UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

FORM 10-Q

(Mark One)

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

For the quarterly period ended September 30, 2014

OR

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

For the transition period from to

Commission file number 001-33497

Amicus Therapeutics, Inc.
(Exact Name of Registrant as Specified in Its Charter)

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

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

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

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

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

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

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

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

The number of shares outstanding of the registrant's common stock, $.01 par value per share, as of October 31, 2014 was 79,321,630 shares.

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AMICUS THERAPEUTICS, INC.

Form 10-Q for the Quarterly Period Ended September 30, 2014

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

The forward-looking statements in this 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 cost of manufacturing drug supply for our clinical and preclinical studies, including the significant cost of enzyme replacement therapy (ERT) cell line development and manufacturing as well as the cost of manufacturing the vIGF-2 peptide tag;
- the scope, progress, results and costs of preclinical development, laboratory testing and clinical trials for our product candidates including those testing the use of pharmacological chaperones co-formulated and co-administered with ERT and for the treatment of lysosomal storage diseases;
- the costs, timing and outcome of regulatory review of our product candidates;
- the number and development requirements of other product candidates that we pursue;
- the costs of commercialization activities, including product marketing, sales and distribution;
- the emergence of competing technologies and other adverse market developments;
- the costs of preparing, filing and prosecuting patent applications and maintaining, enforcing and defending intellectual property related claims;
- the extent to which we acquire or invest in businesses, products and technologies; 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, 2013 that we believe could cause actual results or events to differ materially from the forward-looking statements that we make. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, joint ventures, collaborations or investments we may make.

You should read this quarterly report on Form 10-Q in conjunction with the documents that we reference herein. We do not assume any obligation to update any forward-looking statements.

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PART I. FINANCIAL INFORMATION

Item 1. Financial Statements (unaudited)

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

	December 31, 2013	September 30, 2014
Assets:		
Current assets:		
Cash and cash equivalents	$ 43,640	$ 19,671
Investments in marketable securities	38,360	65,511
Receivable due from collaboration agreements	1,083	293
Prepaid expenses and other current assets	5,195	1,762
Total current assets	88,278	87,237
Property and equipment, less accumulated depreciation and amortization of $9,973 and $11,157 at December 31, 2013 and September 30, 2014, respectively	4,120	3,129
In-process research & development	23,000	23,000
Goodwill	11,613	11,613
Other non-current assets	552	508
Total Assets	$ 127,563	$ 125,487
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable and accrued expenses	$ 10,162	$ 11,331
Current portion of secured loan	299	2,493
Total current liabilities	10,461	13,824
Deferred reimbursements	36,677	36,677
Secured loan, less current portion	14,174	11,809
Contingent consideration payable	10,600	10,200
Deferred tax liability	9,186	9,186
Other non-current liability	714	514
Commitments and contingencies		
Stockholders' equity:		
Common stock, $.01 par value, 125,000,000 shares authorized, 61,975,416 shares issued and outstanding at December 31, 2013, 125,000,000 shares authorized, 79,257,588 shares issued and outstanding at September 30, 2014	679	853
Additional paid-in capital	423,593	468,650
Accumulated other comprehensive income	1	2
Accumulated deficit	(378,522)	(426,228)
Total stockholders' equity	45,751	43,277
Total Liabilities and Stockholders' Equity	$ 127,563	$ 125,487

See accompanying notes to consolidated financial statements

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2013	2014	2013	2014
Revenue:				
Research revenue	$ 39	$ 293	$ 39	$ 1,224
Total revenue	39	293	39	1,224
Operating Expenses:				
Research and development	$ 10,110	$ 12,049	$ 32,824	$ 32,019

General and administrative	4,635	5,270	14,288	15,199
Changes in fair value of contingent consideration payable	—	(600)	—	(400)
Restructuring charges	—	15	—	(74)
Depreciation and amortization	429	375	1,318	1,183
Total operating expenses	15,174	17,109	48,430	47,927
Loss from operations	(15,135)	(16,816)	(48,391)	(46,703)
Other income (expenses):				
Interest income	36	55	147	133
Interest expense	(7)	(377)	(26)	(1,106)
Change in fair value of warrant liability	517	—	874	—
Other expense	—	(11)	—	(30)
Net loss	$ (14,589)	$ (17,149)	$ (47,396)	$ (47,706)
Net loss per common shares — basic and diluted	$ (0.29)	$ (0.22)	$ (0.96)	$ (0.68)
Weighted-average common shares outstanding — basic and diluted	49,621,188	78,889,346	49,621,188	70,216,251

See accompanying notes to consolidated financial statements

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Amicus Therapeutics, Inc.
Consolidated Statements of Comprehensive Loss
(Unaudited)
(in thousands)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2013	2014	2013	2014
Net loss	$ (14,589)	$ (17,149)	$ (47,396)	$ (47,706)
Other comprehensive (loss)/ income:				
Unrealized gain/ (loss) on available-for-sale securities	2	4	(9)	1
Other comprehensive gain/ (loss) before income taxes	2	4	(9)	1
Provision for income taxes related to other (loss)/ comprehensive income items (a)	—	—	—	—
Other comprehensive gain/ (loss)	$ 2	$ 4	$ (9)	$ 1
Comprehensive loss	$ (14,587)	$ (17,145)	$ (47,405)	$ (47,705)

(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

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Amicus Therapeutics, Inc.
Consolidated Statements of Cash Flows
(Unaudited)
(in thousands)

	Nine Months Ended September 30,	
	2013	2014
Operating activities		
Net loss	$ (47,396)	$ (47,706)
Adjustments to reconcile net loss to net cash used in operating activities:		
Non-cash interest expense	—	176
Depreciation and amortization	1,318	1,183
Stock-based compensation	4,674	4,398
Restructuring charges	—	(74)
Change in fair value of warrant liability	(874)	—
Non-cash changes in the fair value of contingent consideration payable	—	(400)
Changes in operating assets and liabilities:		

Receivable due from collaboration agreements	1,064	790
Prepaid expenses and other current assets	618	3,433
Other non-current assets	—	26
Accounts payable and accrued expenses	(679)	1,213
Non-current liabilities	—	(200)
Deferred reimbursements	3,601	—
Net cash used in operating activities	(37,674)	(37,161)
Investing activities		
Sale and redemption of marketable securities	68,348	47,959
Purchases of marketable securities	(33,654)	(75,109)
Purchases of property and equipment	(645)	(192)
Net cash provided by/(used in) investing activities	34,049	(27,342)
Financing activities		
Proceeds from issuance of common stock, net of issuance costs	—	38,736
Payments of secured loan agreement	(299)	(299)
Proceeds from exercise of stock options	—	2,097
Net cash (used in)/provided by financing activities	(299)	40,534
Net decrease in cash and cash equivalents	(3,924)	(23,969)
Cash and cash equivalents at beginning of period	33,971	43,640
Cash and cash equivalents at end of period	$ 30,047	$ 19,671
Supplemental disclosures of cash flow information		
Cash paid during the period for interest	$ 24	$ 864
Non-cash activities	$ —	$ —

See accompanying notes to consolidated financial statements

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Note 1. Description of Business and Significant Accounting Policies

Corporate Information, Status of Operations and Management Plans

Amicus Therapeutics, Inc. (the Company) was incorporated on February 4, 2002 in Delaware and is a biopharmaceutical company focused on the discovery, development and commercialization of 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 (migalastat) for Fabry disease. Migalastat is a novel, small molecule pharmacological chaperone in development as a monotherapy and in combination with enzyme replacement therapy (ERT) for Fabry disease. The Company is leveraging its Chaperone-Advanced Replacement Therapy, or CHART™ platform along with other proprietary technologies to develop next-generation ERTs for Fabry, Pompe, Mucopolysaccharidosis Type I (MPS I) and Gaucher diseases. The Company's activities since inception have consisted principally of raising capital, establishing facilities, and performing research and development.

