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

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**FORM 10-Q**

(Mark One)

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

For the quarterly period ended June 30, 2011

OR

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

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

Commission file number 001-33497

**Amicus Therapeutics, Inc.**

(Exact Name of Registrant as Specified in Its Charter)

Delaware

(State or Other Jurisdiction of  
Incorporation or Organization)

71-0869350

(I.R.S. Employer  
Identification Number)

6 Cedar Brook Drive, Cranbury, NJ 08512

(Address of Principal Executive Offices and Zip Code)

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

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

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

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

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

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

The number of shares outstanding of the registrant's common stock, \$.01 par value per share, as of July 28, 2011 was 34,600,431 shares.

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AMICUS THERAPEUTICS, INC

Form 10-Q for the Quarterly Period Ended June 30, 2011

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We have filed applications to register certain trademarks in the United States and abroad, including AMICUS<sup>TM</sup>, AMICUS THERAPEUTIC<sup>TM</sup> (and design), AMIGAL<sup>TM</sup> and PLICERA<sup>TM</sup>.

## SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

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

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

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

We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make. We have included important factors in the cautionary statements included in Part I Item 1A — “Risk Factors” of the Annual Report on Form 10-K for the year ended December 31, 2010 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.

**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)

	<u>December 31,</u> <u>2010</u>	<u>June 30,</u> <u>2011</u>
<b>Assets:</b>		
Current assets:		
Cash and cash equivalents	\$ 29,572	\$ 25,789
Investments in marketable securities	77,873	57,257
Receivable due from GSK	—	1,977
Prepaid expenses and other current assets	2,236	3,076
<b>Total current assets</b>	<u>109,681</u>	<u>88,099</u>
Property and equipment, accumulated depreciation and amortization of \$8,095 and \$8,959 at December 31, 2010 and June 30, 2011, respectively	2,604	1,922
Other non-current assets	267	267
<b>Total Assets</b>	<u>\$ 112,552</u>	<u>\$ 90,288</u>
<b>Liabilities and Stockholders' Equity</b>		
Current liabilities:		
Accounts payable and accrued expenses	\$ 8,290	\$ 7,019
Current portion of capital lease obligations	40	—
Current portion of deferred revenue	6,640	8,606
Current portion of secured loan	1,253	1,253
<b>Total current liabilities</b>	<u>16,223</u>	<u>16,878</u>
Deferred revenue, less current portion	25,639	22,319
Warrant liability	4,712	6,066
Secured loan, less current portion	1,044	418
Commitments and contingencies		
Stockholders' equity:		
Common stock, \$.01 par value, 50,000,000 shares authorized, 34,508,932 shares issued and outstanding at December 31, 2010, 50,000,000 shares authorized, 34,596,809 shares issued and outstanding at June 30, 2011	406	406
Additional paid-in capital	290,248	295,898
Accumulated other comprehensive loss	(28)	(14)
Deficit accumulated during the development stage	(225,692)	(251,683)
<b>Total stockholders' equity</b>	<u>64,934</u>	<u>44,607</u>
<b>Total Liabilities and Stockholders' Equity</b>	<u>\$ 112,552</u>	<u>\$ 90,288</u>

