AMICUS THERAPEUTICS INC
Form 10-Q
May 05, 2014
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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 March 31, 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 April 28, 2014 was 64,340,526 shares.

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AMICUS THERAPEUTICS, INC.

Form 10-Q for the Quarterly Period Ended March 31, 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 i 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 ERT cell line development and manufacturing as well as the cost of manufacturing the vIGF-2 peptide tag;
- the scope, progress, results and costs of preclinical development, laboratory testing and clinical trials for our product candidates including those testing the use of pharmacological chaperones co-formulated and co-administered with ERT and for the treatment of lysosomal storage diseases;
- the costs, timing and outcome of regulatory review of our product candidates;
- the number and development requirements of other product candidates that we pursue;
- the costs of commercialization activities, including product marketing, sales and distribution;
- the emergence of competing technologies and other adverse market developments;
- the costs of preparing, filing and prosecuting patent applications and maintaining, enforcing and defending intellectual property related claims;
- the extent to which we acquire or invest in businesses, products and technologies;
- our ability to successfully incorporate Callidus Biopharma, Inc. (Callidus) and its product candidates and technology into our business; and
- our ability to establish collaborations and obtain milestone, royalty or other payments from any such collaborators.

We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make. We have included important factors in the cautionary statements included in 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.

(a development stage company)

Consolidated Balance Sheets

(Unaudited)

(in thousands, except share and per share amounts)

	December 31, 2013	March 31, 2014
Assets:		
Current assets:		
Cash and cash equivalents	$ 43,640	$ 27,533
Investments in marketable securities	38,360	44,121
Receivable due from GSK	759	38
Prepaid expenses and other current assets	5,519	1,758
Total current assets	88,278	73,450
Property and equipment, less accumulated depreciation and amortization of $9,973 and $10,385 at December 31, 2013 and March 31, 2014, respectively	4,120	3,748
In-process research & development	23,000	23,000
Goodwill	11,613	11,613
Other non-current assets	552	546
Total Assets	$ 127,563	$ 112,357
Liabilities and Stockholders Equity		
Current liabilities:		
Accounts payable and accrued expenses	$ 10,162	$ 9,167
Current portion of secured loan	299	199
Total current liabilities	10,461	9,366
Deferred reimbursements	36,677	36,677
Secured loan, less current portion	14,174	14,216
Contingent consideration payable	10,600	11,105
Deferred tax liability	9,186	9,186
Other non-current liability	714	723
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, 64,340,259 shares issued	679	703

and outstanding at March 31, 2014				
Additional paid-in capital		423,593		424,844
Accumulated other comprehensive income		1		2
Deficit accumulated during the development stage		(378,522)		(394,465)
Total stockholders equity		45,751		31,084
Total Liabilities and Stockholders Equity	$	127,563	$	112,357

See accompanying notes to consolidated financial statements

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Amicus Therapeutics, Inc.

(a development stage company)

Consolidated Statements of Operations

(Unaudited)

(in thousands, except share and per share amounts)

	Three Months Ended March 31,		Period from February 4, 2002 (inception) to March 31, 2014
	2013	**2014**	
Revenue:			
Research revenue	$	$ 456	$ 58,312
Collaboration and milestone revenue			64,382
Total revenue		456	122,694
Operating Expenses:			
Research and development	$ 11,989	$ 9,992	$ 367,829
General and administrative	4,823	5,176	156,682
Changes in fair value of contingent consideration payable		505	505
Restructuring charges		(8)	3,502
Impairment of leasehold improvements			1,030
Depreciation and amortization	439	412	13,899
In-process research and development			418
Total operating expenses	17,251	16,077	543,865
Loss from operations	(17,251)	(15,621)	(421,171)
Other income (expenses):			
Interest income	65	42	14,605
Interest expense	(10)	(355)	(2,823)
Change in fair value of warrant liability	(262)		2,461
Other (expense)/ income		(9)	243
Loss before tax benefit	(17,458)	(15,943)	(406,685)
Income tax benefit			12,220
Net loss	(17,458)	(15,943)	(394,465)
Deemed dividend			(19,424)
Preferred stock accretion			(802)
Net loss attributable to common stockholders	$ (17,458)	$ (15,943)	$ (414,691)
Net loss attributable to common stockholders per common share basic and diluted	$ (0.35)	$ (0.25)	
Weighted-average common shares outstanding basic and diluted	49,621,188	64,353,952	

See accompanying notes to consolidated financial statements

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Amicus Therapeutics, Inc.

(a development stage company)

Consolidated Statements of Comprehensive Loss

(Unaudited)

(in thousands)

	Three Months Ended March 31,		Period from February 4, 2002 (inception) to March 31, 2014
	2013	2014	
Net loss	$ (17,458)	$ (15,943)	$ (394,465)
Other comprehensive income/(loss):			
Unrealized gain on available-for- sale securities	1	1	2
Other comprehensive income, before income taxes	1	1	2
Provision for income taxes related to other comprehensive income Items (a)			
Other comprehensive income	$ 1	$ 1	$ 2
Comprehensive loss	$ (17,457)	$ (15,942)	$ (394,463)

(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.