In August 2014, the Company announced positive 18 month data from the Study 012. A summary of the 18-month results are as follows:

- Migalastat had a comparable effect to ERT on patients' kidney function as measured by the change in eGFR and mGFR.
- Levels of plasma lyso-Gb3, an important biomarker of disease, remained low and stable in patients with amenable mutations who switched from ERT to migalastat.
- Migalastat was generally safe and well-tolerated.
- Of 48 patients with GLP HEK-amenable mutations who completed Study 012, 46 (96%) elected to continue with the 12-month treatment extension and 45 remain on migalastat today as their only treatment for Fabry disease.

The Company looks forward to meeting with the EMEA in the fourth quarter of 2014 and the US FDA early in 2015 to make migalastat available for all amenable Fabry patients as quickly as possible.

In July 2014, the Company completed a $40 million at the market (ATM) equity offering under which the Company sold shares of its common stock, par value $0.01 per share, with Cowen and Company LLC as sales agent. Under the ATM equity program, the Company sold 14.3 million shares of common stock raising approximately $38.7 million in net proceeds.

For further information on the ATM Agreement, see — Note 7. Stockholder's Equity.

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

For further information, see — Note 4. Acquisition of Callidus Biopharma, Inc.

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

In November 2013, the Company entered into a securities purchase agreement (the 2013 SPA) with certain entities controlled by Redmile Group, LLC and GSK for the private placement of a combination of shares of the Company's common stock and warrants to purchase shares of the Company's common stock. The warrants have a term of one year and are exercisable between July 1, 2014 and June 30, 2015 at an exercise price of $2.50 per share. The aggregate offer proceeds were $15 million.

In September 2013, the Company entered into a collaboration agreement with Biogen Idec (Biogen) to discover, develop and commercialize novel small molecules that target the glucocerobrosidase (GCase) enzyme for the treatment of Parkinson's disease.

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

For further information, see — Note 10. Collaborative Agreements.

The Company had an accumulated deficit of approximately $426.2 million at September 30, 2014 and anticipates incurring losses through the fiscal year ending December 31, 2014 and beyond. The Company has not yet generated commercial sales revenue and has been able to fund its operating losses to date through the sale of its redeemable convertible preferred stock, issuance of convertible notes, net proceeds from its initial public offering (IPO) and subsequent stock offerings, payments from partners during the terms of the collaboration agreements and other financing arrangements.

Including the net proceeds from the completed ATM equity program, the Company believes that its existing cash and cash equivalents and short-term investments will be sufficient to fund the current operating plan into 2016.

Basis of Presentation

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

The accompanying unaudited consolidated financial statements and related notes should be read in conjunction with the Company's financial statements and related notes as contained in the Company's Annual Report on Form 10-K for the year ended December 31, 2013. 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, 2013.

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Significant Accounting Policies

There have been no material changes to the Company's significant accounting policies during the three months ended September 30, 2014, as compared to the significant accounting policies disclosed in Note 2 of the Consolidated Financial Statements in the Company's Annual Report on Form 10-K for the year ended December 31, 2013, except for the adoption of Accounting Standards Update (ASU) 2014-10, as described below. However, the following accounting policies are the most critical in fully understanding and evaluating the Company's financial condition and results of operations.

Revenue Recognition

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

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

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

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

- a payment for an identifiable benefit; and

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

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

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

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

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

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

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

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

Fair Value Measurements

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

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

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New Accounting Standards

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

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

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

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Note 2. Cash, Money Market Funds and Marketable Securities

As of September 30, 2014, the Company held $19.7 million in cash and cash equivalents and $65.5 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 comprehensive income/ (loss) in the statement 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, 2013 and September 30, 2014 (in thousands):

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

	As of September 30, 2014			
	Cost	Unrealized Gain	Unrealized Loss	Fair Value
Cash balances	$ 19,671	$ —	$ —	$ 19,671
Corporate debt securities	53,674	1	(13)	53,662
Commercial paper	11,486	13	—	11,499
Certificate of deposit	350	—	—	350
	$ 85,181	$ 14	$ (13)	$ 85,182
Included in cash and cash equivalents	$ 19,671	$ —	$ —	$ 19,671
Included in marketable securities	65,510	14	(13)	65,511
Total cash and available for sale securities	$ 85,181	$ 14	$ (13)	$ 85,182

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, 2013, unrealized holding loss of $13 thousand and for the nine months ended September 30, 2014, unrealized holding gain of $1 thousand, were recognized.

For the year ended December 31, 2013 and the nine months ended September 30, 2014, 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, 2013 and September 30, 2014 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 $23.6 million and $42.6 million as of December 31, 2013 and September 30, 2014, respectively.

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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 months ended September 30, 2013 and 2014, were as follows (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2013	2014	2013	2014
Balance, beginning	$ 3	(2)	$ 14	$ 1
Current period changes in fair value, (a)	2	4	(9)	1
Reclassification of earnings, (a)	—	—	—	—
Balance, ending	$ 5	$ 2	$ 5	$ 2

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

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

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

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
(In thousands, except per share amounts)	2013	2014	2013	2014
Historical				
Numerator:				
Net loss attributable to common stockholders per common	$ (14,589)	$ (17,149)	$ (47,396)	$ (47,706)
Denominator				
Weighted average common shares outstanding — basic and diluted	49,621,188	78,889,346	49,621,188	70,216,251

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

	As of September 30,	
	2013	2014
Options to purchase common stock	10,112	10,329
Outstanding warrants, convertible to common stock	1,404	1,600
Unvested restricted stock units	—	955
Total number of potentially issuable shares	11,516	12,884

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Note 4. Acquisition of Callidus Biopharma, Inc.

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

In consideration for the merger, the Company agreed to issue an aggregate of 7.2 million shares of its common stock, par value $0.01 per share, to the former stockholders of Callidus. As of September 30, 2014, approximately 25 thousand shares remain issuable to former Callidus shareholders. In addition, the Company will be obligated to make additional payments to the former stockholders of Callidus upon the achievement by the Company of certain clinical milestones of up to $35 million and regulatory approval milestones of up to $105 million as set forth in the Merger Agreement, provided that the aggregate consideration shall not exceed $130 million. The Company may, at its election, satisfy certain milestone payments identified in the Merger Agreement aggregating $40 million in shares of its Common Stock (calculated based on a price per share equal to the average of the last closing bid price per share for the Common Stock on The NASDAQ Global Market for the ten (10) trading days immediately preceding the date of payment). The milestone payments not permitted to be satisfied in Common Stock (as well as any payments that the Company is permitted to, but chooses not to, satisfy in Common Stock), as a result of the terms of the Merger Agreement, the rules of The NASDAQ Global Market, or otherwise, will be paid in cash.

The fair value of the contingent acquisition consideration payments on the acquisition date was $10.6 million and was estimated by applying a probability-based income approach utilizing an appropriate discount rate. This estimation was based on significant inputs that are not observable in the market, referred to as Level 3 inputs. Key assumptions included a discount rate of 13.0% and various probability factors. As of September 30, 2014, the range of outcomes and assumptions used to develop these estimates has changed to better reflect the probability of certain milestone outcomes. (see — Note 8. Assets and Liabilities Measured at Fair Value for additional discussion regarding fair value measurements of the contingent acquisition consideration payable). The Company determined the fair value of the contingent consideration to be $10.2 million at September 30, 2014, resulting in a decrease in the contingent consideration payable and related income of $0.4 million for the nine months ended September 30, 2014.

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

As part of the Callidus acquisition, the Company recognized $9.2 million of deferred tax liabilities, which relates to the tax impact of future amortization or possible impairments associated with the identified intangible assets acquired, and are not deductible for tax purposes. The Company also recorded goodwill in the Company's consolidated balance sheet as of the acquisition date. The goodwill results from the recognition of the deferred tax liability on the intangible assets as well as synergies expected from the acquisition and other benefits that do not qualify for separate recognition as acquired intangible assets. None of the goodwill is expected to be deductible for income tax purposes. The final valuation of this acquisition was completed on March 31, 2014.