*See accompanying notes to consolidated financial statements*

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

	Three Months Ended June 30,		Six Months Ended June 30,		Period from February 4, 2002 (inception) to June 30, 2011
	2010	2011	2010	2011	2011
<b>Revenue:</b>					
Research revenue	\$ —	\$ 2,380	\$ —	\$ 6,686	\$ 37,794
Collaboration revenue	—	1,660	—	3,320	54,242
<b>Total revenue</b>	<b>\$ —</b>	<b>\$ 4,040</b>	<b>\$ —</b>	<b>\$ 10,006</b>	<b>\$ 92,036</b>
<b>Operating Expenses:</b>					
Research and development	\$ 8,137	\$ 11,618	\$ 17,026	\$ 22,743	\$ 237,507
General and administrative	4,020	6,720	7,945	11,122	104,491
Restructuring charges	—	—	—	—	1,522
Impairment of leasehold improvements	—	—	—	—	1,030
Depreciation and amortization	529	426	1,066	864	9,342
In-process research and development	—	—	—	—	418
<b>Total operating expenses</b>	<b>12,686</b>	<b>18,764</b>	<b>26,037</b>	<b>34,729</b>	<b>354,310</b>
<b>Loss from operations</b>	<b>(12,686)</b>	<b>(14,724)</b>	<b>(26,037)</b>	<b>(24,723)</b>	<b>(262,274)</b>
<b>Other income (expenses):</b>					
Interest income	35	46	88	105	14,018
Interest expense	(55)	(41)	(137)	(89)	(2,274)
Change in fair value of warrant liability	1,391	2,078	1,595	(1,354)	(3,218)
Other income	—	—	—	70	231
<b>Loss before tax benefit</b>	<b>(11,315)</b>	<b>(12,641)</b>	<b>(24,491)</b>	<b>(25,991)</b>	<b>(253,517)</b>
Benefit from income taxes	—	—	—	—	1,834
<b>Net loss</b>	<b>(11,315)</b>	<b>(12,641)</b>	<b>(24,491)</b>	<b>(25,991)</b>	<b>(251,683)</b>
Deemed dividend	—	—	—	—	(19,424)
Preferred stock accretion	—	—	—	—	(802)
<b>Net loss attributable to common stockholders</b>	<b>\$ (11,315)</b>	<b>\$ (12,641)</b>	<b>\$ (24,491)</b>	<b>\$ (25,991)</b>	<b>\$ (271,909)</b>
<b>Net loss attributable to common stockholders per common shares — basic and diluted</b>	<b>\$ (0.41)</b>	<b>\$ (0.37)</b>	<b>\$ (0.94)</b>	<b>\$ (0.75)</b>	
<b>Weighted-average common shares outstanding — basic and diluted</b>	<b>27,623,297</b>	<b>34,530,693</b>	<b>25,956,366</b>	<b>34,514,947</b>	

*See accompanying notes to consolidated financial statements*

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

	<b>Six Months Ended June 30,</b>		<b>Period from February 4, 2002 (inception) to June 30, 2011</b>
	<b>2010</b>	<b>2011</b>	
<b>Operating activities</b>			
Net loss	\$ (24,491)	\$ (25,991)	\$ (251,683)
Adjustments to reconcile net loss to net cash used in operating activities:			
Non-cash interest expense	—	—	525
Depreciation and amortization	1,066	864	9,342
Amortization of non-cash compensation	—	—	522
Stock-based compensation — employees	3,219	5,302	32,361
Stock-based compensation — non-employees	—	—	853
Stock-based license payments	—	—	1,220
Change in fair value of warrant liability	(1,595)	1,354	3,218
Loss on disposal of asset	—	—	360
Impairment of leasehold improvements	—	—	1,030
Non-cash charge for in-process research and development	—	—	418
Debt instrument convertible beneficial conversion feature	—	—	135
Changes in operating assets and liabilities:			
Prepaid expenses and other current assets	399	(2,817)	(5,053)
Other non-current assets	—	—	(288)
Accounts payable and accrued expenses	(3,883)	(1,271)	7,019
Deferred revenue	—	(1,354)	30,925
Net cash used in operating activities	(25,285)	(23,913)	(169,096)
<b>Investing activities</b>			
Sale and redemption of marketable securities	52,525	57,224	630,838
Purchases of marketable securities	(38,125)	(36,594)	(688,229)
Purchases of property and equipment	(135)	(182)	(12,651)
Net cash provided by/(used in) investing activities	14,265	20,448	(70,042)
<b>Financing activities</b>			
Proceeds from the issuance of preferred stock, net of issuance costs	—	—	143,022
Proceeds from the issuance of common stock and warrants, net of issuance costs	17,131	—	113,307
Proceeds from the issuance of convertible notes	—	—	5,000
Payments of capital lease obligations	(211)	(40)	(5,587)
Payments of secured loan agreement	(626)	(626)	(2,087)
Proceeds from exercise of stock options	9	348	1,639
Proceeds from exercise of warrants (common and preferred)	—	—	264
Proceeds from capital asset financing arrangement	—	—	5,611
Proceeds from secured loan agreement	—	—	3,758
Net cash provided by/(used in) financing activities	16,303	(318)	264,927
Net increase/(decrease) in cash and cash equivalents	5,283	(3,783)	25,789
Cash and cash equivalents at beginning of period	19,339	29,572	—
Cash and cash equivalents at end of period	<u>\$ 24,622</u>	<u>\$ 25,789</u>	<u>\$ 25,789</u>

