under collaboration agreements that meet the criteria for revenue recognition the Company has the following accounting policies:

- Research and development expenses related to a collaboration agreement will be recorded on a gross basis in the income statement and not presented net of any reimbursement received from a collaboration agreement; and
- The reimbursement of research and development expenses from a collaborator will be recognized in the income statement as Research Revenue for the period in which the research activity occurred.

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

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criteria are met then the contingent milestones can be considered as substantive milestones and will be recognized as revenue in the period that the milestone is achieved.

### ***Fair Value Measurements***

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

Level 1 inputs utilize quoted prices (unadjusted) in active markets for identical assets or liabilities that the Company has the ability to access at measurement date. Level 2 inputs are inputs other than quoted prices included in Level 1 that are observable for the asset or liability, either directly or indirectly. Level 2 inputs may include

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

*New Accounting Standards*

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

**Note 2. Cash, Money Market Funds and Marketable Securities**

As of September 30, 2013, the Company held \$30.0 million in cash and cash equivalents and \$30.4 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) in the statements of comprehensive loss. If a decline in the fair value of a marketable security below the Company's cost basis is determined to be other than temporary, such marketable security is written down to its estimated fair value as a new cost basis and the amount of the write-down is included in earnings as an impairment charge. To date, only temporary impairment adjustments have been recorded.

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

Cash and available for sale securities consisted of the following as of December 31, 2012 and September 30, 2013 (in thousands):

	As of December 31, 2012				
	Cost	Unrealized Gain	Unrealized Loss	Fair Value	
Cash balances	\$ 33,971	\$	\$	\$ 33,971	
Corporate debt securities	42,503	5	(11)	42,497	

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Commercial paper	19,725	19	19,744
Certificate of deposit	2,909	1	2,910
	\$ 99,108	\$ 25	\$ (11) \$ 99,122
Included in cash and cash equivalents	\$ 33,971	\$	\$ 33,971
Included in marketable securities	65,137	25	(11) 65,151
Total cash and available for sale securities	\$ 99,108	\$ 25	\$ (11) \$ 99,122

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	Cost	As of September 30, 2013		Fair Value
		Unrealized Gain	Unrealized Loss	
Cash balances	\$ 30,047	\$ 2	\$ (4)	\$ 30,047
Corporate debt securities	21,100	7		21,098
Commercial paper	8,993			9,000
Certificate of deposit	350			350
	\$ 60,490	\$ 9	\$ (4)	\$ 60,495
Included in cash and cash equivalents	\$ 30,047	\$	\$	\$ 30,047
Included in marketable securities	30,443	9	(4)	30,448
Total cash and available for sale securities	\$ 60,490	\$ 9	\$ (4)	\$ 60,495

Unrealized gains and losses are reported as a component of other comprehensive income/(loss) in the statements of comprehensive loss. For the year ended December 31, 2012, unrealized holding gains included in the statement of comprehensive loss were approximately \$10,000. For the nine months ended September 30, 2013, unrealized holding losses included in the statements of comprehensive loss were approximately \$9,000.

For the year ended December 31, 2012 and the nine months ended September 30, 2013, there were no realized gains or losses. The cost of securities sold is based on the specific identification method.

Unrealized loss positions in the available for sale securities as of December 31, 2012 and September 30, 2013 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 \$33.1 million and \$14.8 million as of December 31, 2012 and September 30, 2013, respectively.

The Company holds available-for-sale investment securities which are reported at fair value on the Company's balance sheet. Unrealized holding gains and losses are reported within accumulated other comprehensive income ( AOCI ) in the statements of comprehensive loss. The changes in AOCI associated with the unrealized holding gain on available-for-sale investments during the three and nine month period ended September 30, 2012 and 2013, were as follows (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2012	2013	2012	2013
Balance, beginning	\$ 17	\$ 3	\$ 4	\$ 14
Current period changes in fair value, (a)	20	2	33	(9)
Reclassification of earnings, (a)				
Balance, ending	\$ 37	\$ 5	\$ 37	\$ 5

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



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

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

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

(In thousands, except share amounts)	Three Months Ended September 30,		Nine Months Ended September 30,	
	2012	2013	2012	2013
<b>Historical</b>				
Numerator:				
Net loss attributable to common stockholders	\$ (16,290)	\$ (14,589)	\$ (38,770)	\$ (47,396)
<b>Denominator</b>				
Weighted average common shares outstanding basic and diluted	48,513,647	49,621,188	44,255,885	49,621,188

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 10.4 million and 11.5 million for the nine months ended September 30, 2012 and 2013, respectively. Potentially dilutive common stock equivalents were excluded from the diluted earnings per share denominator for all periods because of their anti-dilutive effect.

**Note 4. Stockholders' Equity*****Common Stock and Warrants***

As of September 30, 2013, the Company was authorized to issue 125 million shares of common stock. Dividends on common stock will be paid when, and if declared by the Company's board of directors. Each holder of common stock is entitled to vote on all matters and is entitled to one vote for each share held.

In July 2012, Amicus and GSK entered into the SPA pursuant to which GSK purchased 2.9 million unregistered shares of the Company's common stock at a price of \$6.30 per share. The total purchase price for these shares was \$18.6 million. The Company received all proceeds from the sale of such shares on July 26, 2012. As of September 30, 2013, GSK had a 19.8% ownership position in the Company.

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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 Securities and Exchange Commission (the SEC) on May 27, 2009. The aggregate offering proceeds were \$65.6 million.

In October 2010, GSK purchased approximately 6.9 million shares of the Company's common stock at \$4.56 per share, in connection with the Original Collaboration Agreement. The total value of this equity investment was approximately \$31.0 million.

In March 2010, the Company sold 4.9 million shares of its common stock and warrants to purchase 1.9 million shares of common stock in a registered direct offering to a selected group of institutional investors through a Registration Statement on Form S-3 that was declared effective by the SEC on May 27, 2009. The shares of

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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 nine month anniversary of the date they were issued, at an exercise price of \$4.43 per share. The gross offering proceeds were \$18.5 million. There were approximately 1.4 million warrants outstanding at September 30, 2013.