For further information, see — Note 5 Goodwill & — Note 6 Intangible Assets.

Supplemental Pro Forma Information

The following pro forma information for the three and nine months ending September 30, 2013 assumes the Merger Agreement occurred as of January 1, 2013. The pro forma information is not necessarily indicative either of the combined results of operations that actually would have been realized had the Merger Agreement been consummated during the period for which pro forma information is presented, or is it intended to be a projection of future results or trends.

(in thousands)	Three Months ended September 30, 2013	Nine Months ended September 30, 2013
Revenue	$ —	$ —
Net loss	$ (15,838)	$ (49,569)

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Note 5. Goodwill

In connection with the acquisition of Callidus in November 2013, the Company recognized $11.6 million of goodwill, resulting from the recognition of the deferred tax liability on the intangible assets as well as synergies expected from the acquisition and other benefits that do not qualify for separate recognition as acquired intangible assets.

Goodwill is tested for impairment on an annual basis. In between annual tests the Company assesses any events occurring or changes in the circumstances that would reduce the fair value of the goodwill below its carrying amount.

The following table represents the changes in goodwill for the nine months ended September 30, 2014:

	(in thousands)
Balance at December 31, 2013	$ 11,613
Change in goodwill related to the acquisition of Callidus	—
Balance at September 30, 2014	$ 11,613

Note 6. Intangible Assets

In connection with the acquisition of Callidus in November 2013, the Company recognized $23.0 million of IPR&D.

Intangible assets consisting of IPR&D are considered indefinite-lived until the completion or abandonment of the associated research and development efforts. During the period the assets are considered indefinite lived, they will not be amortized but will be tested for impairment on an annual basis. In between annual tests the Company assesses any events occurring or changes in the circumstances that would reduce the fair value of the intangible asset below its carrying amount.

The following table represents the changes in intangible assets for the nine months ended September 30, 2014:

	(in thousands)
Balance at December 31, 2013	$ 23,000
Change in IPR&D related to the acquisition of Callidus	—
Balance at September 30, 2014	$ 23,000

Note 7. Stockholders' Equity

Common Stock and Warrants

As of September 30, 2014, the Company was authorized to issue 125 million shares of common stock. Dividends on common stock will be paid when, and if declared by the board of directors. Each holder of common stock is entitled to vote on all matters and is entitled to one vote for each share held.

In March 2014, the Company entered into the Sales Agreement with Cowen to create an ATM equity program under which the Company sold shares of its common stock, par value $0.01 per share, with an aggregate offering price of up to $40 million (ATM Shares) through Cowen. Upon delivery of a placement notice and subject to the terms and conditions of the ATM agreement, Cowen used its commercially reasonable efforts to sell the ATM Shares, based upon the Company's instructions. The Company had provided Cowen with customary indemnification rights, and Cowen was entitled to a commission at a fixed commission rate of up to 3.0% of the gross proceeds per Share sold. Sales of the ATM Shares under the Sales Agreement were to be made in transactions that were deemed to be "at the market offerings" as defined in Rule 415 under the Securities Act of 1933, as amended, including sales made by means of ordinary brokers' transactions, including on The NASDAQ Global Market, at market prices or as otherwise agreed with Cowen. The Company began the sale of ATM Shares in May 2014. The Company sold 14.3 million shares of common stock resulting in net proceeds of $38.7 million, which included Cowen's commission of $1.1 million and legal fees of $0.1 million. The Company completed all sales under the ATM equity program in July 2014.

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In November 2013, the Company entered into the 2013 SPA with certain entities controlled by Redmile Group, LLC and GSK and for the private placement of shares of the Company's common stock, par value $0.01 and a combination of shares of common stock (the Shares) and warrants (the Warrants) to purchase shares of the Common Stock (collectively, the Units). Each of the investors was one of the Company's shareholders prior to consummation of these transactions. The Shares and the Units sold to the investors were offered and sold in reliance on exemptions from registration pursuant to Rule 506 of Regulation D promulgated under the Securities Act based on the nature of such investors and certain representations made to the Company. Pursuant to the 2013 SPA, Amicus agreed to issue 1.5 million Shares at $2.00 per Share to GSK and (b) 6 million Units at $2.00 per Unit to Redmile Group, with each Unit consisting of one Share and .267 Warrants resulting in an aggregate of 6 million Shares and 1.6 million Warrants underlying the Units to be issued. Each Warrant is exercisable between July 1, 2014 and June 30, 2015 with an exercise price of $2.50, subject to certain adjustments. The Company received total proceeds of $15 million for general corporate and working capital purposes as a result of the private placement and the transaction closed in November 2013.

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

In November 2013, in connection with its acquisition of Callidus, the Company agreed to issue an aggregate of 7.2 million shares of its common stock, par value $0.01 per share, to the former stockholders of Callidus. As of September 30, 2014, approximately 25 thousand shares remain issuable to former Callidus shareholders.

Nonqualified Cash Deferral Plan

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

As of September 30, 2014, the amounts deferred under the Deferral Plan have not been invested. The investments are expected to be made in the fourth quarter of fiscal year 2014. All of the investments held in the Deferral Plan will be classified as trading securities and recorded at fair value with changes in the investments' fair value recognized in the period they occur. The corresponding liability for the Deferral Plan is included in other non-current liability in the Company's consolidated balance sheets.

Equity Incentive Plan

In June 2014, the Company's shareholders approved the Amended and Restated 2007 Equity Incentive Plan (the Plan). The amendment to the Plan makes an additional 6 million shares of the Company's common stock available for issuance and increases the maximum number of shares within the Plan that may be issued as restricted stock, restricted stock units (RSUs), stock grants and any other similar awards from 1.1 million to 1.5 million shares. As of September 30, 2014, awards issued under the Plan include both stock options and RSUs.

Stock Option Grants

The fair value of the stock 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,	
	2013	2014	2013	2014
Expected stock price volatility	81.7%	81.0%	82.0%	81.3%
Risk free interest rate	1.8%	1.9%	1.3%	1.9%
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

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A summary of the Company's stock options for the nine months ended September 30, 2014 is as follows:

	Number of Shares (in thousands)	Weighted Average Exercise Price	Weighted Average Remaining Contractual Life	Aggregate Intrinsic Value (in millions)
Balance at December 31, 2013	9,041.1	$ 5.65		
Options granted	2,894.1	$ 2.87		
Options exercised	(594.4)	$ 3.53		
Options forfeited	(1,012.3)	$ 5.82		
Balance at September 30, 2014	10,328.5	$ 4.97	6.9 years	$ 17.6

Vested and unvested expected to vest, September 30, 2014	9,649.5	$ 5.10	6.8 years	$	15.6
Exercisable at September 30, 2014	5,551.1	$ 6.40	5.2 years	$	4.9

As of September 30, 2014, the total unrecognized compensation cost related to non-vested stock options granted was $8.3 million and is expected to be recognized over a weighted average period of 2.4 years.

Restricted Stock Units

In April 2014, the Compensation Committee made awards of restricted stock units (the RSUs) to certain employees of the Company. The RSUs awarded under the Plan are generally subject to graded vesting of 50% of the RSUs on the 13th month anniversary of the grant date and the remaining 50% of the RSUs on the 20th month anniversary of the grant date, in each case, contingent on such employee's continued service on such date.

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

A summary of non-vested RSU activity under the Plan for the nine months ended September 30, 2014 is as follows:

	Number of Shares (in thousands)	Weighted Average Grant Date Fair Value	Weighted Average Remaining Years	Aggregate Intrinsic Value (in millions)
Non-vested units as of December 31, 2013	—	$ —		
Granted	975	$ 2.28		
Vested	—	$ —		
Forfeited	(20)	$ 2.15		
Non-vested units as of September 30, 2014	955	$ 2.28	1.0	$ 3.5

For the nine months ended September 30, 2014, there were no RSUs that vested and all non-vested units are expected to vest over their normal term. As of September 30, 2014, there was $1.5 million of total unrecognized compensation cost related to unvested RSUs with service-based vesting conditions. These costs are expected to be recognized over a weighted average period of 1.0 years.