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

	<b>Six Months Ended June 30,</b>		<b>Period from February 4, 2002 (inception) to June 30, 2011</b>
	<b>2010</b>	<b>2011</b>	
<b>Supplemental disclosures of cash flow information</b>			
Cash paid during the period for interest	\$ 157	\$ 89	\$ 1,973
<b>Non-cash activities</b>			
Conversion of notes payable to preferred stock	\$ —	\$ —	\$ 5,000
Conversion of preferred stock to common stock	\$ —	\$ —	\$ 148,951
Accretion of redeemable convertible preferred stock	\$ —	\$ —	\$ 802
Beneficial conversion feature related to the issuance of Series C redeemable convertible preferred stock	\$ —	\$ —	\$ 19,424

*See accompanying notes to consolidated financial statements*



**Note 1. Description of Business and Significant Accounting Policies**

***Corporate Information, Status of Operations and Management Plans***

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

In October 2010, the Company entered into the License and Collaboration Agreement with Glaxo Group Limited, an affiliate of GlaxoSmithKline PLC (GSK), to develop and commercialize Amigal. Under the terms of the License and Collaboration Agreement, GSK received an exclusive worldwide license to develop, manufacture and commercialize Amigal. In consideration of the license grant, the Company received an upfront, license payment of \$30 million and a premium related to the equity portion of the transaction of \$3.2 million from GSK and is eligible to receive further payments of approximately \$170 million upon the successful achievement of development and commercialization milestones, as well as tiered double-digit royalties on global sales of Amigal. GSK and the Company will jointly fund development costs in accordance with an agreed upon development plan. For further information, see "— Note 8. Collaborative Agreements."

The Company had an accumulated deficit of approximately \$251.7 million at June 30, 2011 and anticipates incurring losses through the year 2011 and beyond. The Company has not yet generated commercial sales revenue and has been able to fund its operating losses to date through the sale of its redeemable convertible preferred stock, issuance of convertible notes, net proceeds from our initial public offering (IPO) and subsequent stock offerings, payments from partners during the terms of the collaboration agreements and other financing arrangements. The Company believes that its existing cash and cash equivalents and short-term investments will be sufficient to cover its cash flow requirements for 2011.

***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, 2010. 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, 2010.

### ***Revenue Recognition***

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

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

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

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

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

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

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

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

### ***New Accounting Standards***

In June 2011, the FASB issued guidance on the reporting and presentation of comprehensive income. This guidance eliminates the option to present the components of other comprehensive income as part of the statement of changes in stockholders' equity and requires an entity to present items of net income, other comprehensive income and total comprehensive income either in a single continuous statement of comprehensive income or in two separate but consecutive statements. The guidance also requires companies to display reclassification adjustments for each component of other comprehensive income in both net income and other comprehensive income. The amendments in this guidance do 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 new guidance is effective for fiscal years, and interim periods within those years, beginning after December 15, 2011 and must be applied retrospectively. The Company will be required to adopt this guidance no later than the quarter beginning January 1, 2012. As the new guidance requires additional presentation only, there will be no impact to the Company's consolidated results of operations or financial position.