(a development stage company)

Consolidated Statements of Cash Flows

(Unaudited) **(in thousands)**

	Three Months Ended March 31,		Period from February 4, 2002 (inception) to March 31,
	2013	2014	2014
Operating activities			
Net loss	$ (17,458)	$ (15,943)	$ (394,465)
Adjustments to reconcile net loss to net cash used in operating activities:			
Non-cash interest expense		59	584
Depreciation and amortization	439	412	13,899
Amortization of non-cash compensation			522
Stock-based compensation	1,574	1,260	49,366
Non-cash charge for stock based compensation issued to consultants			853
Restructuring charges		(8)	3,502
Change in fair value of warrant liability	262		(2,461)
Non-cash changes in the fair value of contingent consideration payable		505	505
Loss on disposal of asset			388
Stock-based license payments			1,220
Impairment of leasehold improvements			1,030
Non-cash charge for in-process research and development			418
Beneficial conversion feature related to bridge financing			135
Changes in operating assets and liabilities:			
Receivable due from GSK	1,917	721	(38)
Prepaid expenses and other current assets	517	3,761	(1,758)
Other non-current assets			(466)
Accounts payable and accrued expenses	(2,418)	(987)	5,723
Deferred reimbursements	1,267		36,677
Net cash used in operating activities	(13,900)	(10,220)	(284,366)
Investing activities			
Sale and redemption of marketable securities	34,135	11,465	850,244
Purchases of marketable securities	(26,998)	(17,227)	(894,481)
Purchases of property and equipment	(372)	(40)	(18,948)
Net cash provided by/(used in) investing activities	6,765	(5,802)	(63,185)
Financing activities			
Proceeds from the issuance of preferred stock, net of issuance costs			143,022
Proceeds from the issuance of common stock and warrants, net of issuance costs			208,441
Proceeds from the issuance of convertible notes			5,000
Payments of capital lease obligations			(5,587)
Payments of secured loan agreement	(100)	(100)	(4,554)
Payments related to deferred financing			(110)
Proceeds from exercise of stock options		15	3,356
Proceeds from exercise of warrants (common and preferred)			264
Proceeds from capital asset financing arrangement			5,611

Proceeds from secured loan agreement						19,641
Net cash (used in)/provided by financing activities		(100)		(85)		375,084
Net (decrease)/increase in cash and cash equivalents		(7,235)		(16,107)		27,533
Cash and cash equivalents at beginning of period		33,971		43,640		
Cash and cash equivalents at end of period	$	26,736	$	27,533	$	27,533

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Amicus Therapeutics, Inc.

(a development stage company)

Consolidated Statements of Cash Flows (continued)

(Unaudited)

(in thousands)

	Three Months Ended March 31,		Period from February 4, 2002 (inception) to March 31, 2014
	2013	2014	
Supplemental disclosures of cash flow information			
Cash paid during the period for interest	$ 7	$ 208	$ 2,355
Non-cash activities			
Conversion of warrants to common stock	$	$	$ 386
Conversion of notes payable to Series B redeemable convertible preferred stock	$	$	$ 5,000
Conversion of preferred stock to common stock	$	$	$ 148,951
Accretion of redeemable convertible preferred stock	$	$	$ 802
Beneficial conversion feature related to the issuance of Series C redeemable convertible preferred stock	$	$	$ 19,424

See accompanying notes to consolidated financial statements

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Note 1. Description of Business and Significant Accounting Policies

Corporate Information, Status of Operations and Management Plans

Amicus Therapeutics, Inc. (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 to develop next-generation therapies that combine pharmacological chaperones with enzyme therapies for Pompe, Mucopolysaccharidosis Type I (MPS I) and Gaucher diseases. Current CHART programs for Pompe disease include the pharmacological chaperone AT2220 (duvoglustat HCl) co-administered with currently marketed Pompe ERTs (Myozyme®/Lumizyme®), as well as AT2220 co-formulated with a proprietary Pompe ERT. The Company s activities since inception have consisted principally of raising capital, establishing facilities, and performing research and development. Accordingly, the Company is considered to be in the development stage.

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

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. 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.

In November 2013, the Company entered into a securities purchase agreement (the 2013 SPA) with GSK and certain entities controlled by Redmile Group, LLC for the private placement of a combination of shares of the Company s common stock and warrants to purchase shares of the Company s common stock. The warrants have a term of one year and are exercisable between July 1, 2014 and June 30, 2015 at an exercise price of $2.50 per share. The aggregate offer proceeds were $15 million and GSK s resulting equity stake in the Company was 17.6% at March 31, 2014.

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

For further information, see Note 10. Collaborative Agreements.

The Company had an accumulated deficit of approximately $394.5 million at March 31, 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. In March 2010, the Company sold

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4.95 million shares of its common stock and also sold warrants to purchase 1.9 million shares of common stock in a registered direct offering to a select group of institutional investors for net proceeds of $17.1 million. In October 2010, the Company sold 6.87 million shares of its common stock as part of the collaboration agreement, dated October 28, 2010, by and between the Company and GSK (the Original Collaboration Agreement) for proceeds of $31.0 million. In March 2012, the Company sold 11.5 million shares of its common stock in a stock offering for net proceeds of $62.0 million. In July 2012, the Company sold 2.9 million shares of its common stock as part of an expanded collaboration agreement, entered into in July 2012, by and between the Company and GSK (the Expanded Collaboration Agreement) for proceeds of $18.6 million. In November 2013, the Company sold 7.5 million shares of its common stock and also sold warrants to purchase 1.6 million shares of its common stock in a private placement for proceeds of $15 million. In March 2014, the Company entered into a sales agreement with Cowen and Company, LLC (the Sales Agreement) to create an at-the-market equity program under which the Company from time to time may offer and sell shares of its common stock, par value $0.01 per share, having an aggregate offering price of up to $40 million through Cowen as sales agent. The Company believes that its existing cash and cash equivalents and short-term investments will be sufficient to cover its cash flow requirements into the second half of 2015.