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

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Compensation Expense Related to Equity Awards

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2013	2014	2013	2014
Stock compensation expense recognized in:				
Research and development expense	$ 824	$ 698	$ 2,634	$ 1,950
General and administrative expense	693	953	2,040	2,448
Total stock compensation expense	$ 1,517	$ 1,651	$ 4,674	$ 4,398

Note 8. 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 and nine months ended September 30, 2014. No transfers of assets between Level 1 and Level 2 of the fair value measurement hierarchy occurred during the three and nine months ended September 30, 2014.

Secured Debt

As disclosed in Note 9, the Company entered into a loan and security agreement (the 2013 Loan Agreement) with MidCap Funding III, LLC, Oxford Finance LLC and Silicon Valley Bank (SVB) in December 2013. The Company also entered into an equipment loan with SVB in 2011. The carrying amount

of the Company's borrowings approximates fair value at September 30, 2014. The Company's secured debt is classified as Level 2 and the fair value is estimated using quoted prices for similar liabilities in active markets, as well as inputs that are observable for the liability (other than quoted prices), such as interest rates that are observable at commonly quoted intervals.

In connection with the 2013 Loan Agreement, as disclosed in Note 9, the Company recorded a contingent liability of approximately $0.3 million representing the fair value of a contingent payment of up to $0.4 million related to a success fee payable within six months of a trigger event, with the trigger event being regulatory acceptance of a New Drug Application (NDA) or the Marketing Authorization Application (MAA) submission. This is effective five years from the closing of the 2013 Loan Agreement. The success fee payable to the lender was probability adjusted and discounted utilizing an appropriate discount rate and classified as Level 3.

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For the nine months ended September 30, 2014, change in fair value of the contingent success fee payable was $30 thousand, and is recorded in other income/(expense) in the Company's consolidated statements of operations.

	(in thousands)
Fair value balance at December 31, 2013	$ 264
Change in fair value	30
Fair value balance at September 30, 2014	$ 294

Contingent Consideration Payable

The Company recorded a liability upon the acquisition of Callidus to estimate the fair value of future contingent consideration payments which may be made to former Callidus stockholders, as outlined under the terms of the merger agreement with Callidus. These contingent payments are owed if upon the achievement by the Company of certain clinical milestones of up to $35 million and regulatory approval milestones of up to $105 million as set forth in the Merger Agreement, provided that the aggregate consideration shall not exceed $130 million. The fair values of these Level 3 liabilities are estimated using a probability-weighted discounted cash flow analysis. Such valuations require significant estimates and assumptions including but not limited to: determining the timing and estimated costs to complete the in-process projects, projecting regulatory approvals, estimating future cash flows, and developing appropriate discount rates.

Subsequent changes in the fair value of these contingent consideration liabilities are recorded to the "Change in fair value of contingent consideration payable" expense line item in the consolidated statements of operations under operating expenses. For the nine months ended September 30, 2014, the recognized amount of the contingent consideration payable decreased by $0.4 million as a result of changes in certain probability factors and the time value of money.

	(in thousands)
Fair value balance at December 31, 2013	$ 10,600
Change in fair value	(400)
Fair value balance at September 30, 2014	$ 10,200

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 was determined using the Black-Scholes model. The Company determined the warrant liability is most appropriately classified within Level 3 of the fair value hierarchy. This liability was subject to a fair value mark-to-market adjustment each period and the Company recognized the change in the fair value of the warrant liability as non-operating expense of $0.3 million for the three months ended March 31, 2013 and the resulting fair value of the warrant liability at March 31, 2013 was $1.2 million. The warrants expired on March 2, 2014 and hence the warrant liability is no longer recognized on the consolidated balance sheet as of September 30, 2014.

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

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

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

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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, 2014, are identified in the following table (in thousands):

	Level 1	Level 2	Total
Assets:			
Cash/ money market funds	$ 19,671	$ —	$ 19,671
Corporate debt securities	—	53,662	53,662
Commercial paper	—	11,499	11,499
Certificate of deposit	—	350	350
	$ 19,671	$ 65,511	$ 85,182

	Level 1	Level 2	Level 3	Total
Liabilities:				
Secured debt	$ —	$ 14,302	$ —	$ 14,302
Contingent success fee payable	—	—	294	294
Contingent consideration payable	—	—	10,200	10,200
	$ —	$ 14,302	$ 10,494	$ 24,796

Note 9. Short-Term Borrowings and Long-Term Debt

In December 2013, the Company entered into the 2013 Loan Agreement with a lending syndicate consisting of MidCap Funding III, LLC, Oxford Finance LLC, and SVB which provides an aggregate of $25 million (the Term Loan). The Company drew $15 million of the aggregate principal amount of the Term Loan at the end of December 2013 (the First Tranche) and may draw up to an additional $10 million through the end of the fourth quarter of 2014 (the Second Tranche). The principal outstanding balance of the First Tranche bears interest at a rate per annum fixed at 8.5%. If the Company draws from the Second Tranche, the principal outstanding balance of the Second Tranche will also have a fixed interest rate, which will be determined by reference to the applicable index rate at the time of the draw. The Company will make interest-only payments on the Term Loan beginning January 1, 2014 and continuing through April 1, 2015, after which the Company will repay the aggregate principal outstanding balance of the Term Loan in 33 equal monthly installments of principal, plus accrued interest at the applicable rate. The Term Loan matures on December 27, 2017. At September 30, 2014, the total principal amount due under the Term Loan was $15 million.

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

For the three months ended September 30, 2014, the Company amortized the debt discount and the deferred financing using the effective interest method and recorded amortization expense of $45 thousand and $6 thousand, respectively, under operating expense on the consolidated statement of operations. For the nine months ended September 30, 2014, the Company amortized the debt discount and the deferred financing using the effective interest method and recorded amortization expense of $131 thousand and $18 thousand, respectively, under operating expense on the consolidated statement of operations.

In February 2012, the Company borrowed approximately $1.0 million from a loan and security agreement (the 2011 Loan Agreement) with SVB which was to be repaid over the following 2.5 years. The 2011 Loan Agreement contains financial covenants and the Company has at all times been in compliance with these covenants. As of September 30, 2014, the 2011 Loan Agreement has been fully repaid and there is no amount currently due.

The carrying amount of the Company's borrowings approximates fair value at September 30, 2014.

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Note 10. Collaborative Agreements

GSK

In October 2010, the Company entered into the Original Collaboration Agreement with Glaxo Group Limited, an affiliate of GSK, to develop and commercialize migalastat. Under the terms of the Original Collaboration Agreement, GSK received an exclusive worldwide license to develop, manufacture and commercialize migalastat. In consideration of the license grant, the Company received an upfront, license payment of $30 million from GSK and was eligible to receive further payments of approximately $173.5 million upon the successful achievement of development, regulatory and commercialization milestones, as well as tiered double-digit royalties on global sales of migalastat GSK and the Company were jointly funding development costs in accordance with an agreed upon development plan. Additionally, GSK purchased approximately 6.9 million shares of the Company's common stock at $4.56 per share, a 30% premium on the average price per share of the Company's stock over a 60 day period preceding the closing date of the transaction. The total value of this equity investment to the Company was approximately $31 million.

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

Additionally, simultaneous with entry into the Expanded Collaboration Agreement, Amicus and GSK entered into a Securities Purchase Agreement (the 2012 SPA) pursuant to which GSK purchased approximately 2.9 million shares of Amicus common stock at a price of $6.30 per share for proceeds of $18.6 million.