## *Stock Option Plans*

During the three and nine months ended September 30, 2013, the Company recorded stock based compensation expense of approximately \$1.5 million and \$4.7 million, respectively. The stock-based compensation expense had no impact on the Company's cash flows from operations and financing activities. As of September 30, 2013, the total unrecognized compensation cost related to non-vested stock options granted was \$10.0 million and is expected to be recognized over a weighted average period of 2.4 years. The following table summarizes information related to stock compensation expense recognized in the statements of operations (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2012	2013	2012	2013
Stock compensation expense recognized in:				
Research and development expense	\$ 955	\$ 824	\$ 2,748	\$ 2,634
General and administrative expense	643	693	1,995	2,040
Total stock compensation expense	\$ 1,598	\$ 1,517	\$ 4,743	\$ 4,674

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 September 30,		Nine Months Ended September 30,	
	2012	2013	2012	2013
Expected stock price volatility	76.2%	81.7%	77.5%	82.0%
Risk free interest rate	0.9%	1.8%	0.8%	1.3%
Expected life of options (years)	6.25	6.25	6.25	6.25
Expected annual dividend per share	\$ 0.00	\$ 0.00	\$ 0.00	\$ 0.00

A summary of option activities related to the Company's stock options for the nine months ended September 30, 2013 is as follows:

	Number of Shares (in thousands)	Weighted Average Exercise Price	Weighted Average Remaining Contractual Life	Aggregate Intrinsic Value (in thousands)
Balance at December 31, 2012	7,974.2	\$ 6.35		

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Options granted	2,458.8	\$	3.04		
Options exercised		\$			
Options forfeited	(321.8)	\$	5.08		
Balance at September 30, 2013	10,111.2	\$	5.58	7.3 years	\$ 37.8
Vested and unvested expected to vest, September 30, 2013	9,518.4	\$	5.69	7.2 years	\$ 37.8
Exercisable at September 30, 2013	5,501.2	\$	6.80	6.0 years	\$ 37.8

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

In August 2011, the Company entered into a loan and security agreement (the "2011 Loan Agreement") with Silicon Valley Bank (SVB), in order to finance certain capital expenditures that the Company made 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 \$3.0 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 under the 2011 Loan Agreement that will be repaid over the following 2.5 years. The 2011 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, are greater than \$20 million plus outstanding debt due to SVB. The Company has at all times been in compliance with these covenants during the term of the agreement.

At September 30, 2013, the total amount due under the 2011 Loan Agreement was \$0.4 million. The carrying amount of the Company's borrowings approximates fair value at September 30, 2013.

**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 or nine months ended September 30, 2013. No transfers of assets between Level 1 and Level 2 of the fair value measurement hierarchy occurred during the three or nine months ended September 30, 2013.

***Secured Debt***

As disclosed in Note 5, the Company entered into the 2011 Loan Agreement with SVB. The carrying amount of the Company's borrowings approximates fair value at September 30, 2013. The Company's secured debt is classified as Level 2 and the fair value is estimated using quoted prices for similar liabilities in active markets, as well as inputs that are observable for the liability (other than quoted prices), such as interest rates that are observable at commonly quoted intervals.

***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

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determined that the warrant liability should be classified within Level 3 of the fair value hierarchy by evaluating each input for the Black-Scholes model against the fair value hierarchy criteria and using the lowest level of input as the basis for the fair value classification. There are six inputs: closing price of the Company's stock on the day of evaluation; the exercise price of the warrants; the remaining term of the warrants; the volatility of the Company's 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 and approximately 0.5 million warrants were exercised in 2012. The weighted average assumptions used in the Black-Scholes valuation model for the warrants are as follows:

	Nine Months Ended September 30,			
	2012		2013	
Expected stock price volatility		76.0%		59.7%
Risk free interest rate		0.19%		0.03%
Expected life of warrants (years)		1.42		0.42
Expected annual dividend per share	\$	0.00	\$	0.00

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

	Balance as of December 31, 2012			
	Level 1	Level 2	Level 3	Total
<b>Assets:</b>				
Cash/Money market funds	\$ 33,971	\$	\$	33,971
Corporate debt securities		42,497		42,497
Commercial paper		19,744		19,744
Certificate of deposit		2,910		2,910
	\$ 33,971	\$ 65,151	\$	99,122
<b>Liabilities:</b>				
Secured debt	\$	\$ 697	\$	\$ 697
Warrants liability			908	908
	\$	\$ 697	\$ 908	\$ 1,605

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A summary of the fair value of the Company's assets and liabilities aggregated by the level in the fair value hierarchy within which those measurements fall as of September 30, 2013, are identified in the following table (in thousands):

	Balance as of September 30, 2013			
	Level 1	Level 2	Level 3	Total
<b>Assets:</b>				
Cash/Money market funds	\$ 30,047	\$	\$	30,047
Corporate debt securities		21,098		21,098
Commercial paper		9,000		9,000
Certificate of deposit		350		350
	\$ 30,047	\$ 30,448	\$	60,495
<b>Liabilities:</b>				
Secured debt	\$	398	\$	398
Warrants liability			34	34
	\$	398	\$ 34	\$ 432

The change in the fair value of the Level 3 liability was a decrease of \$0.5 million and \$0.6 million for the three months ended September 30, 2013, and 2012, respectively. The change in fair value for the Level 3 liability was a decrease of \$0.9 million and an increase of \$1.9 million for the nine months ended September 30, 2013 and 2012, respectively.

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

On October 28, 2010, the Company entered into the Original Collaboration Agreement with Glaxo Group Limited, an affiliate of GSK, to develop and commercialize migalastat HCl. Under the terms of the Original Collaboration Agreement, GSK received an exclusive worldwide license to develop, manufacture and commercialize migalastat HCl. In consideration of the license grant, the Company received an upfront, license payment of \$30.0 million from GSK and was eligible to receive further payments of approximately \$173.5 million upon the successful achievement of development, regulatory and commercialization milestones, as well as tiered double-digit royalties on global sales of migalastat HCl. Potential payments included up to (i) \$13.5 million related to the attainment of certain clinical development objectives and the acceptance of regulatory filings in select worldwide markets, (ii) \$80.0 million related to market approvals for migalastat HCl in selected territories throughout the world, and (iii) \$80.0 million associated with the achievement of certain sales thresholds. GSK and the Company were jointly funding development costs in accordance with an agreed upon development plan. Additionally, GSK purchased approximately 6.9 million shares of the Company's common stock at \$4.56 per share, a 30% premium on the average price per share of the Company's stock over a 60 day period preceding the closing date of the transaction. The total value of this equity investment to the Company was approximately \$31.0 million.