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.

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.

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

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

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

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

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

In order to determine the revenue recognition for contingent milestones, the Company evaluates the contingent milestones using the criteria as provided by the Financial Accounting Standards Boards (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.

Business Combinations

The Company allocates the purchase price of acquired businesses to the tangible and intangible assets acquired and liabilities assumed based upon their estimated fair values on the acquisition date. The purchase price allocation process requires management to make significant estimates and assumptions, especially at the acquisition date with respect to intangible assets and in-process research and development (IPR&D). In connection with the purchase price allocations for acquisitions, the Company estimates the fair value of contingent acquisition consideration payments utilizing a probability-based income approach inclusive of an estimated discount rate.

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.

Intangible Assets and Goodwill

The Company records goodwill in a business combination when the total consideration exceeds the fair value of the net tangible and identifiable intangible assets acquired. Purchased in-process research and development is accounted for as an indefinite lived intangible asset until the underlying project is completed, at which point the intangible asset will be accounted for as a definite lived intangible asset, or abandoned, at which point the intangible asset will be written off or partially impaired. Goodwill and indefinite lived intangible assets are assessed annually for impairment on October 1 and whenever events or circumstances indicate that the carrying amount of an asset may not be recoverable. If it is determined that the full carrying

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amount of an asset is not recoverable, an impairment loss is recorded in the amount by which the carrying amount of the asset exceeds its fair value.

Valuation of Contingent Consideration Payable

Each period the Company reassess the fair value of the contingent acquisition consideration payable associated with certain acquisitions and records changes in the fair value as contingent consideration expense. Increases or decreases in the fair value of the contingent acquisition consideration payable can result from changes in estimated probability adjustments with respect to regulatory approval, changes in the assumed timing of when milestones are likely to be achieved and changes in assumed discount periods and rates. Significant judgment is employed in determining the appropriateness of these assumptions each period. Accordingly, future business and economic conditions, as well as changes in any of the assumptions described in the accounting for business combinations above can materially impact the amount of contingent consideration expense that the Company records in any given period.

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.

New Accounting Standards

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

position and determined that there was no impact to the Company s consolidated results of operations or financial position.

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

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Note 2. Cash, Money Market Funds and Marketable Securities

As of March 31, 2014, the Company held $27.5 million in cash and cash equivalents and $44.1 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 March 31, 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 March 31, 2014			
	Cost	Unrealized Gain	Unrealized Loss	Fair Value
Cash balances	$ 27,533	$	$	$ 27,533
Corporate debt securities	31,580	1	(8)	31,573
Commercial paper	12,189	9		12,198
Certificate of deposit	350			350
	$ 71,652	$ 10	$ (8)	$ 71,654
Included in cash and cash equivalents	$ 27,533	$	$	$ 27,533
Included in marketable securities	44,119	10	(8)	44,121
Total cash and available for sale securities	$ 71,652	$ 10	$ (8)	$ 71,654

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 three months ended March 31, 2014, unrealized holding

gain of $1 thousand, respectively, were recognized.

For the year ended December 31, 2013 and the three months ended March 31, 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 March 31, 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

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available for sale securities in unrealized loss positions was $23.6 million and $24.2 million as of December 31, 2013 and March 31, 2014, respectively.

The Company holds available-for-sale investment securities which are reported at fair value on the Company s balance sheet. Unrealized holding gains and losses are reported within accumulated other comprehensive income (AOCI) in the statements of comprehensive loss. The changes in AOCI associated with the unrealized holding gain on available-for-sale investments during the three months ended March 31, 2013 and 2014, were as follows (in thousands):

	Three Months Ended March 31,	
	2013	2014
Balance, beginning	$ 14	$ 1
Current period changes in fair value, (a)	1	1
Reclassification of earnings, (a)		
Balance, ending	$ 15	$ 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:

(In thousands, except per share amounts)	Three Months Ended March 31,	
	2013	2014
Historical		
Numerator:		
Net loss attributable to common stockholders	$ (17,458)	$ (15,943)
Denominator:		
Weighted average common shares outstanding basic and diluted	49,621,188	64,353,952

Dilutive common stock equivalents would include the dilutive effect of common stock options and warrants for common stock equivalents. Potentially dilutive common stock equivalents totaled approximately 10.5 million and 11.3 million for the three months ended March 31, 2013 and 2014, respectively. Potentially dilutive common stock equivalents were excluded from the diluted earnings per share denominator for all

periods because of their anti-dilutive effect.

Note 4. Acquisition of Callidus Biopharma, Inc.

In November 2013, the Company acquired Callidus, through the merger of the Company s subsidiary, CB Acquisition Corp. with and into Callidus (see Note 1. Description of Business). Callidus was a privately-held biologics company focused on developing best-in-class ERTs for LSDs and its lead ERT is AT-B200 for Pompe disease in late preclinical development. The acquisition of the Callidus assets and technology 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 March 31, 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

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immediately preceding the date of payment). The milestone payments not permitted to be satisfied in Common Stock (as well as any payments that the Company is permitted to, but chooses not to, satisfy in Common Stock), as a result of the terms of the Merger Agreement, the rules of The NASDAQ Global Market, or otherwise, will be paid in cash.