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

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

Biogen

In September 2013, the Company entered into a license and collaboration agreement (the "Biogen Agreement") with Biogen to discover, develop and commercialize novel small molecules that target the GCase enzyme for the treatment of Parkinson's disease. Biogen was responsible for funding all discovery, development, and commercialization activities and the Company was reimbursed for all full-time employees working on the project as part of a cost sharing arrangement.

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 Biogen 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.

For the nine months ended September 30, 2014, the Company recognized $1.2 million in Research Revenue for work performed under the cost sharing arrangement of the Biogen Agreement.

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

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Note 11. Restructuring Charges

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

In December 2013, the Company initiated and completed a facilities consolidation effort, closing one of its subleased locations in San Diego, CA. The Company recorded a total charge of $2.0 million during the fourth quarter of 2013 which included $1.2 million for employment termination costs payable and a facilities consolidation charge of $0.8 million consisting of lease payments of $0.7 million related to the net present value of the net future minimum lease payments at the cease-use date and the write-down of the net book value of the fixed assets in the vacated building of $0.1 million. At September 30, 2014, $77 thousand of the restructuring charges related to employment termination costs were unpaid and classified under accrued expenses on the balance sheet.

The following table summarizes the restructuring charges and utilization for the nine months ended September 30, 2014 (in thousands):

	Balance as of December 31, 2013	Charges	Cash Payments	Adjustments	Balance as of September 30, 2014
Employment termination costs	$ 1,139	$ —	$ (1,062)	$ —	$ 77
Facilities consolidation	703	—	(299)	(74)	330
	$ 1,842	$ —	$ (1,361)	$ (74)	$ 407

Note 12. Subsequent Events

In October 2014, GSK sold 11,315,825 shares of our common stock at a price of $5.29 per share, representing their entire holdings in our common stock. We did not sell any shares or receive any proceeds and the total number of shares of our outstanding common stock did not change as a result of this sale.

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

Overview

We are a biopharmaceutical company focused on the discovery, development and commercialization of next-generation medicines for a range of rare and orphan diseases, with a focus on improved therapies for lysosomal storage disorders (LSDs). Our development programs include next-generation enzyme replacement therapies (ERTs) for LSDs, including Fabry disease, Pompe disease and Mucopolysaccharoidosis Type I (MPS I). We are also developing novel

small molecules as monotherapy treatments for Fabry disease and Parkinson's disease. We believe that our platform technologies and our advanced product pipeline uniquely position us at the forefront of developing therapies for rare and orphan diseases.

Program Status

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

We are conducting two global Phase 3 registration studies (Study 011 and Study 012) of the oral pharmacological chaperone migalastat HCl ("migalastat") as a monotherapy. Both studies enrolled males and females with Fabry disease who have alpha-Gal A mutations that are amenable to migalastat monotherapy. Amenable mutations are defined as having an absolute increase of 3% of wild type alpha-Gal A enzyme activity and a relative increase of 20% when exposed to migalastat in a cell-based *in vitro* assay.

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

Study 011 was designed to measure the reduction of the disease substrate (Globotriaosylceramide, or GL-3) in the interstitial capillaries of the kidney following treatment with oral migalastat (150 mg every other day). The 24-month study began with a 6-month double-blind, placebo-controlled treatment period, after which all patients were treated with migalastat for a 6-month open-label follow-up period, and a subsequent 12-month open-label extension phase. The study also measured clinical outcomes, including renal function, as secondary endpoints.

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

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

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

In April 2014, we announced positive 12- and 24-month results from Study 011 in patients with GLP HEK amenable mutations.

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

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- Subjects who remained on migalastat demonstrated a durable reduction in kidney interstitial capillary GL-3, as well as a durable reduction in lyso-Gb3.
- Kidney function, as measured by estimated glomerular filtration rate (eGFR) and iohexol measured GFR (mGFR), remained stable following 18-24 months of treatment with migalastat.
- From a safety perspective, migalastat was generally safe and well-tolerated.

In October 2014, we announced further positive data from Study 011. Assessment of kidney function by estimated glomerular filtration rate (eGFR) for patients receiving migalastat in Study 011 for at least 18 months and continuing migalastat treatment in Study 041 (an open-label extension study) showed continued stability of kidney function for an average of 32 months. Decline in kidney function is a key cause of morbidity and mortality in patients with Fabry disease. Measured (iohexol) GFR (mGFR) was not collected in Study 041; mGFR was previously reported with topline Study 011 results.

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

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

In August 2014, we announced positive 18-month data from the Study 012. A summary of the 18-month results are as follows:

- Migalastat had a comparable effect to ERT on patients' kidney function as measured by the change in eGFR and mGFR.
- Levels of plasma lyso-Gb3, an important biomarker of disease, remained low and stable in patients with amenable mutations who switched from ERT to migalastat.
- Migalastat was generally safe and well-tolerated.
- Of 48 patients with GLP HEK-amenable mutations who completed Study 012, 46 (96%) elected to continue with the 12-month treatment extension and 45 remain on migalastat today as their only treatment for Fabry disease.

We plan on meeting with the EMEA in the fourth quarter of 2014 and the US FDA in early 2015 as we work to make migalastat available for all amenable Fabry patients as quickly as possible.

Migalastat HCl Combination Programs for Fabry Disease

Using our CHART platform, we completed an open-label Phase 2 safety and pharmacokinetics study (Study 013) that investigated two oral doses of migalastat (150 mg and 450 mg) co-administered with Fabrazyme® (agalsidase beta) or Replagal® (agalsidase alfa) in males with Fabry disease. Preliminary results from Study 013 showed increased levels of active alpha-Gal A enzyme levels in plasma and increased alpha-Gal A enzyme in skin following co-administration compared to ERT alone. In parallel, we and GSK completed preclinical studies to evaluate migalastat co-formulated with a proprietary investigational ERT (JR-051, recombinant human alpha-Gal A enzyme). Based on these results, we plan to advance migalastat co-formulated with ERT for Fabry disease. We conducted a Phase 1study in healthy volunteers to investigate the PK of IV migalastat and identify optimal dosings of migalastat to use in a Phase 1/2 clinical study of migalastat co-formulated with ERT in Fabry patients. We are currently evaluating a long-term strategy for supplying late-stage clinical and commercial ERT, which may include developing or in-licensing a recombinant alpha-Gal A enzyme. Upon finalization of a long term ERT supply agreement, we plan to initiate a Phase 1/2 clinical study of migalastat co-formulated with ERT in Fabry patients.

Next-Generation ERT for Pompe Disease

In 2013, we completed a Phase 2 safety and pharmacokinetics study (Study 010) that investigated single ascending oral doses of a pharmacological chaperone, AT2220 (50 mg, 100 mg, 250 mg, and 600 mg) co-administered with Myozyme® or

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Lumizyme® (alglucosidase alfa or recombinant human GAA enzyme, rhGAA) marketed by Genzyme, in patients with Pompe disease. Each patient received one infusion of ERT alone, and then a single oral dose of 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.

We are utilizing our CHART platform in combination with our uniquely-engineered, proprietary recombinant human acid-alpha glucosidase (rhGAA, designated ATB200) to develop a next-generation ERT for Pompe disease. We acquired ATB200 as well as our enzyme targeting technology through our purchase of Callidus Biopharma. ATB200 is differentiated from other Pompe ERTs by its unique carbohydrate structure.

In preclinical studies, ATB200 was shown to have superior uptake and activity in disease-relevant tissues that correlated with clearance of accumulated glycogen substrate when compared to current standard of care. ATB200 may be further improved through the application of the Company's proprietary conjugation technology to attach vIGF2 (a variant of the insulin-like growth factor 2) to further enhance lysosomal drug targeting. Preclinical results have shown that ATB200 and ATB200 conjugated with vIGF-2 were better than Lumizyme for clearing glycogen in skeletal muscles in *Gaa* knock-out mice. These studies also demonstrated that ATB200 and ATB200+vIGF-2 co-formulated with a pharmacological chaperone can lead to even further glycogen reduction.