On July 17, 2012, the Company entered into the Expanded Collaboration Agreement with GSK pursuant to which the Company and GSK continue to develop and commercialize migalastat HCl, currently in Phase 3 development for the treatment of Fabry disease. The Expanded Collaboration Agreement amends and replaces in its entirety the Original Collaboration Agreement. Under the terms of the Expanded Collaboration Agreement, the Company and GSK will co-develop all formulations of migalastat HCl for Fabry disease, including the



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development of migalastat HCl co-formulated with an investigational ERT for Fabry disease in collaboration with another GSK collaborator JCR Pharmaceutical Co. Ltd. The Company will commercialize all migalastat HCl products for Fabry disease in the United States while GSK will commercialize all such products in the rest of the world.

The exclusive license granted to GSK under the Original Collaboration Agreement to commercialize migalastat HCl worldwide was replaced under the Expanded Collaboration Agreement, which grants two exclusive licenses:

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(i) an exclusive license from GSK to the Company to commercialize migalastat HCl in the United States, and (ii) an exclusive license from the Company to GSK to commercialize migalastat HCl in the rest of world. Amicus and GSK each have a license to manufacture migalastat HCl for commercialization of monotherapy and chaperone-ERT co-administration migalastat HCl products while GSK maintains an exclusive license to manufacture such products for development purposes (subject to limited exceptions) and to manufacture the Co-formulated Product. In the event of a change of control in the Company during the term of the Expanded Collaboration Agreement, GSK has the option to purchase an exclusive license to develop, manufacture and commercialize migalastat HCl in the United States.

GSK is eligible to receive U.S. regulatory approval milestones totaling \$20.0 million for migalastat HCl monotherapy and migalastat HCl-ERT co-administration, and additional regulatory approval and product launch milestone payments totaling up to \$35.0 million within seven years following the launch of the Co-formulated Product. The Company will also be responsible for certain pass-through milestone payments and single-digit royalties on the net U.S. sales of the Co-formulated Product that GSK must pay to a third party. In addition, the Company is no longer eligible to receive any milestones or royalties it would have been eligible to receive under the Original Collaboration Agreement other than a \$3.5 million clinical development milestone achieved in the second quarter of 2012 and paid by GSK to Amicus in the third quarter of 2012.

The Company and GSK will continue to jointly fund development costs for all formulations of migalastat HCl in accordance with agreed upon development plans pursuant to which the Company and GSK funded 25% and 75% of such costs, respectively, for the monotherapy and co-administration programs during 2012 and 40% and 60%, respectively, in 2013 and beyond. Effective upon entry into the Expanded Collaboration Agreement, costs for the development of the Co-formulated Product are also funded 40% and 60% between Amicus and GSK, respectively.

Additionally, simultaneous with entry into the Expanded Collaboration Agreement, Amicus and GSK entered into an SPA pursuant to which GSK purchased approximately 2.9 million shares of Amicus common stock at a price of \$6.30 per share. The SPA provides GSK with customary registration rights for the shares purchased and includes a six-month lock-up provision. The total purchase price was \$18.6 million and the Company received all proceeds from the sale of such shares on July 26, 2012. As of September 30, 2013, GSK had a 19.8% ownership position in the Company as a result of the Original Collaboration Agreement and the Expanded Collaboration Agreement.

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

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

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

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

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

Accordingly, the Company does not believe that, for accounting purposes, the new U.S. licensing rights to migalastat HCl obtained from GSK represent a separate, identifiable benefit from the licenses in the Original Collaboration Agreement. The contingent amounts payable to GSK are not sufficiently separable from GSK's original license and the research and development reimbursements such that Amicus could not have entered into a similar exchange transaction with another party. Additionally, the Company cannot reasonably estimate the fair value of the US licensing rights to migalastat HCl.

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

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

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

As a result, all revenue recognition was suspended until the total arrangement consideration becomes fixed and determinable. In addition, future milestone payments made by the Company will be applied against the balance of this deferred reimbursements account. In the third quarter of 2013, we paid GSK a pass-through milestone payment of \$0.8 million in connection with the development of the Co-formulated product. This payment is reflected as a reduction of the deferred reimbursements in the Consolidated Balance Sheet as of September 30, 2013.

Revenue recognition for research expense reimbursements, the original upfront license fee, and the equity premiums will resume once the total arrangement consideration becomes fixed and determinable which will occur when the balance of the deferred reimbursements account is

sufficient to cover all the remaining contingent milestone payments.

Under the Original Collaboration Agreement, the Company evaluated the contingent milestones and determined that they were substantive milestones and would be recognized as revenue in the period that the milestone is achieved. The Company determined that the research based milestones were commensurate with the enhanced value of each delivered item as a result of the Company's specific performance to achieve the milestones. The research-based milestones would have related to past performances when achieved and were reasonable in relation to the other payment terms within the Original Collaboration Agreement. In September 2012, the Company achieved a clinical development milestone and recognized \$3.5 million of milestone revenue. Under the terms of the Expanded Collaboration Agreement, the Company is no longer entitled to receive any milestone payments from GSK.

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*Biogen Idec*

On September 10, 2013, the Company entered into a license and collaboration agreement (the "Biogen Agreement") with Biogen to discover, develop and commercialize novel small molecules for the treatment of Parkinson's disease. Under terms of the multi-year agreement, the Company and Biogen will collaborate in the discovery of a new class of small molecules that target the GCase enzyme, for further development and commercialization by Biogen. Biogen will be responsible for funding all discovery, development, and commercialization activities. In addition the Company will be reimbursed for all full-time employees working on the project as part of a cost sharing arrangement. The Company is also eligible to receive development and regulatory milestones, as well as modest royalties in global net sales.

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

- Research and development expenses related to a collaboration agreement will be recorded on a gross basis in the income statement and not presented net of any reimbursement received from a collaboration agreement; and
- The reimbursement of research and development expenses from a collaborator will be recognized in the income statement as Research Revenue for the period in which the research activity occurred.

As of September 30, 2013, the Company recognized \$0.04 million in Research Revenue for work performed under the cost sharing arrangement of the Biogen Agreement.