The fair value of the contingent acquisition consideration payments on the acquisition date was $10.6 million and was estimated by applying a probability-based income approach utilizing an appropriate discount rate. This estimation was based on significant inputs that are not observable in the market, referred to as Level 3 inputs. Key assumptions included a discount rate of 13.5% and various probability factors. As of March 31, 2014, the range of outcomes and assumptions used to develop these estimates has not changed (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 $11.1 million at March 31, 2014, resulting in an increase in the contingent consideration payable and related expense of $0.5 million.

The purchase price allocation was subject to completion of our analysis of the fair value of the assets and liabilities as of the effective date of the Merger Agreement. The final valuation was completed as of March 31, 2014. The final allocation of the purchase price at March 31, 2014 was as follows:

		(in thousands)
Upfront equity payments	$	15,000
Contingent acquisition consideration payable		10,600
Total consideration		25,600
Cash and cash equivalents		34
Property, plant and equipment		173
Intangible assets IPR&D		23,000
Total identifiable assets acquired	$	23,207
Accounts payable		(34)
Deferred tax liability		(9,186)
Total liabilities assumed		(9,220)
Net identifiable assets acquired		13,987
Goodwill		11,613
Net assets acquired	$	25,600

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

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

The Company recognized $0.5 million of acquisition-related transaction costs in selling, general and administrative expenses during 2013, which consisted primarily of legal fees and severance related to the acquisition. The expenses recognized in 2014 were de minimis and consisted primarily of accounting fees.

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

Supplemental Pro Forma Information

The following pro forma information for the three months ending March 31, 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.

		Three Months Ended March 31, 2013
Revenue	$	
Net loss	$	17,839

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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 and between annual tests if the Company becomes aware of any events occurring or changes in the circumstances that would indicate a reduction in the fair value of the goodwill below its carrying amount.

The following table represents the changes in goodwill for the three months ended March 31, 2014:

		(in thousands)
Balance at December 31, 2013	$	11,613
Change in goodwill related to the acquisition of Callidus		
Balance at March 31, 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 and in between annual tests if the Company becomes aware of any events occurring or changes in the circumstances that would indicate a reduction in the fair value of the intangible asset below its carrying amount.

The following table represents the changes in intangible assets for the three months ended March 31, 2014:

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

Note 7. Stockholders Equity

Common Stock and Warrants

As of March 31, 2014, the Company was authorized to issue 125,000,000 shares of common stock. Dividends on common stock will be paid when, and if declared by the board of directors. Each holder of common stock is entitled to vote on all matters and is entitled to one vote for each share held.

In March 2014, the Company entered into a Sales Agreement with Cowen and Company, LLC (Cowen) to create an at-the-market (ATM) equity program under which the Company from time to time may offer and sell shares of its 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 will use its commercially reasonable efforts to sell the ATM Shares from time to time, based upon the Company s instructions. The Company has provided Cowen with customary indemnification rights, and Cowen will be 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, if any, under the Agreement may be made in transactions that are 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 has no obligation to sell any of the ATM Shares, and may at any time suspend offers under the Agreement or terminate the Agreement. The Company or Cowen may suspend or terminate the offering of common stock upon notice and subject to other conditions. Since the inception of the ATM agreement, the Company has not sold any shares of common stock under the ATM agreement.

In November 2013, the Company entered into a securities purchase agreement (the 2013 SPA) with GSK and certain entities controlled by Redmile Group, LLC for the private placement of shares of the Company s common stock, par value $0.01

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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 March 31, 2014, GSK s resulting equity stake in the Company was 17.6%.

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 March 31, 2014, approximately 25 thousand shares remain issuable to former Callidus shareholders.

In July 2012, Amicus and GSK entered into a securities purchase agreement (the 2012 SPA) pursuant to which GSK purchased 2.9 million unregistered shares of Amicus common stock at a price of $6.30 per share. The total purchase price for these shares was $18.6 million. As of March 31, 2014, GSK had a 17.6% ownership position in the Company.

In March 2012, the Company sold 11.5 million shares of its common stock at a public offering price of $5.70 through a Registration Statement on Form S-3 that was declared effective by the SEC on May 27, 2009. The aggregate offering proceeds were $65.6 million.

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

In March 2010, the Company sold 4.9 million shares of its common stock and warrants to purchase 1.9 million shares of common stock in a registered direct offering to a selected group of institutional investors through a Registration Statement on Form S-3 that was declared effective by the SEC on May 27, 2009. The shares of common stock and warrants were sold in units consisting of one share of common stock and one warrant to purchase 0.375 shares of common stock at a price of $3.74 per unit. The warrants had a term of four years and were exercisable any time on or after the six month anniversary of the date they were issued, at an exercise price of $4.43 per share. The gross offering proceeds were $18.5 million. The warrants expired on March 2, 2014.

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Stock Option Plans

During the three months ended March 31, 2014, the Company recorded stock-based compensation expense of approximately $1.3 million. The stock-based compensation expense had no impact on the Company s cash flows from operations and financing activities. As of March 31, 2014, the total unrecognized compensation cost related to non-vested stock options granted was $7.7 million and is expected to be recognized over a weighted average period of 2.4 years. The following table summarizes information related to stock compensation expense recognized in the statements of operations (in thousands):

	Three Months Ended March 31,	
	2013	2014
Stock compensation expense recognized in:		
Research and development expense	$ 924	$ 550
General and administrative expense	650	710
Total stock compensation expense	$ 1,574	$ 1,260

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

	Three Months Ended March 31,	
	2013	2014
Expected stock price volatility	82.0%	81.4%
Risk free interest rate	1.2%	2.0%
Expected life of options (years)	6.25	6.25
Expected annual dividend per share	$ 0.00	$ 0.00