These results taken together with data from our clinical and preclinical studies of pharmacological chaperones in combination with ERT, support our further development of a next-generation ERT for Pompe disease We are currently investigating ATB200, with and without a pharmacological chaperone, and with and without the vIGF-2 tag in preclinical studies to determine the optimal product to bring forward into clinical studies in Pompe patients.

Collaboration with GSK

In November 2013, we entered into the Revised Agreement (the Revised Agreement) with GlaxoSmithKline plc (GSK), pursuant to which we have obtained global rights to develop and commercialize migalastat as a monotherapy and in combination with ERT for Fabry disease. Under the Revised Agreement, Amicus received a payment of $1.9 million to reimburse development costs between November 19, 2013 and December 31, 2013, in December 2013, and $0.8 million for reimbursement of development costs for the period August 1, 2013 to November 18, 2013 in January 2014, according to the earlier Expanded Agreement. The Revised Agreement amends and replaces in its entirety the Expanded Collaboration Agreement entered into between Amicus and GSK in July 2012. Under the terms of the Revised Agreement, there is no upfront payment from Amicus to GSK. For the next-generation Fabry ERT (migalastat co-formulated with ERT), GSK is eligible to receive single-digit royalties on net sales in eight major markets outside the U.S. For migalastat monotherapy, GSK is eligible to receive post-approval and sales-based milestones, as well as tiered royalties in the mid-teens in eight major markets outside the U.S.

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

Collaboration with Biogen

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

Acquisition of Callidus Biopharma, Inc.

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

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

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Other Potential Alliances and Collaborations

We continually evaluate other potential collaborations and business development opportunities that would bolster our ability to develop therapies for rare and orphan diseases including licensing agreements and acquisitions of businesses and assets. We believe such opportunities may be important to the advancement of our current product candidate pipeline, the expansion of the development of our current technology, gaining access to new technologies and in our transformation from a development stage company to a commercial biotechnology company.

Financial Operations Overview

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. For the nine months ended September 30, 2014, we accumulated a loss of $47.7 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

In September 2013, we entered into collaboration with Biogen to discover, develop and commercialize novel small molecules for the treatment of Parkinson's disease. For the three and nine months ended September 30, 2014, we recognized $0.3 million and $1.2 million, respectively, as Research Revenue for reimbursed research and development costs.

GSK

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

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

Research and Development Expenses

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

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

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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. For the nine months ended September 30, 2014, we incurred research and development expense in the aggregate of $32.0 million.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
Projects	2013	2014	2013	2014
Third party direct project expenses				
Monotherapy Studies				
Migalastat HCl (Fabry Disease — Phase 3)	$ 2,644	$ 3,141	$ 6,996	$ 8,961
Afegostat tartrate (Gaucher Disease — Phase 2*)	4	2	78	10
Combination Studies				
Fabry CHART (Fabry Disease — Phase 2)	30	284	568	902
Pompe CHART (Pompe Disease — Phase 2)	929	2,455	2,973	4,080
Gaucher CHART (Gaucher Disease — Preclinical)	—	—	21	—
Neurodegenerative Diseases (Preclinical)	57	35	100	265
Total third party direct project expenses	3,664	5,917	10,736	14,218
Other project costs (1)				
Personnel costs	4,610	4,361	16,087	12,811
Other costs (2)	1,836	1,771	6,001	4,990
Total other project costs	6,446	6,132	22,088	17,801
Total research and development costs	$ 10,110	$ 12,049	$ 32,824	$ 32,019

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

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General and Administrative Expense

General and administrative expense consists primarily of salaries and other related costs, including stock-based compensation expense, for persons serving in our executive, finance, accounting, legal, information technology and human resource functions. Other general and administrative expense includes facility-related costs not otherwise included in research and development expense, promotional expenses, costs associated with industry and trade shows, and professional fees for legal services, including patent-related expense and accounting services. For the nine months ended September 30, 2014, we spent $15.2 million on general and administrative expense.

Interest Income and Interest Expense

Interest income consists of interest earned on our cash and cash equivalents and marketable securities. Interest expense consists of interest incurred on our secured loan agreement.

Critical Accounting Policies and Significant Judgments and Estimates

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

There were no significant changes during the quarter ended September 30, 2014 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, 2013. However, we believe that the following accounting policies are the most critical to aid you in fully understanding and evaluating our financial condition and results of operations.

Revenue Recognition

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

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

determined to be a single unit of accounting, the total consideration is applied as revenue for the single unit of accounting and is recognized over the period of inception through the date where the last deliverable within the single unit of accounting is expected to be delivered.

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

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

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

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

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

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

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

Nonqualified Cash Deferral Plan

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

As of September 30, 2014, the amounts deferred under the Deferral Plan have not been invested. The investments are expected to be made in the fourth quarter of fiscal year 2014. All of the investments held in the Deferral Plan will be classified as investments held-to-maturity and recorded at fair value with changes in the investments' fair value recognized as earnings in the period they occur. The corresponding liability for the Deferral Plan is included in other non-current liability in our consolidated balance sheets.

Equity Incentive Plan

In June 2014, our shareholders approved the Amended and Restated 2007 Equity Incentive Plan (the Plan). The amendment to the Plan makes an additional 6.0 million shares of our common stock available for issuance and increases the maximum number of shares within the Plan that may be issued as restricted stock, restricted stock units (RSUs), stock grants and any other similar awards from 1.1 million to 1.5 million shares. As of September 30, 2014, awards issued under the Plan include both stock options and RSUs.

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Stock Option Grants

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

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

The fair value of the stock 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,	
	2013	2014	2013	2014
Expected stock price volatility	81.7%	81.0%	82.0%	81.3%
Risk free interest rate	1.8%	1.9%	1.3%	1.9%
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 our stock options for the six months ended September 30, 2014 is as follows:

	Number of Shares (in thousands)	Weighted Average Exercise Price	Weighted Average Remaining Contractual Life	Aggregate Intrinsic Value (in millions)
Balance at December 31, 2013	9,041.1	$ 5.65		
Options granted	2,894.1	$ 2.87		
Options exercised	(594.4)	$ 3.53		
Options forfeited	(1,012.3)	$ 5.82		
Balance at September 30, 2014	10,328.5	$ 4.97	6.9 years	$ 17.6
Vested and unvested expected to vest, September 30, 2014	9,649.5	$ 5.10	6.8 years	$ 15.6
Exercisable at September 30, 2014	5,551.1	$ 6.40	5.2 years	$ 4.9

As of September 30, 2014, the total unrecognized compensation cost related to non-vested stock options granted was $8.3 million and is expected to be recognized over a weighted average period of 2.4 years.

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Restricted Stock Units

In April 2014, the Compensation Committee made awards of restricted stock units to certain employees of the Company. The RSUs were awarded under the Plan are generally subject to graded vesting of 50% of the RSUs on the 13th month anniversary of the grant date and the remaining 50% of the RSUs on the 20th month anniversary of the grant date, in each case, contingent on such employee's continued service on such date.

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

A summary of non-vested RSU activity under the plan for the nine months ended September 30, 2014 is as follows:

	Number of Shares (in thousands)	Weighted Average Grant Date Fair Value	Weighted Average Remaining Years	Aggregate Intrinsic Value (in millions)
Non-vested units as of December 31, 2013	—	$ —		
Granted	975	$ 2.28		
Vested	—	$ —		
Forfeited	(20)	$ 2.15		
Non-vested units as of September 30, 2014	955	$ 2.28	1.0	$ 3.5

For the nine months ended September 30, 2014, there were no RSUs that vested and all non-vested units are expected to vest over their normal term. As of September 30, 2014, there was $1.5 million of total unrecognized compensation cost related to unvested RSUs with service-based vesting conditions. These costs are expected to be recognized over a weighted average period of 1.0 year.