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

**Note 8. Subsequent Events**

The Company evaluated events that occurred subsequent to September 30, 2013 through the date of issuance of these financial statements and concluded that there were no material or non-recognized events during this period.

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

**Overview**

We are a biopharmaceutical company at the forefront of therapies for rare and orphan diseases. We are developing novel, first-in-class treatments for a broad range of human genetic diseases, with a focus on delivering benefits to individuals with LSDs. Our development programs include novel small molecules as monotherapy treatments and in combination with the current standard treatment for Fabry and other LSDs, ERT. Our Chaperone-Advanced Replacement Therapy, or CHART, programs include pharmacological chaperones co-administered with currently marketed ERTs, as well as proprietary therapeutic enzymes co-formulated with our pharmacological chaperones as next-generation ERTs. We believe that our platform technologies, our advanced product pipeline, a strong balance sheet and our strategic collaborations uniquely positions us at the forefront of developing therapies for rare and orphan diseases.

We are developing our lead product candidate, migalastat HCl for Fabry disease, in collaboration with GSK as a monotherapy and in combination with ERT. Current development within our Fabry program includes two monotherapy Phase 3 global registration studies for patients with genetic mutations identified as amenable to this pharmacological chaperone in a cell-based assay (Study 011 and Study 012). We are also developing migalastat HCl in combination with ERT. We have completed a Phase 2 study investigating migalastat HCl co-administered with currently marketed ERTs Fabrazyme® and Replagal® (Study 013), and we are advancing Investigational New Drug-enabling studies of migalastat HCl co-formulated with a proprietary investigational ERT (recombinant human alpha-Gal A enzyme).

Our current CHART™ programs for Pompe disease are investigating the pharmacological chaperone AT2220 (duvoglustat HCl) in combination with ERT. AT2220 co-administered with currently marketed ERTs (Myozyme®/Lumizyme®) is in Phase 2. Our next-generation therapy for Pompe disease (AT2220 co-formulated with a proprietary recombinant human GAA enzyme) is in preclinical studies. In addition, we are engaged in preclinical development of a proprietary human recombinant IDUA (rhIDUA) enzyme to eventually be co-formulated with a novel pharmacological chaperone as next-generation therapy for MPS I.

**Program Status**

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

In Study 011, we are comparing migalastat HCl to placebo to potentially support global registration of migalastat HCl as a monotherapy for people with Fabry disease who have genetic mutations that are amenable to this chaperone monotherapy in a cell-based assay ( amenable mutations ). Study 011 randomized 67 patients (24 males and 43 females) diagnosed with Fabry disease who had amenable mutations. The 6-month, double-blind period (Stage 1) patients were randomized to migalastat HCl 150 mg or placebo on an every-other-day ( QOD ) oral dosing schedule. During the 6-12 month period of Study 011 (Stage 2) patients continued treatment with migalastat HCl or switched from placebo to migalastat HCl.

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The primary analysis compared the number of responders in the migalastat HCl versus placebo groups in Stage 1, based on a 50% or greater reduction in interstitial capillary globotriaosylceramide (GL-3) as measured in kidney biopsies. Pathologists blinded to biopsy sequence are using the published, quantitative Barisoni Lipid Inclusion Scoring System with virtual microscopy (BLISS-VM) for the histological evaluation of interstitial capillary GL-3 in kidney biopsies from baseline to month 6 Stage 1 and from baseline to month 12 Stage 2. Secondary endpoints for Study 011 include safety and tolerability, urine GL-3 and kidney function.

In December 2012, Amicus and GSK announced top-line six-month Stage 1 results from Study 011. In the primary responder analysis, 13 of 32, or 41%, of individuals in the migalastat HCl group versus 9 of 32, or 28% of individuals in the placebo group demonstrated a 50% or greater reduction in kidney interstitial capillary GL-3 from baseline to month 6 which was not statistically significant ( $p=0.3$ ). Taken alone a pre-specified secondary analysis of the absolute percent change in kidney interstitial capillary GL-3 from baseline to month 6, showed a median



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reduction of 41% in the migalastat HCl group versus a median reduction of 6% in the placebo group (p=0.093).

Study 012 is a randomized, open-label 18-month study investigating the safety and efficacy of migalastat HCl (150 mg, QOD) to current standard of care ERTs (i.e., Fabrazyme® and Replagal®) to support global registration. In December 2012, this study achieved full enrollment of 60 patients, who were randomized 1.5:1 to switch from ERT to migalastat HCl or remain on ERT. The study recruited males and females with Fabry disease and genetic mutations shown to be amenable to migalastat HCl monotherapy in a cell-based assay. All subjects had been receiving ERT infusions for a minimum of 12 months (at least 3 months at the labeled dose). The primary outcome measure is renal function assessed by measured Glomerular Filtration Rate (GFR) at 18 months.

A meeting with the U.S. Food and Drug Administration (the FDA) was held in the second quarter of 2013 to review the statistical analysis plan for Study 011 and to discuss the Stage 1 results. The FDA agreed to a follow-up meeting to review the full data from all Phase 2 and Phase 3 clinical studies of migalastat HCl. Based on feedback from this meeting and pending sufficiently positive results from both ongoing Phase 3 Fabry monotherapy studies (Study 011 and Study 012) as well as Phase 2 clinical studies, we intend to submit a U.S. new drug application (NDA) for migalastat HCl monotherapy.

We expect to provide an update on the migalastat HCl monotherapy program, including anticipated timelines for upcoming data results, by the end of 2013.

*Migalastat HCl Combination Programs for Fabry Disease*

We have completed an open-label Phase 2 drug-drug interaction study (Study 013) that evaluated the effects of a single oral dose of migalastat HCl (150 mg or 450 mg) co-administered prior to the currently marketed ERTs for Fabry disease (i.e., Fabrazyme® or Replagal®) in males with Fabry disease. Results from this study were presented in February 2013 and demonstrated an increase in  $\alpha$ -Gal A enzyme levels, the enzyme deficient in Fabry patients, in plasma and in skin following co-administration of migalastat HCl with ERT versus ERT alone. Based on the results from this study, the next chaperone-ERT combination study for Fabry disease is being designed to investigate intravenous treatment of migalastat HCl co-formulated with JCR Pharmaceuticals Co., Ltd.'s proprietary recombinant human  $\alpha$ -Gal A enzyme (JR-051).