In May 2011, the FASB amended the FASB Accounting Standards Codification to converge the fair value measurement guidance in U.S. GAAP and International Financial Reporting Standards. Some of the amendments clarify the application of existing fair value measurement requirements, while other amendments change particular principles in fair value measurement guidance. In addition, the amendments require additional fair value disclosures. The amendments are effective for fiscal years beginning after December 15, 2011 and should be applied prospectively. The Company is currently evaluating the impact, if any, that the provisions of the amendments will have on its consolidated results of operations or financial position.

In April 2010, the FASB issued guidance on revenue recognition related to the milestone method of revenue recognition. This guidance provides criteria on defining a substantive milestone and determining when it may be appropriate to apply the milestone method of revenue recognition for research or development transactions. Early adoption is permitted retrospectively from the beginning of an entity's fiscal year. The Company early adopted this guidance on the milestone method of revenue recognition and retrospectively applied this guidance to the beginning of 2010. This method was first applied in conjunction with the License and Collaboration Agreement with GSK during the fourth quarter of 2010; there have been no milestones recognized in the year of adoption. This guidance did not have a material impact on the timing or pattern of revenue recognition relative to the agreement nor is expected to in future periods.

***Subsequent Events***

***Equipment Financing Agreement***

In order to finance certain capital expenditures anticipated to be made by the Company in connection with its planned move in March 2012 following the expiration of its current leases for office and laboratory space in Cranbury, New Jersey the Company entered into a loan and security agreement with Silicon Valley Bank (SVB) on August 4, 2011 that provides for up to \$3 million of equipment financing through January 2014. Borrowings under the loan agreement are collateralized by equipment purchased with the proceeds of the loan and bear interest at a variable rate of SVB prime + 2.5%. The current SVB prime rate is 4.0%. The loan agreement contains customary terms and conditions, including a financial covenant whereby the Company must maintain a minimum amount of liquidity measured at the end of each month greater than \$20 million of unrestricted cash, cash equivalents and marketable securities plus all outstanding borrowings.

***Departure of Matthew R. Patterson***

On August 8, 2011, the Company announced that its President, Matthew R. Patterson, will leave Amicus at the end of August 2011 to pursue other opportunities. In addition to his duties as President, Mr. Patterson served as Acting Chief Executive Officer of the Company from April 18, 2011 through August 4, 2011 at which time John Crowley returned as Chief Executive Officer.

**Note 2. Cash, Cash Equivalents and Short Term Investments**

As of June 30, 2011, the Company held \$25.8 million in cash and cash equivalents and \$57.3 million of short term investment securities. The short term investment securities are classified as available-for-sale and as such, are reported at fair value on the Company's balance sheet. Unrealized holding gains and losses are reported within accumulated other comprehensive income/(loss) as a separate component of stockholders' equity. If a decline in the fair value of a marketable security below the Company's cost basis is determined to be other than temporary, such marketable security is written down to its estimated fair value as a new cost basis and the amount of the write-down is included in earnings as an impairment charge. To date, only temporary impairment adjustments have been recorded.

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

Cash and available for sale securities consisted of the following as of December 31, 2010 and June 30, 2011 (in thousands):