A summary of option activities related to the Company s stock options for the three months ended March 31, 2014 is as follows:

	Number of Shares (in thousands)	Weighted Average Exercise Price	Weighted Average Remaining Contractual Life	Aggregate Intrinsic Value (in thousands)
Balance at December 31, 2013	9,041.1	$ 5.65		
Options granted	1,344.0	$ 2.43		
Options exercised	(5.5)	$ 2.70		
Options forfeited	(719.9)	$ 6.01		
Balance at March 31, 2014	9,659.7	$ 5.17	7.2 years	$ 32.0
Vested and unvested expected to vest, March 31, 2014	9,058.6	$ 5.31	7.0 years	$ 32.0
Exercisable at March 31, 2014	5,435.0	$ 6.48	5.8 years	$ 31.8

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.

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Cash, Money Market Funds and Marketable Securities

The Company classifies its cash and money market funds within the fair value hierarchy as Level 1 as these assets are valued using quoted prices in active market for identical assets at the measurement date. The Company considers its investments in marketable securities as available-for-sale and classifies these assets within the fair value hierarchy as Level 2 primarily utilizing broker quotes in a non-active market for valuation of these securities. No changes in valuation techniques or inputs occurred during the three months ended March 31, 2014. No transfers of assets between Level 1 and Level 2 of the fair value measurement hierarchy occurred during the three months ended March 31, 2014.

Secured Debt

As disclosed in Note 8, 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, in addition to an earlier existing loan with Silicon Valley Bank. The carrying amount of the Company s borrowings approximates fair value at March 31, 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 8, 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. For the three months ended March 31, 2014, change in fair value of the contingent success fee payable was nine 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		9
Fair value balance at March 31, 2014	$	273

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 Callidus of certain clinical milestones of up to $35 million and regulatory approval milestones of up to $105 million as set forth in the Merger Agreement, provided that the aggregate consideration shall not exceed $130 million. The 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. From December 31, 2013 through March 31, 2014, the recognized amount of the contingent consideration payable increased by $0.5 million primarily as the result of the time value of money.

		(in thousands)
Fair value balance at December 31, 2013	$	10,600
Change in fair value		505
Fair value balance at March 31, 2014	$	11,105

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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 is subject to 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 March 31, 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

A summary of the fair value of the Company s assets and liabilities aggregated by the level in the fair value hierarchy within which those measurements fall as of March 31, 2014, are identified in the following table (in thousands):

	Level 1	Level 2	Total
Assets:			
Cash/ money market funds	$ 27,533	$	$ 27,533
Corporate debt securities		31,573	31,573
Commercial paper		12,198	12,198
Certificate of deposit		350	350
	$ 27,533	$ 44,121	$ 71,654

	Level 1	Level 2	Level 3	Total
Liabilities:				
Secured debt	$	$ 14,415	$	$ 14,415

Contingent success fee payable			273	273
Contingent consideration payable			11,105	11,105
	$	$ 14,415	$ 11,378	$ 25,793

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

In August 2011, the Company entered into a loan and security agreement (the 2011 Loan Agreement) with SVB, in order to finance certain capital expenditures by the Company in connection with its move in March 2012 to new office and laboratory space in Cranbury, New Jersey. The 2011 Loan Agreement provided for up to $3 million of equipment financing through January 2014. Borrowings under the 2011 Loan Agreement were collateralized by equipment purchased with the proceeds of the loan and bear interest at a variable rate of SVB prime + 2.5%. The current SVB prime rate is 4.0%. In February 2012, the Company borrowed approximately $1.0 million from the 2011 Loan Agreement which will be repaid over the following 2.5 years. The 2011 Loan Agreement contains financial covenants and the Company has at all times been in compliance with these covenants. At March 31, 2014 the total amount due under the 2011 Loan Agreement was $0.2 million.

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 March 31, 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 non-current liability on the Company s consolidated balance sheet.

For the three months ended March 31, 2014, the Company amortized the debt discount and the deferred financing using the effective interest method and recorded amortization expense of $48 thousand under operating expense on the consolidated statement of operations.

The carrying amount of the Company s borrowings approximates fair value at March 31, 2014.

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. Potential payments included up to (i) $13.5 million related to the attainment of certain clinical development objectives and the acceptance of regulatory filings in select worldwide markets, (ii) $80 million related to market approvals for migalastat in selected territories throughout the world, and (iii) $80 million associated with the achievement of certain sales thresholds. GSK and the Company were jointly funding development costs in accordance with an agreed upon development plan. Additionally, GSK purchased approximately 6.9 million shares of the Company s common stock at $4.56 per share, a 30% premium on the average price per share of the Company s stock over a 60 day period preceding the closing date of the transaction. The total value of this equity investment to the Company was approximately $31 million.

In July 2012, the Company entered into the Expanded Collaboration Agreement with GSK pursuant to which the Company and GSK continue to develop and commercialize migalastat, 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.

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Under the terms of the Expanded Collaboration Agreement, the Company and GSK were to co-develop all formulations of migalastat HCl for Fabry disease, including the development of migalastat 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 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 Agreement entered into between Amicus and GSK in July 2012. Under the terms of the Revised Agreement, there was no upfront payment from Amicus to GSK. For the next-generation Fabry ERT (migalastat HCl co-formulated with ERT), GSK is eligible to receive single-digit royalties on net sales in eight major markets outside the U.S. For migalastat HCl monotherapy, GSK is eligible to receive post-approval and sales-based milestones up to $40 million, as well as tiered royalties in the mid-teens in eight major markets outside the U.S.