*AT2220 CHART™ Programs for Pompe Disease*

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

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In addition, we have begun development of a next-generation therapy for Pompe disease, which consists of AT2220 co-formulated with our own proprietary recombinant human (rh) GAA enzyme. We believe this approach has the potential to improve the properties of the rhGAA enzyme itself while incorporating AT2220 as a small molecule stabilizer to increase exposure and tissue uptake of active enzyme, and reduce immunogenicity relative to currently marketed ERTs.

We expect to provide an update on our CHART™ programs for Pompe disease by the end of 2013.

### *Collaboration with GSK*

On July 17, 2012, we entered into the Expanded Collaboration Agreement with GSK pursuant to which we and GSK continue to develop and commercialize migalastat HCl, currently in Phase 3 development for the treatment of Fabry disease. For further information, please see Note 7. Collaborative Agreements.

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***Collaboration with Biogen***

On September 10, 2013, we entered into a collaboration agreement with Biogen to discover, develop and commercialize novel small molecules for the treatment of Parkinson's disease. The collaboration will build upon preclinical studies at the Company and independent published research that suggest increasing activity of the GCase enzyme in the brain may correct alpha-synuclein pathology and other deficits associated with Parkinson's disease. For further information, please see Note 7. Collaborative Agreements.

***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 our transformation from a development stage company to a commercial biotechnology company.

**Financial Operations Overview**

We have generated significant losses to date and expect to continue to generate losses as we continue the clinical and preclinical development of our drug candidates. 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 September 30, 2013, we have accumulated a deficit of \$366.3 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.

***Revenue***

Biogen

On September 10, 2013, we entered into a collaboration with Biogen to discover, develop and commercialize novel small molecules for the treatment of Parkinson's disease. For the three and nine months ended September 30, 2013, we recognized \$0.04 million as Research Revenue for reimbursed research and development costs.

GSK

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Under the Original Collaboration Agreement, GSK paid us an initial, non-refundable license fee of \$30.0 million and a premium of \$3.2 million related to GSK's purchase of an equity investment in the Company which was being recognized as Collaboration and Milestone Revenue on a straight-line basis over the development period. The reimbursements for research and development costs under the Original Collaboration Agreement that met the criteria for revenue recognition were recognized as Research Revenue. For the three months and nine months ended September 30, 2012, we recognized \$5.5 million and \$11.6 million, respectively, as Research Revenue.

On July 17, 2012, we entered into the Expanded Collaboration Agreement with GSK. Due to a change in the accounting for revenue recognition for the Expanded Collaboration Agreement, all revenue recognition will be suspended until the total arrangement consideration becomes fixed and determinable. Starting on July 17, 2012, any payments received from GSK are recorded as deferred reimbursements on the balance sheet. In addition, future milestone payments we may pay GSK will be applied against the balance of this deferred reimbursements account. Revenue recognition would resume once the total arrangement consideration becomes fixed and determinable which would occur when the balance of the deferred reimbursements account is sufficient to cover all the remaining contingent milestone payments due to GSK. As a result, we no longer recognize any revenue related to Collaboration and Milestone Revenue or Research Revenue as of the date of the Expanded Collaboration Agreement. There is no cash effect of this change in accounting, and there is no scenario where we would have to refund any of the upfront payment, milestone payments, or research reimbursement payments. As a result, for the

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three months and nine months ended September 30, 2013, we did not recognize any revenue related to Collaboration and Milestone Revenue or Research Revenue from GSK.

***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 certain future research and development costs with GSK and Biogen in accordance with their respective collaboration agreements. Research and development expenses consist of the following:

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

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

We expense research and development costs as incurred, including payments made to date under our license agreements. We believe that significant investment in product development is a competitive necessity and plan to continue these investments in order to realize the potential of our product candidates. From our inception in February 2002 through September 30, 2013, we have incurred research and development expenses in the aggregate of \$348.7 million.

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

Projects	Three Months Ended September 30,		Nine Months Ended September 30,		Period from February 4, 2002 (inception) to September 30, 2013
	2012	2013	2012	2013	2013
Third party direct project expenses					
Monotherapy Studies					
Migalastat HCl (Fabry Disease Phase 3)	\$ 2,658	\$ 2,644	\$ 12,927	\$ 6,996	\$ 87,049
Afegostat tartrate (Gaucher Disease Phase 2*)	36	4	78	78	26,379
AT2220 (Pompe Disease Phase 2)	5		5		13,252
Combination Studies					
Migalastat HCl Co-Administration (Fabry Disease Phase 2)	331	25	1,292	487	3,917
Migalastat HCl Co-Formulation (Fabry Disease Preclinical)	66	5	367	81	535
Afegostat tartrate Co-Administration (Gaucher Disease Preclinical)				21	55
AT2220 Co-Administration (Pompe Disease Phase 2)	612	813	1,538	2,633	6,761
AT2220 Co-Formulation (Pompe Disease Preclinical)		116		340	340
Neurodegenerative Diseases (Preclinical)	(28)	57	313	100	9,126
Total third party direct project expenses	3,680	3,664	16,520	10,736	147,414
Other project costs (1)					
Personnel costs	5,439	4,610	16,084	16,087	130,424
Other costs (2)	2,380	1,836	6,622	6,001	70,879
Total other project costs	7,819	6,446	22,706	22,088	201,303
Total research and development costs	\$ 11,499	\$ 10,110	\$ 39,226	\$ 32,824	\$ 348,717

(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 monotherapy program into Phase 3 development at this time.

The successful development of our product candidates is highly uncertain. At this time, we cannot reasonably estimate or know the nature, timing and costs of the efforts that will be necessary to complete the remainder of the development of our product candidates. As a result, we are not able to reasonably estimate the period, if any, in which material net cash inflows may commence from our product candidates, including migalastat 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 following:

- the number of clinical sites included in the trials;

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

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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 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 expenses consist primarily of salaries and other related costs, including stock-based compensation expenses for persons serving in our executive, finance, accounting, legal, information technology and human resource departments. Other general and administrative expenses consist include facility-related costs not otherwise included in research and development expenses, 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 September 30, 2013, we have spent \$146.9 million on general and administrative expenses.