	<b>As of December 31, 2010</b>			
	<b>Cost</b>	<b>Unrealized Gain</b>	<b>Unrealized Loss</b>	<b>Fair Value</b>
Cash balances	\$ 29,572	\$ —	\$ —	\$ 29,572
U.S. government agency securities	12,000	—	(9)	11,991
Corporate debt securities	42,075	2	(33)	42,044
Commercial paper	23,476	12	—	23,488
Certificate of deposit	350	—	—	350
	<u>\$ 107,473</u>	<u>\$ 14</u>	<u>\$ (42)</u>	<u>\$ 107,445</u>
Included in cash and cash equivalents	\$ 29,572	\$ —	\$ —	\$ 29,572
Included in marketable securities	77,901	14	(42)	77,873
<b>Total cash and available for sale securities</b>	<u>\$ 107,473</u>	<u>\$ 14</u>	<u>\$ (42)</u>	<u>\$ 107,445</u>
	<b>As of June 30, 2011</b>			
	<b>Cost</b>	<b>Unrealized Gain</b>	<b>Unrealized Loss</b>	<b>Fair Value</b>
Cash balances	\$ 25,789	\$ —	\$ —	\$ 25,789
U.S. government agency securities	2,000	—	(3)	1,997
Corporate debt securities	44,933	2	(24)	44,911
Commercial paper	9,988	11	—	9,999
Certificate of deposit	350	—	—	350
	<u>\$ 83,060</u>	<u>\$ 13</u>	<u>\$ (27)</u>	<u>\$ 83,046</u>
Included in cash and cash equivalents	\$ 25,789	\$ —	\$ —	\$ 25,789
Included in marketable securities	57,271	13	(27)	57,257
<b>Total cash and available for sale securities</b>	<u>\$ 83,060</u>	<u>\$ 13</u>	<u>\$ (27)</u>	<u>\$ 83,046</u>

Unrealized gains and losses are reported as a component of accumulated other comprehensive income/(loss) in stockholders' equity. For the year ended December 31, 2010, unrealized holding gains included in accumulated other comprehensive income was \$0.1 million. For the six months ended June 30, 2011, unrealized holding gains included in accumulated other comprehensive income were de minimis.

For the year ended December 31, 2010 and the six months ended June 30, 2011, 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, 2010 and June 30, 2011 reflect temporary impairments that have not been recognized and have been in a loss position for less than twelve months. The fair value of these available for sale securities in unrealized loss positions was \$46.1 million and \$29.8 million as of December 31, 2010 and June 30, 2011, respectively.

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

The Company calculates net loss per share as a measurement of the Company's performance while giving effect to all dilutive potential common shares that were outstanding during the reporting period. The Company has a net loss for all periods presented; accordingly, the inclusion of common stock options 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 June 30,		Six Months Ended June 30,	
	2010	2011	2010	2011
<b>Statement of Operations</b>				
Net loss attributable to common stockholders	\$ (11,315)	\$ (12,641)	\$ (24,491)	\$ (25,991)
Net loss attributable to common stockholders per common share — basic and diluted	\$ (0.41)	\$ (0.37)	\$ (0.94)	\$ (0.75)

Dilutive common stock equivalents would include the dilutive effect of common stock options and warrants for common stock equivalents. Potentially dilutive common stock equivalents totaled approximately 7.1 million and 7.6 million for the six months ended June 30, 2010 and 2011, 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. Comprehensive Loss**

The components of comprehensive loss are as follows (in thousands):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2010	2011	2010	2011
Net loss	\$ (11,315)	\$ (12,641)	\$ (24,491)	\$ (25,991)
Change in unrealized net gain/(loss) on marketable securities	4	(15)	(37)	14
Comprehensive loss	<u>\$ (11,311)</u>	<u>\$ (12,656)</u>	<u>\$ (24,528)</u>	<u>\$ (25,977)</u>

Accumulated other comprehensive loss equals the unrealized net gains and losses on marketable securities which are the only components of other comprehensive loss included in the Company's financial statements.

**Note 5. Stockholders' Equity****Common Stock and Warrants**

As of June 30, 2011, the Company was authorized to issue 50,000,000 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 October 2010, in connection with the License and Collaboration Agreement, GSK purchased approximately 6.9 million shares of the Company's common stock at \$4.56 per share. The total value of this equity investment was approximately \$31 million and represents a 19.9% ownership position in the Company.

In March 2010, the Company sold 4.9 million shares of its common stock and warrants to purchase 1.9 million shares of common stock in a registered direct offering to a selected group of institutional investors through a Registration Statement on Form S-3 that was declared effective by the SEC on May 27, 2009. The shares of common stock and warrants were sold in units consisting of one share of common stock and one warrant to purchase 0.375 shares of common stock at a price of \$3.74 per unit. The warrants have a term of four years and are exercisable any time on or after the six month anniversary of the date they were issued, at an exercise price of \$4.43 per share. The aggregate offering proceeds were \$18.5 million. The Company intends to use the net proceeds from the sale of the common stock and warrants for general corporate purposes and to further advance the development of the Company's lead product candidate, Amigal, and the completion of certain activities required for the submission of a license application globally.