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

Compensation Expense Related to Equity Awards

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2013	2014	2013	2014
Stock compensation expense recognized in:				
Research and development expense	$ 824	$ 698	$ 2,634	$ 1,950
General and administrative expense	693	953	2,040	2,448
Total stock compensation expense	$ 1,517	$ 1,651	$ 4,674	$ 4,398

Warrants

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

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

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

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

(In thousands, except per share amounts)	Three Months Ended September 30,		Nine Months Ended September 30,	
	2013	2014	2013	2014
Historical				
Numerator:				
Net loss attributable to common stockholders per common	$ (14,589)	$ (17,149)	$ (47,396)	$ (47,706)
Denominator				
Weighted average common shares outstanding — basic and diluted	49,621,188	78,889,346,	49,621,188	70,216,251

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

	As of September 30,	
	2013	2014
Options to purchase common stock	10,112	10,329
Outstanding warrants, convertible to common stock	1,404	1,600
Unvested restricted stock units	—	955
Total number of potentially issuable shares	11,516	12,884

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Results of Operations

Three Months Ended September 30, 2014 Compared to Three Months Ended September 30, 2013

Revenue. In September 2013, we entered into a collaboration with Biogen to discover, develop and commercialize novel small molecules for the treatment of Parkinson's disease. For the three months ended September 30, 2014, and September 30, 2013, we recognized $293 thousand and $39 thousand, respectively, as Research Revenue for reimbursed research and development costs.

Research and Development Expense. Research and development expense was $12.0 million for the three months ended September 30, 2014, representing an increase of $1.9 million or 19.0% from $10.1 million for the three months ended September 30, 2013. The increase in research and development costs was due primarily to contract research and manufacturing of $2.3 million arising from the timing of studies and changes in research plans. These research plans included increased spending in the Pompe CHART programs, Fabry migalastat program and Fabry CHART programs. This was partially offset by a decrease in personnel costs for $0.2 million.

General and Administrative Expense. General and administrative expense was $5.3 million for the three months ended September 30, 2014, representing an increase of $0.7 million or 15.0% from $4.6 million for the three months ended September 30, 2013. The main drivers of the increases were personnel costs of $0.3 million and legal fees of $0.2 million.

Changes in Fair Value of Contingent Consideration Payable. For the three months ended September 30, 2014, we recorded income of $0.6 million representing a decrease in fair value of contingent consideration payable, which was related to the Callidus acquisition.

Restructuring Charges. Restructuring charges were recorded in 2013 due to the corporate restructuring implemented in the fourth quarter of 2013. For the three months ended September 30, 2014, we recorded a $15 thousand increase to the liability to reflect the change in fair value of the net future discounted cash flows of the San Diego office lease.

Interest Income. Interest income was $55 thousand for the three months ended September 30, 2014, representing an increase of $19 thousand or 53.0% from $36 thousand for the three months ended September 30, 2013. The increase in interest income was due to the overall higher average cash and investment balances as a result of ATM sales and cash received from option exercises.

Interest Expense. Interest expense was approximately $377 thousand for the three months ended September 30, 2014 compared to $7 thousand for the three months ended September 30, 2013. Interest expense was higher due to the $15 million loan secured in December 2013.

Change in Fair Value of Warrant Liability. In connection with the sale of our common stock and warrants from the registered direct offering in March 2010, we recorded the warrants as a liability at their fair value using a Black-Scholes model and re-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. As these warrants expired in March 2014, for the three months ended September 30, 2014, there was no expense or income as compared to an income of $0.5 million related to the decrease in fair value of these warrants for the three months ended September 30, 2013.

Other Income/Expense. Other income/expenses for the three months ended September 30, 2014 included charges of $11 thousand for increase in the fair value of the success fee payable, which was related to the $15 million secured loan in 2013. There was no other income/expense for the three months ended September 30, 2013.

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Results of Operations

Nine Months Ended September 30, 2014 Compared to Nine Months Ended September 30, 2013

Revenue. In September 2013, we entered into a collaboration with Biogen to discover, develop and commercialize novel small molecules for the treatment of Parkinson's disease. For the nine months ended September 30, 2014, we recognized $1.2 million as Research Revenue for reimbursed research and development costs, compared to $39 thousand for the nine months ended September 30, 2013.

Research and Development Expense. Research and development expense was $32.0 million for the nine months ended September 30, 2014, representing a decrease of $0.8 million or 2.0% from $32.8 million for the nine months ended September 30, 2014. The decrease in research and development costs was due to a decrease in personnel costs of $3.3 million, consultants $3.1 million, recruitment fees of $0.2 million and facilities costs of $0.3 million. This was partially offset by an increase in contract manufacturing and research costs. Contract research and manufacturing costs increased by $3.2 million due to the timing of studies and changes in research plans. These research plans included increased spending in the Fabry migalastat program, Fabry CHART programs and the Pompe CHART programs.

General and Administrative Expense. General and administrative expense was $15.2 million for the nine months ended September 30, 2014, representing an increase of $0.9 million or 6.0% from $14.3 million for the nine months ended September 30, 2013. The variance was primarily due to higher legal and accounting fees associated with the Callidus acquisition and the ATM equity program.

Changes in Fair Value of Contingent Consideration Payable. For the nine months ended September 30, 2014, we recorded income of $0.4 million representing a decrease in fair value of contingent consideration payable, which was related to the Callidus acquisition.

Restructuring Charges. Restructuring charges were recorded in 2013 due to the corporate restructuring implemented in the fourth quarter of 2013. For the nine months ended September 30, 2014, we recorded a $0.1 million adjustment to the liability to reflect the change in fair value of the net future discounted cash flows of the San Diego office lease.

Interest Income. Interest income was $0.1 million for the nine months ended September 30, 2014 and September 30, 2013.

Interest Expense. Interest expense was approximately $1.1 million for the nine months ended September 30, 2014 compared to $26 thousand for the nine months ended September 30, 2013. Interest expense was higher due to the $15 million loan secured in December 2013.

Change in Fair Value of Warrant Liability. In connection with the sale of our common stock and warrants from the registered direct offering in March 2010, we recorded the warrants as a liability at their fair value using a Black-Scholes model and remeasure the fair value at each reporting date until exercised or expired. Changes in the fair value of the warrants are reported in the statements of operations as non-operating income or expense. As these warrants expired in March 2014, for the nine months ended September 30, 2014, there was no expense or income as compared to an income of $0.9 million related to the decrease in fair value of these warrants for the nine months ended September 30, 2013.

Other Income/Expense. Other income/ expenses for the nine months ended September 30, 2014 included charges of $30 thousand for increase in the fair value of the success fee payable, which was related to the $15 million secured loan in 2013.There was no other income/expense for the nine months ended September 30, 2013.

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Liquidity and Capital Resources

Source of Liquidity

In March 2014, we entered into a Sales Agreement with Cowen and Company, LLC (Cowen) to create an at-the-market (ATM) equity program under which we would from time to time may offer and sell shares of our common stock, par value $0.01 per share, having an aggregate offering price of up to $40 million (ATM Shares) through Cowen. Upon delivery of a placement notice and subject to the terms and conditions of the ATM agreement, Cowen used its commercially reasonable efforts to sell the ATM Shares, based upon our instructions. We provided Cowen with customary indemnification rights, and Cowen was entitled to a commission at a fixed commission rate of up to 3.0% of the gross proceeds per Share sold. Sales of the Shares under the Agreement were made in transactions that were deemed to be "at the market offerings" as defined in Rule 415 under the Securities Act of 1933, as amended, including sales made by means of ordinary brokers' transactions, including on The NASDAQ Global Market, at market prices or as otherwise agreed with Cowen. We began the sales of ATM Shares in May 2014 and sold 14.3 million shares of common stock resulting in net proceeds of $38.7 million, which included Cowen's commission of $1.1 million and legal fees of $0.1 million. All sales under the ATM equity program have been completed.