***Interest Income and Interest Expense***

Interest income consists of interest earned on our cash and cash equivalents and marketable securities. Interest expenses consist of interest incurred on the 2011 Loan.

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

While there were no significant changes during the quarterly period ended September 30, 2013 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, 2012, we believe that the following accounting policies are the most critical to fully understand and evaluate our financial condition and results of operations.



***Revenue Recognition***

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

In multiple element arrangements, revenue is allocated to each separate unit of accounting and each deliverable in an arrangement is evaluated to determine whether it represents separate units of accounting. A deliverable constitutes a separate unit of accounting when it has standalone value and there is no general right of return for the delivered elements. In instances when the aforementioned criteria are not met, the deliverable is combined with the

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undelivered elements and the allocation of the arrangement consideration and revenue recognition is determined for the combined unit as a single unit of accounting. Allocation of the consideration is determined at arrangement inception on the basis of each unit's relative selling price. In instances where there is determined to be a single unit of accounting, the total consideration is applied as revenue for the single unit of accounting and is recognized over the period of inception through the date where the last deliverable within the single unit of accounting is expected to be delivered.

Our current revenue recognition policies, which were adopted and 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 VSOE if available, (ii) TPE if VSOE is not available, or (iii) BEBP 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 BEBP would be established considering internal factors such as an internal pricing analysis or an income approach using a discounted cash flow model.

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

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

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

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

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

In order to determine the revenue recognition for contingent milestones, we evaluate the contingent milestones using the criteria as provided by the FASB guidance on the milestone method of revenue recognition at the inception of a collaboration agreement. The criteria requires that:

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(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 costs 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 the following:

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

## Stock-Based Compensation

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

We use the Black-Scholes option pricing model when estimating the value for stock-based awards. Use of a valuation model requires management to make certain assumptions with respect to selected model inputs. Expected volatility was calculated based on a blended weighted average of historical information of our stock and the weighted average of historical information of similar public entities for which historical information was available. We will continue to use a blended weighted average approach using our own historical volatility and other similar public entity volatility information until our historical volatility is relevant to measure expected volatility for future option grants. The average expected life was determined using a simplified method of estimating the expected exercise term which is the mid-point between the vesting date and the end of the contractual term. As our stock price volatility has been over 75% and we have experienced significant business transactions (i.e., our collaborations), we believe that we do not have sufficient reliable exercise data in order to justify a change from the use of the simplified method of estimating the expected exercise term of employee stock option grants. The risk-free interest rate is based on U.S. Treasury, zero-coupon issues with a remaining term equal to the expected life assumed at the date of grant. Forfeitures are estimated based on expected turnover as well as a historical analysis of actual option forfeitures.

The following table summarizes information related to stock compensation expense recognized in the statements of operations (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2012	2013	2012	2013
Stock compensation expense recognized in:				
Research and development expense	\$ 955	\$ 824	\$ 2,748	\$ 2,634
General and administrative expense	643	693	1,995	2,040
Total stock compensation expense	\$ 1,598	\$ 1,517	\$ 4,743	\$ 4,674

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

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	Three Months Ended September 30,		Nine Months Ended September 30,	
	2012	2013	2012	2013
Expected stock price volatility	76.2%	81.7%	77.5%	82.0%
Risk free interest rate	0.9%	1.8%	0.8%	1.3%
Expected life of options (years)	6.25	6.25	6.25	6.25
Expected annual dividend per share	\$ 0.00	\$ 0.00	\$ 0.00	\$ 0.00

Table of Contents**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 September 30, 2012 and September 30, 2013 are as follows:

	September 30, 2012	September 30, 2013
Expected stock price volatility	76.0%	59.74%
Risk free interest rate	0.19%	0.03%
Expected life of warrants (years)	1.42	0.42
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.

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

(In thousands, except share amounts)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2012	2013	2012	2013
<b>Historical</b>				
Numerator:				
Net loss attributable to common stockholders	\$ (16,290)	\$ (14,589)	\$ (38,770)	\$ (47,396)
Denominator:				
Weighted average common shares outstanding basic and diluted	48,513,647	49,621,188	44,255,885	49,621,188

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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 10.4 million and 11.5 million for the nine months ended September 30, 2012 and 2013, respectively. Potentially dilutive common stock equivalents were excluded from the diluted earnings per share denominator for all periods because of their anti-dilutive effect.

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**Results of Operations**

***Three Months Ended September 30, 2013 Compared to Three Months Ended September 30, 2012***

**Revenue.** For the three months ended September 30, 2013, we recognized \$0.04 million as Research Revenue, as part of the Biogen Agreement. As a result of the Expanded Collaboration Agreement with GSK effective July 2012, we no longer recognize any revenue related to the Collaboration and Milestone Revenue or Research Revenue from GSK for 2013.

Under the Original Collaboration Agreement, GSK paid us an initial, non-refundable license fee of \$30.0 million and a premium of \$3.2 million related to GSK's purchase of an equity investment in the Company, which was being recognized as Collaboration and Milestone Revenue on a straight-line basis over the development period until entry into the Expanded Collaboration Agreement. Due to a change in the accounting for revenue recognition for the Expanded Collaboration Agreement, all revenue recognition related to the collaboration will be suspended until the total arrangement consideration becomes fixed and determinable. Any payments received from GSK will be recorded as deferred reimbursements on the balance sheet. In addition, future milestone payments we may pay GSK will be applied against the balance of this deferred reimbursements account. Revenue recognition would resume once the total arrangement consideration becomes fixed and determinable which would occur when the balance of the deferred reimbursements account is sufficient to cover all the remaining contingent milestone payments. As a result, we no longer recognize any revenue related to Collaboration and Milestone Revenue or Research Revenue as of the date of the Expanded Collaboration Agreement. There is no cash effect of this change in accounting, and there is no scenario where we would have to refund any of the upfront payment, milestone payments, or research reimbursement payments. We have not generated any commercial sales revenue since our inception. We have received cash payments from GSK related to the research reimbursement of \$3.7 million and \$1.0 million for the three months ended September 30, 2012, and 2013, respectively. In the third quarter of 2013, we paid GSK a pass-through milestone payment of \$0.8 million in connection with the development of the Co-formulated Product. This payment is reflected as a reduction of the deferred reimbursements in the Consolidated Balance Sheet as of September 30, 2013.