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

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

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

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

- a payment for an identifiable benefit, and

- the identifiable benefit is separable from the existing relationship between the Company and GSK, and

- the identifiable benefit can be obtained from a party other than GSK, and

- the Company can reasonably estimate the fair value of the identifiable benefit,

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

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

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

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

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

As a result, all revenue recognition was suspended until the total arrangement consideration becomes fixed and determinable. In addition, future milestone payments made by the Company will be applied against the balance of this deferred reimbursements account. In the third quarter of 2013, the Company paid GSK a pass-through milestone payment of $0.8 million in connection with the development of the Co-formulated product. This payment is reflected as a reduction of the deferred reimbursements in the consolidated balance sheet as of December 31, 2013.

Revenue recognition for research expense reimbursements, the original upfront license fee, and the equity premiums will resume once the total arrangement consideration becomes fixed and determinable which will occur when the balance of the deferred reimbursements account is sufficient to cover all the remaining contingent milestone payments.

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

Biogen

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

eligible to receive development and regulatory milestones, as well as modest royalties in global net sales.

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

- Research and development expenses related to a collaboration agreement will be recorded on a gross basis in the income statement and not presented net of any reimbursement received from a collaboration agreement; and

- The reimbursement of research and development expenses from a collaborator will be recognized in the income statement as Research Revenue for the period in which the research activity occurred.

As of March 31, 2014, the Company recognized $0.5 million in Research Revenue for work performed under the cost sharing arrangement of the Biogen Agreement.

The Company evaluated the contingent milestones included in the Biogen Agreement at the inception of the Biogen Agreement and determined that the contingent milestones are substantive milestones and will be recognized as revenue in the period that the milestone is achieved. The Company determined that the research based milestones are commensurate with the enhanced value of each delivered item as a result of the Company s specific performance to achieve the milestones. The research

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based milestones would relate to past performances when achieved and are reasonable relative to the other payment terms within the Biogen Agreement, including the cost sharing arrangement.

Note 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 $1.9 million during the fourth quarter of 2013 which included $1.2 million for employment termination costs payable and a facilities consolidation charge of $0.8 million consisting of lease payments of $0.7 million related to the net present value of the net future minimum lease payments at the cease-use date and the write-down of the net book value of the fixed assets in the vacated building of $0.1 million. At March 31, 2014, $0.5 million of the restructuring charges related to employment termination costs were unpaid and classified under accrued expenses on the balance sheet.

The following table summarizes the restructuring charges and utilization for the three months ended March 31, 2014 (in thousands):

	Balance as of December 31, 2013	Charges	Cash Payments	Adjustments	Balance as of March 31, 2014
Employment termination costs	$ 1,139	$	$ (673)	$	$ 466
Facilities consolidation	703		(130)	(8)	565
	$ 1,842	$	$ (803)	$ (8)	$ 1,031

Note 12. Subsequent Events

The Company evaluated events that occurred subsequent to March 31, 2014 and there were no material recognized or non-recognized subsequent events during this period.

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

Overview

We are a biopharmaceutical company 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 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 study 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 as a post-hoc analysis from baseline to month 6, which showed statistically significant reductions 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.

We also analyzed the mean change in GL-3 from baseline to month 6 in a subgroup of patients with amenable mutations in a GLP-validated human embryonic kidney (HEK) cell-based *in vitro* assay (GLP HEK amenable) assay. 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, 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.

All subjects enrolled in Study 011 had amenable mutations in the clinical trail HEK assay available at study initiation (clinical trail assay). Following the completion of enrollment, the GLP HEK assay was developed with a third party to measure the criteria for amenability with more quality control and rigor. However, 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 patients in Study 011.

In April 2014, we released top-line 12- and 24-month results data 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), which met the 12-month pre-specified primary analysis, and a reduction of disease substrate in another important biomarker of disease, plasma lyso-Gb3.

- Subjects who remained on migalastat demonstrated a durable reduction in kidney interstitial capillary GL-3, as well as a significant and durable reduction in lyso-Gb3.

- Kidney function, as measured by estimated glomerular filtration rate (eGFR) and iohexol (measured) GFR, or mGFR, remained stable following 18-24 months of treatment with migalastat.

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- From a safety perspective, migalastat was generally safe and well-tolerated.

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 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. The primary outcome measure is renal function assessed by GFR at 18 months, evaluated in the migalastat and ERT groups using descriptive statistics. This study achieved full enrollment in December 2012 and top-line results are expected in the third quarter of 2014.

Pending positive data from Study 012, we plan to meet with U.S. and EU regulatory authorities to discuss data from both Phase 3 studies as well as the registration pathway for migalastat monotherapy.

Migalastat HCl Combination Programs for Fabry Disease

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. 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 initiated a Phase 1 study in healthy volunteers to investigate the PK of IV migalastat to identify optimal doses for a Phase 1/2 clinical study of migalastat co-formulated with ERT in Fabry patients. We expect to initate this Phase 1/2 study in the second half of 2014. We are currently evaluating its long-term strategy for supplying late-stage clinical and commercial ERT, which may include developing or in-licensing a recombinant alpha-Gal A enzyme comparable to JR-051.