As a result of our significant research and development expenditures and the lack of any approved products to generate product sales revenue, we have not been profitable and have generated operating losses since we were incorporated in 2002. We have funded our operations principally with $148.7 million of proceeds from redeemable convertible preferred stock offerings, $75.0 million of gross proceeds from our IPO in June 2007, $18.5 million of gross proceeds from our Registered Direct Offering in March 2010, $65.6 million of gross proceeds from our stock offering in March 2012, $52.9 million from GSK's investments in the Company in October 2010, July 2012 and November 2013, $40.0 million of gross proceeds from ATM sales and $80.0 million from non-refundable license fees from collaborations.

In December 2013 we entered into a credit and security agreement with a lending syndicate which provides an aggregate of $25 million. We drew $15 million of the aggregate principal amount in December 2013.

As of September 30, 2014, we had cash, cash equivalents and marketable securities of $85.2 million. We invest cash in excess of our immediate requirements with regard to liquidity and capital preservation in a variety of interest-bearing instruments, including obligations of U.S. government agencies and money market accounts. Wherever possible, we seek to minimize the potential effects of concentration and degrees of risk. Although we maintain cash balances with financial institutions in excess of insured limits, we do not anticipate any losses with respect to such cash balances.

Net Cash Used in Operating Activities

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 collaboration agreement of $1.1million; 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.

Net cash used in operations for the nine months ended September 30, 2014 was $37.2 million, due primarily to the net loss for the nine months ended September 30, 2014 of $47.7 million and the change in operating assets and liabilities of $5.3 million. The change in operating assets and liabilities consisted of a decrease in receivables from collaboration agreements of $0.8 million; a decrease of $3.4 million in prepaid assets primarily related to Net Operating Loss (NOL) receivable; an increase in accounts payable and accrued expenses of $1.2 million, mainly related to program expenses as well as reclass of success fee from noncurrent liability to a current liability.

Net Cash Provided by/ (Used in) Investing Activities

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 used in investing activities for the nine months ended September, 2014 was $27.3 million. Net cash used in investing activities reflects $75.1 million for the purchase of marketable securities and $0.2 million for the acquisition of property and equipment, partially offset by $48.0 million from the sale and redemption of marketable securities.

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Net Cash (Used in)/ Provided by Financing Activities

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.

Net cash provided by financing activities for the nine months ended September 30, 2014 was $40.5 million. Net cash provided reflects $38.7 million in net proceeds from sales of common stock under our ATM agreement with Cowen, $2.1 million from stock option exercises, partially offset by $0.3 million for the payments of 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;
- the cost of manufacturing drug supply for our clinical and preclinical studies, including the significant cost of ERT cell line development and manufacturing as well as the cost of manufacturing the vIGF-2 peptide tag;
- the scope, progress, results and costs of preclinical development, laboratory testing and clinical trials for our product candidates including those testing the use of pharmacological chaperones co-formulated and co-administered with ERT and for the treatment of lysosomal storage diseases;
- the costs, timing and outcome of regulatory review of our product candidates;
- the number and development requirements of other product candidates that we pursue;

- the costs of commercialization activities, including product marketing, sales and distribution;
- the emergence of competing technologies and other adverse market developments;
- the costs of preparing, filing and prosecuting patent applications and maintaining, enforcing and defending intellectual property related claims;
- the extent to which we acquire or invest in businesses, products or technologies; and
- our ability to establish collaborations and obtain milestone, royalty or other payments from any such collaborators.

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

Financial Uncertainties Related to Potential Future Payments

Milestone Payments / Royalties

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

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

For duvoglustat, we will owe royalties only to MSSM.

We have acquired rights to develop and commercialize afegostat through licenses granted by various parties. Two agreements related to afegostat require us to make milestone payments if certain specified pre-commercialization events occur. Upon the satisfaction of certain milestones and assuming successful development of afegostat, we may be obligated, under the agreements that we have in place, to make future milestone payments aggregating up to approximately $7.9 million. However, such potential milestone payments are subject to many uncertain variables that would cause such payments, if any, to vary in size. We will owe royalties to MSSM, NovoNordisk and University of Maryland, Baltimore County.

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

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ITEM 3. Quantitative and Qualitative Disclosures about Market Risk

Market risk is the risk of change in fair value of a financial instrument due to changes in interest rates, equity prices, creditworthiness, financing, exchange rates or other factors. Our primary market risk exposure relates to changes in interest rates in our cash, cash equivalents and marketable securities. We place our investments in high-quality financial instruments, primarily money market funds, corporate debt securities, asset backed securities and U.S. government agency notes with maturities of less than one year, which we believe are subject to limited interest rate and credit risk. The securities in our investment portfolio are not leveraged, are classified as available-for-sale and, due to the short-term nature, are subject to minimal interest rate risk. We currently do not hedge interest rate exposure and consistent with our investment policy, we do not use derivative financial instruments in our investment portfolio. At September 30, 2014, we held $85.2 million in cash, cash equivalents and available for sale securities and due to the short-term maturities of our investments, we do not believe that a 10% change in average interest rates would have a significant impact on the fair value of our investments. Our outstanding debt has a fixed interest rate and therefore, we have no exposure to interest rate fluctuations.

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

ITEM 4. CONTROLS AND PROCEDURES

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

During the fiscal quarter covered by this report, there has been no change in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) that occurred during the 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, 2013.

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

Recent Sales of Unregistered Securities

None.

Issuer Purchases of Equity Securities

The Company did not purchase any shares of its common stock for the three months ended September 30, 2014.

ITEM 3. DEFAULTS UPON SENIOR SECURITIES

None.

ITEM 4. MINE SAFETY DISCLOSURES

None.

ITEM 5. OTHER INFORMATION

None.

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ITEM 6. EXHIBITS

Exhibit Number	Description
3.1 (1)	Restated Certificate of Incorporation
3.2 (2)	Amended and Restated By-laws
10.1 (3)	Amicus Therapeutics, Inc. Cash Deferral Plan
10.2 (4)	Amendment No.1 to the Amicus Therapeutics, Inc. Cash Deferral Plan
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 three months ended September 30, 2014, formatted in XBRL (Extensible Business Reporting Language) and filed electronically herewith: (i) the Consolidated Balance Sheets; (ii) the Consolidated Statements of Operations; (iii) the Consolidated Statements of Comprehensive Loss; (iv) the Consolidated Statements of Cash Flows; (v) and the Notes to the Consolidated Financial Statements.

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

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

(3) Incorporated by reference to Exhibit 10.1 to our Current Report on Form 8-K filed on July 2, 2014

(4) Incorporated by reference to Exhibit 10.1 to our Current Report on Form 8-K filed October 16, 2014.

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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 6, 2014 By: /s/ John F. Crowley
John F. Crowley
Chairman and Chief Executive Officer
(Principal Executive Officer)

Date: November 6, 2014 By: /s/ William D. Baird III
William D. Baird III
Chief Financial Officer
(Principal Financial Officer)

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INDEX TO EXHIBITS

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Exhibit 31.1

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

I, John F. Crowley, certify that:

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

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

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

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

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

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

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

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

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

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

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

Date: November 6, 2014

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

Exhibit 31.2

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

I, William D. Baird III, certify that:

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

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

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

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

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

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

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

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

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

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

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

Date: November 6, 2014

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

Exhibit 32.1

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

Each of the undersigned hereby certifies, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, in his capacity as an officer of Amicus Therapeutics, Inc. (the "Company"), that, to his knowledge, the Quarterly Report of the Company on Form 10-Q for the period ended September 30, 2014, fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934 (15 U.S.C. 78m or 78o(d)) and that the information contained in such report fairly presents, in all material respects, the financial condition and results of operations of the Company. This written statement is being furnished to the Securities and Exchange Commission as an exhibit to such Form 10-Q. A signed original of this statement has been provided to the Company and will be retained by the Company and furnished to the Securities and Exchange Commission or its staff upon request.

Date: November 6, 2014 By: /s/ John F. Crowley
John F. Crowley
Chairman and Chief Executive Officer

Date: November 6, 2014 By: /s/ William D. Baird III
William D. Baird III
Chief Financial Officer