**Research and Development Expense.** Research and development expense was \$10.1 million for the three months ended September 30, 2013, representing a decrease of \$1.4 million or 12.0% from \$11.5 million for the three months ended September 30, 2012. The variance was primarily attributable to a decrease in personnel costs and lower license fees.

**General and Administrative Expense.** General and administrative expense was \$4.6 million for the three months ended September 30, 2013, representing a decrease of \$0.4 million or 8.0% from \$5.0 million for the three months ended September 30, 2012. The decrease was primarily due to decreases in personnel costs and legal fees.

**Interest Income and Interest Expense.** Interest income was \$0.04 million for the three months ended September 30, 2013, representing a decrease of \$0.06 million or 60% from \$0.1 million for the three months ended September 30, 2012. The decrease was due to lower cash and investment balances. Interest expense was approximately \$7,000 for the three months ended September 30, 2013 compared to \$19,000 for the three months ended September 30, 2012. The decrease was due to a lower outstanding debt balance 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 measure the fair value at each reporting



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date until exercised or expired. Changes in the fair value of the warrants are reported in the statements of operations as non-operating income or expense. For the three months ended September 30, 2013 and September 30, 2012 respectively, we reported income of \$0.5 million and \$0.6 million related to the decrease in fair value of these warrants, representing a decrease of \$0.1 million or 16%. The market price of our common stock has been and may continue to be volatile. Consequently, future fluctuations in the price of our common stock may cause significant increases or decreases in the fair value of the warrant liability.

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**Results of Operations**

*Nine Months Ended September 30, 2013 Compared to Nine Months Ended September 30, 2012*

*Revenue.* For the nine months ended September 30, 2012, we recognized \$6.8 million as Collaboration and Milestone Revenue and \$11.6 million as Research Revenue. We have received cash payments from GSK related to the research reimbursement of \$13.0 million and \$5.5 million for the nine months ended September 30, 2012, and 2013, respectively. For the nine months ended September 30, 2013 we recognized \$0.04 million as research revenue, as part of the Biogen Agreement.

*Research and Development Expense.* Research and development expense was \$32.8 million for the nine months ended September 30, 2013, representing a decrease of \$6.4 million or 16% from \$39.2 million for the nine months ended September 30, 2012. The variance was primarily attributable to a decrease in contract research and manufacturing costs due to decreased activity within the Fabry program.

*General and Administrative Expense.* General and administrative expense was \$14.3 million for the nine months ended September 30, 2013, representing a decrease of \$0.6 million or 4% from \$14.9 million for the nine months ended September 30, 2012. The decrease was primarily due to decreases in personnel costs, legal fees and facilities.

*Interest Income and Interest Expense.* Interest income was \$0.1 million for the nine months ended September 30, 2013, representing a decrease of \$0.1 million or 50% from \$0.2 million for the nine months ended September 30, 2012. The decrease was due to lower cash and investment balance during the period. Interest expense was approximately \$26,000 for the nine months ended September 30, 2013 compared to \$0.1 million for the three months ended September 30, 2012. The decrease was due to a lower outstanding debt balance 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 measure the fair value at each reporting date until exercised or expired. Changes in the fair value of the warrants are reported in the statements of operations as non-operating income or expense. For the nine months ended September 30, 2013, we reported income of \$0.9 million related to the decrease in fair value of these warrants as compared to an expense of \$1.9 million for the nine months ended September 30, 2012, representing an increase of \$2.8 million or 147%. The market price of our common stock has been and may continue to be volatile. Consequently, future fluctuations in the price of our common stock may cause significant increases or decreases in the fair value of the warrant liability.

*Other Income/Expense.* There was no other income or expense for the nine months ended September 30, 2013. Other income for the nine months ended September 30, 2012 was \$0.02 million and represents cash received from the sale of property, plant and equipment.

**Liquidity and Capital Resources**

*Source of Liquidity*

As a result of our significant research and development expenditures and the lack of any approved products to generate product sales revenue, we have not been profitable and have generated operating losses since we were incorporated in 2002. We have funded our operations principally with \$148.7 million of proceeds from redeemable convertible preferred stock offerings, \$75.0 million of gross proceeds from our IPO in September 2007, \$18.5 million of gross proceeds from our registered direct offering in March 2010, \$65.6 million of gross proceeds from our stock offering in March 2012, \$49.9 million from GSK's investments in the Company in October 2010 and July 2012, and \$80.0 million from non-refundable license fees from various collaborations. In the future, we expect to fund our operations, in part, through the receipt of cost-sharing payments from GSK. The following table summarizes our significant funding sources as of September 30, 2013:

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<b>Funding (2)</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	2007	5,000,000	75,000
Upfront License Fee from Shire	2007		50,000
Registered Direct Offering	2010	4,946,524	18,500
Upfront License Fee from GSK	2010		30,000
Common Stock - GSK Purchase	2010	6,866,245	31,285
Common Stock Offering	2012	11,500,000	65,550
Common Stock - GSK Purchase	2012	2,949,581	18,582
		47,375,071	\$ 437,605

(1) Represents gross proceeds

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

In addition, we have received reimbursement of research and development expenditures from GSK beginning with entry into the Original Collaboration Agreement (October 28, 2010) through September 30, 2013 of \$28.2 million. We also received \$31.1 million in reimbursement of research and development expenditures from the Shire collaboration from the date of the agreement (November 7, 2007) through year-end 2009.

As of September 30, 2013, we had cash, cash equivalents and marketable securities of \$60.5 million. We invest cash in excess of our immediate requirements with regard to liquidity and capital preservation in a variety of interest-bearing instruments, including obligations of U.S. government agencies and money market accounts. Wherever possible, we seek to minimize the potential effects of concentration and degrees of risk. Although we maintain cash balances with financial institutions in excess of insured limits, we do not anticipate any losses with respect to such cash balances.

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

Net cash used in operations for the nine months ended September 30, 2012 was \$26.4 million, due primarily to the net loss for the nine months ended September 30, 2012 of \$38.8 million and the change in operating assets and liabilities of \$4.4 million. The change in operating assets and liabilities consisted of a decrease in receivables from GSK related to the collaboration agreement of \$1.9 million; a decrease of \$2.8 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 reimbursements of \$0.3 million related to the recognition of the upfront payment from GSK for the collaboration agreement through June 30, 2012 and a decrease in accounts payable and accrued expenses of \$0.3 million related to program expenses.