Next-Generation ERT for Pompe Disease

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

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

We completed a Phase 2 safety and pharmacokinetics study (Study 010) that investigated single ascending oral doses of AT2220 (50 mg, 100 mg, 250 mg, and 600 mg) co-administered with Myozyme® or Lumizyme® (alglucosidase alfa or recombinant human GAA enzyme, rhGAA),

in patients with Pompe disease. Each patient received one infusion of ERT alone, and then a single dose of AT2220 just prior to the next ERT infusion. Results from this study showed an increase in GAA enzyme activity in plasma and muscle compared to ERT alone.

In preclinical studies, AT-B200 was shown to have superior uptake and activity in disease-relevant tissues that correlated with clearance of accumulated glycogen substrate when compared to current standard of care. AT-B200 may be further improved through the application of the Company s proprietary conjugation technology to attach vIGF2 (a variant of the insulin like growth factor 2) to further enhance drug targeting. Preclinical results have shown that AT-B200 and AT-B200 conjugated with vIGF-2 were better than Lumizyme for clearing glycogen in skeletal muscles in Gaa knock-out mice.

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

Collaboration with GSK

In November 2013, we entered into the Revised Agreement with 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. The Revised Agreement amends and replaces in its entirety the Expanded Agreement entered into between Amicus and GSK in July 2012. Under the terms of the Revised Agreement, there is no upfront payment from Amicus to GSK. For the next-generation Fabry ERT (migalastat 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 Revised Agreement, Amicus received a settlement fee 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.

In November 2013, we entered into the 2013 SPA with GSK and certain entities controlled by Redmile Group, LLC for the private placement of a) shares of our common stock and b) a combination of shares of our common stock and warrants to purchase shares of our common stock. The warrants have a term of one year and are exercisable between July 1, 2014 and June

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30, 2015 at an exercise price of $2.50 per share. The aggregate offer proceeds were $15 million and GSK s resulting equity stake in the Company was 17.6% at March 31, 2014.

Collaboration with Biogen

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

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 chooses not to, satisfy in common stock), as a result of the terms of the merger agreement, will be paid in cash.

Other Potential Alliances and Collaborations

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

Financial Operations Overview

We have generated significant losses to date and expect to continue to generate losses as we continue the clinical and preclinical development of our drug candidates. These activities are budgeted to expand over time and will require further resources if we are to be successful. From our inception in February 2002 through March 31, 2014, we have accumulated a deficit of $394.5 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 a collaboration with Biogen to discover, develop and commercialize novel small molecules for the treatment of Parkinson s disease. For the three months ended March 31, 2014, we recognized $0.5 million as Research Revenue for reimbursed research and development costs.

GSK

Under the Original License and Collaboration Agreement, GSK paid us an initial, non-refundable license fee of $30 million and a premium of $3.2 million related to GSK s purchase of an equity investment in Amicus which was being recognized as Collaboration and Milestone Revenue on a straight-line basis over the development period. In addition, in June 2012, we recognized a $3.5 million payment for a clinical development milestone as Collaboration and Milestone Revenue. For the year ended December 31, 2012 we recognized $6.8 million as Collaboration and Milestone Revenue.

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The reimbursements for research and development costs under the Original License and Collaboration Agreement that met the criteria for revenue recognition were recognized as Research Revenue. For the year ended December 31, 2012, we recognized $11.6 million as Research Revenue.

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

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

Research and Development Expenses

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

- internal costs associated with our research and clinical development activities;
- payments we make to third party contract research organizations, contract manufacturers, investigative sites, and consultants;
- technology license costs;
- manufacturing development costs;
- personnel related expenses, including salaries, benefits, travel, and related costs for the personnel involved in drug discovery and development;
- activities relating to regulatory filings and the advancement of our product candidates through preclinical studies and clinical trials; and

- facilities and other allocated expenses, which include direct and allocated expenses for rent, facility maintenance, as well as laboratory and other supplies.

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

We expense research and development costs as incurred, including payments made to date under our license agreements. We believe that significant investment in product development is a competitive necessity and plan to continue these investments in order to realize the potential of our product candidates. From our inception in February 2002 through March 31, 2014, we have incurred research and development expense in the aggregate of $367.8 million.

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

Projects	Three Months Ended March 31, 2013	2014	Period from February 4, 2002 (inception) to March 31, 2014
Third party direct project expenses			
Monotherapy Studies			
Migalastat HCl (Fabry Disease Phase 3)	$ 2,342	$ 2,914	$ 91,944
Afegostat tartrate (Gaucher Disease Phase 2*)	30	6	26,387
AT2220 (Pompe Disease Phase 2)			13,252
Combination Studies			
Migalastat HCl Co-Administration (Fabry Disease Phase 2)	445	6	3,970
Migalastat HCl Co-Formulation (Fabry Disease Preclinical)	76	167	710
Afegostat tartrate Co-Administration (Gaucher Disease Preclinical)	21		55
AT2220 Co-Administration (Pompe Disease Phase 2)	1,097	10	7,541
AT2220 Co- Formulation (Pompe Disease Preclinical)	109	966	1,311
Neurodegenerative Diseases (Preclinical)	14	49	9,219
Total third party direct project expenses	4,134	4,118	154,389
Other project costs (1)			
Personnel costs	5,888	4,294	138,888
Other costs (2)	1,967	1,580	74,552
Total other project costs	7,855	5,874	213,440
Total research and development costs	$ 11,989	$ 9,992	$ 367,829

(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.

The successful development of our product candidates is highly uncertain. At this time, we cannot reasonably estimate or know the nature, timing and costs of the efforts that will be necessary to complete the remainder of the development of our product candidates. As a result, we are not able to reasonably estimate the period, if any, in which material net cash inflows may commence from our product candidates, including migalastat or any of our other preclinical product candidates. This uncertainty is due to the numerous risks and uncertainties associated with the conduct, duration and cost of clinical trials, which vary significantly over the life of a project as a result of evolving events during clinical development, including:

- the number of clinical sites included in the trials;
- the length of time required to enroll suitable patients;
- the number of patients that ultimately participate in the trials;

- the results of our clinical trials; and

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

Our expenditures are subject to additional uncertainties, including the terms and timing of regulatory approvals, and the expense of filing, prosecuting, defending and enforcing any patent claims or other intellectual property rights. We may obtain unexpected results from our clinical trials. We may elect to discontinue, delay or modify clinical trials of some product candidates or focus on others. A change in the outcome of any of the foregoing variables with respect to the development of a product candidate could mean a significant change in the costs and timing associated with the development, regulatory approval and commercialization of that product candidate. For example, if the FDA or other regulatory authorities were to require us to

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conduct clinical trials beyond those which we currently anticipate, or if we experience significant delays in enrollment in any of our clinical trials, we could be required to expend significant additional financial resources and time on the completion of clinical development. Drug development may take several years and millions of dollars in development costs.

General and Administrative Expense

General and administrative expense consists primarily of salaries and other related costs, including stock-based compensation expense, for persons serving in our executive, finance, accounting, legal, information technology and human resource functions. Other general and administrative expense includes facility-related costs not otherwise included in research and development expense, promotional expenses, costs associated with industry and trade shows, and professional fees for legal services, including patent-related expense and accounting services. From our inception in February 2002 through March 31, 2014, we spent $156.7 million on general and administrative expense.

Interest Income and Interest Expense

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

Critical Accounting Policies and Significant Judgments and Estimates

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

While there were no significant changes during the quarter ended March 31, 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, 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.

Business Combinations

We allocate the purchase price of acquired businesses to the tangible and intangible assets acquired and liabilities assumed based upon their estimated fair values on the acquisition date. The purchase price allocation process requires management to make significant estimates and assumptions, especially at the acquisition date with respect to intangible assets and in-process research and development (IPR&D). In connection with the purchase price allocations for acquisitions, we estimate the fair value of contingent acquisition consideration payments utilizing a probability-based income approach inclusive of an estimated discount rate.

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

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

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

Intangible Assets and Goodwill

We record goodwill in a business combination when the total consideration exceeds the fair value of the net tangible and identifiable intangible assets acquired. Purchased in-process research and development is accounted for as an indefinite lived intangible asset until the underlying project is completed, at which point the intangible asset will be accounted for as a definite lived intangible asset, or abandoned, at which point the intangible asset will be written off or partially impaired. Goodwill and indefinite lived intangible assets are assessed annually for impairment on October 1 and whenever events or circumstances indicate that the carrying amount of an asset may not be recoverable. If it is determined that the full carrying amount of an asset is not recoverable, an impairment loss is recorded in the amount by which the carrying amount of the asset exceeds its fair value.

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Valuation of Contingent Consideration Payable

Each period we reassess the fair value of the contingent acquisition consideration payable associated with certain acquisitions and record changes in the fair value as contingent consideration expense. Increases or decreases in the fair value of the contingent acquisition consideration payable can result from changes in estimated probability adjustments with respect to regulatory approval, changes in the assumed timing of when milestones are likely to be achieved and changes in assumed discount periods and rates. Significant judgment is employed in determining the appropriateness of these assumptions each period. Accordingly, future business and economic conditions, as well as changes in any of the assumptions described in the accounting for business combinations above can materially impact the amount of contingent consideration expense that we record in any given period.

Accrued Expenses

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

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

Stock-Based Compensation

In accordance with the applicable guidance, we measure stock-based compensation at a fair value which is determined by measuring the cost of employee services received in exchange for an award of equity instruments based upon the grant date fair value of the award. We chose the

straight-line attribution method for allocating compensation costs and recognized the fair value of each stock option on a straight-line basis over the vesting period of the related awards.

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

	Three Months Ended March 31,	
	2013	2014
Stock compensation expense recognized in:		
Research and development expense	$ 924	$ 550
General and administrative expense	650	710
Total stock compensation expense	$ 1,574	$ 1,260

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

	Three Months Ended March 31,	
	2013	2014
Expected stock price volatility	82.0%	81.4%
Risk free interest rate	1.2%	2.0%
Expected life of options (years)	6.25	6.25
Expected annual dividend per share	$ 0.00	$ 0.00

Warrants

The warrants issued in connection with the March 2010 registered direct offering 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 was being recognized in the consolidated statement of operations. The warrants expired on March 2, 2014 and hence the warrant liability is no longer recognized on the Balance Sheet as of March 31, 2014.

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:

	Three Months Ended March 31,	
(In thousands, except per share amounts)	**2013**	**2014**
Historical		
Numerator:		
Net loss attributable to common stockholders	$ (17,458)	$ (15,943)
Denominator:		
Weighted average common shares outstanding - basic and diluted	49,621,188	64,353,952

Dilutive common stock equivalents would include the dilutive effect of common stock options and warrants for common stock equivalents. Potentially dilutive common stock equivalents totaled approximately 10.5 million and 11.3 million for the three months ended March 31, 2013 and 2014, respectively. Potentially dilutive common stock equivalents were excluded from the diluted earnings per share denominator for all

periods because of their anti-dilutive effect.