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Net cash used in operations for the nine months ended September 30, 2013 was \$37.7 million, due primarily to the net loss for the nine months ended September 30, 2013 of \$47.4 million and the change in operating assets and liabilities of \$4.6 million. The change in operating assets and liabilities consisted of a decrease in receivables from GSK related to the collaboration agreement of \$1.1 million; a decrease of \$0.6 million in prepaid assets primarily related to prepaid rent, service contracts and interest paid on investment ; an increase in deferred revenue of \$3.6 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 \$0.7 million related to program expenses.

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***Net Cash (Used in)/ Provided By Investing Activities***

Net cash used in investing activities for the nine months ended September 30, 2012 was \$56.0 million. Net cash used by investing activities reflects \$47.4 million for the purchase of marketable securities offset by \$99.3 million for the redemption of marketable securities and \$4.2 million for the acquisition of property and equipment.

Net cash provided by investing activities for the nine months ended September 30, 2013 was \$34.0 million. Net cash provided by investing activities reflects \$68.3 million for the redemption of marketable securities, partially offset by \$33.7 million for the purchase of marketable securities and \$0.6 million for the acquisition of property and equipment.

***Net Cash Provided by/ (Used in) Financing Activities***

Net cash provided by financing activities for the nine months ended September 30, 2012 was \$81.0 million, consisting of \$80.2 million from the issuance of common stock, \$1.0 million as proceeds from the new secured loan agreement with SVB and \$1.0 million from the exercise of stock options. This was partially offset by the payments on our secured loan agreement of \$1.1 million.

Net cash used in financing activities for the nine months ended September 30, 2013 was \$0.3 million, consisting of payments on our secured loan agreement.

***Funding Requirements***

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

- the progress and results of our clinical trials of our drug candidates, including migalastat HCl;
- the continuation of our collaboration with GSK and GSK's achievement of milestone payments thereunder;
- the scope, progress, results and costs of preclinical development, laboratory testing and clinical trials for our product candidates including those testing the use of pharmacological chaperones co-formulated and co-administered with ERT and for the treatment of diseases of neurodegeneration;
- the costs, timing and outcome of regulatory review of our product candidates;
- the number and development requirements of other product candidates that we pursue;

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- 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 2014, if at all. In the absence of additional funding, we expect our continuing operating losses to result in increases in our cash used in operations over the next several quarters and years. We believe that our existing cash and cash equivalents and short term investments will be sufficient to cover our cash flow requirements into the fourth quarter of 2014.

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***Financial Uncertainties Related to Potential Future Payments***

***Milestone Payments***

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

Under the Expanded Collaboration Agreement, GSK is eligible to receive U.S. regulatory approval milestones totaling \$20.0 million for migalastat HCl monotherapy and migalastat HCl co-administered with ERT, and additional regulatory approval and product launch milestone payments totaling up to \$35.0 million within seven years following the launch of the Co-formulated Product. We will also be responsible for certain pass-through milestone payments and single-digit royalties on the net U.S. sales of the Co-formulated Product that GSK must pay to a third party. In addition, we are no longer eligible to receive any milestones or royalties we would have been eligible to receive under the Original Collaboration Agreement other than a \$3.5 million clinical development milestone achieved in the second quarter of 2012 and paid by GSK to us in the third quarter of 2012.

***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 it to commercialization. To date, we have not made any royalty payments on sales of our products and believe we are at least a couple years away from selling any products that would require us to make any such royalty payments.

In accordance with our license agreement with MSSM, in the third quarter of 2012, we paid \$0.4 million of the \$3.5 million milestone payment received from GSK to MSSM. In the fourth quarter of 2010, we paid \$3.0 million of the \$30.0 million upfront payment received from GSK to MSSM. We will also be obligated to pay MSSM royalties on worldwide net sales of migalastat HCl.

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

**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 September 30, 2013, we held \$60.5 million in cash, cash equivalents and available for sale securities and due to the short-term maturities of our investments, we do not believe that a 10% change in average interest rates would have a significant impact on our interest income. Our outstanding debt has a fixed interest rate and therefore, we have no exposure to interest rate fluctuations.

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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 last fiscal quarter that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

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**PART II. OTHER INFORMATION**

**ITEM 1. LEGAL PROCEEDINGS**

We are not a party to any material legal proceedings.

**ITEM 1A. RISK FACTORS**

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, 2012.

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

**Recent Sales of Unregistered Securities**

None.

**Use of Proceeds**

*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 per share through a Registration Statement on Form S-3 (File No. 333-158405) 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 underwriting 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.

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As of September 30, 2013, approximately \$11.8 million in net proceeds from this stock offering were maintained in money market funds and in investment-grade, interest bearing instruments, pending their use. We have used the remaining proceeds from this offering to advance the clinical and preclinical pharmacological chaperone programs 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**

The Company did not purchase any shares of its common stock for the three months ended September 30, 2013.

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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**

<b>Exhibit Number</b>	<b>Description</b>
3.1 (1)	Restated Certificate of Incorporation
3.2 (2)	Amended and Restated By-laws
31.1*	Certification of Principal Executive Officer pursuant to Rules 13a-14 and 15d-14 promulgated pursuant to the Securities Exchange Act of 1934, as amended
31.2*	Certification of Principal Financial Officer pursuant to Rules 13a-14 and 15d-14 promulgated pursuant to the Securities Exchange Act of 1934, as amended
32.1*	Certification of Principal Executive Officer and Principal Financial Officer pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
101	The following financial information from this Quarterly Report on Form 10-Q for the quarter ended September 30, 2013, formatted in XBRL (Extensible Business Reporting Language) and filed electronically herewith: (i) the Consolidated Balance Sheets as of September 30, 2013 and December 31, 2012; (ii) the Consolidated Statements of Operations for the three and nine months ended September 30, 2013 and 2012; (iii) the Consolidated Statements of Comprehensive Loss for the three and nine months ended September 30, 2013 and 2012; (iv) the Consolidated Statements of Cash Flows for the nine months ended September 30, 2013 and 2012; (v) and the Notes to the Consolidated Financial Statements.

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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

\* 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: November 12, 2013

By: /s/ John F. Crowley

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

Date: November 12, 2013

By: /s/ William D. Baird III

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

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