**Stock Option Plans**

During the three and six months ended June 30, 2011, the Company recorded compensation expense of approximately \$3.8 million and \$5.3 million, respectively. The stock-based compensation expense had no impact on the Company's cash flows from operations and financing activities. As of June 30, 2011, the total unrecognized compensation cost related to non-vested stock options granted was \$9.1 million and is expected to be recognized over a weighted average period of 2.6 years.

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

	<b>Three Months Ended June 30, 2010</b>	<b>Six Months Ended June 30, 2010</b>	<b>Three Months Ended June 30, 2011</b>	<b>Six Months Ended June 30, 2011</b>
Expected stock price volatility	80.0%	80.5%	78.4%	78.8%
Risk free interest rate	2.4%	2.5%	2.1%	2.2%
Expected life of options (years)	6.25	6.25	6.25	6.25
Expected annual dividend per share	\$ 0.00	\$ 0.00	\$ 0.00	\$ 0.00

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A summary of option activities related to the Company's stock options for the six months ended June 30, 2011 is as follows:

	Number of Shares <u>(in thousands)</u>	Weighted Average Exercise Price	Weighted Average Remaining Contractual Life	Aggregate Intrinsic Value <u>(in millions)</u>
Balance at December 31, 2010	5,104.1	\$ 7.27		
Options granted	1,056.8	\$ 6.02		
Options exercised	(87.9)	\$ 3.97		
Options forfeited	(350.1)	\$ 8.04		
Balance at June 30, 2011	<u>5,722.9</u>	\$ 7.07	7.5 years	\$ 5.0
Vested and unvested expected to vest, June 30, 2011	5,429.6	\$ 7.14	7.4 years	\$ 4.7
Exercisable at June 30, 2011	3,142.8	\$ 8.16	6.4 years	\$ 2.3

On April 18, 2011, John F. Crowley resigned as Chairman and Chief Executive Officer of the Company and was appointed by the Board of Directors of the Company as Executive Chairman. On June 28, 2011, the Company announced that it had entered into a new employment agreement with Mr. Crowley pursuant to which he would return as Chief Executive Officer in August 2011; Mr. Crowley subsequently resumed his duties as Chief Executive Officer on August 4, 2011. The changes in Mr. Crowley's roles both on April 18, 2011 and June 28, 2011 caused two separate modifications of the terms of his outstanding stock option grants resulting in additional compensation expense of approximately \$2.7 million.

*Restricted Stock Awards* — Restricted stock awards are granted subject to certain restrictions, including in some cases service conditions. The grant-date fair value of restricted stock awards, which has been determined based upon the market value of the Company's shares on the grant date, is expensed over the vesting period.

Upon Mr. Crowley's resignation as Chairman and Chief Executive Officer in April 2011, the Board appointed Matthew R. Patterson as Acting Chief Executive Officer in addition to his duties as President of the Company. In connection with this appointment, the Company granted Mr. Patterson 50,000 shares of restricted stock. These shares will vest in full upon the earliest of (i) eighteen months after the grant date, or October 18, 2012, (ii) two business days following the announcement of preliminary results from the Company's first phase 3 study evaluating Amigal for Fabry disease (Study 011), or (iii) the date on which Mr. Patterson's employment with the Company ceases due to a termination by the Company without cause or a resignation by Mr. Patterson for good reason which includes the appointment of a person other than Mr. Patterson to the permanent Chief Executive Officer position. On August 8, 2011 the Company announced that Mr. Patterson expects to leave Amicus at the end of August.

The following table summarizes information on the Company's restricted stock:

	<u>Restricted Stock</u>	
	<u>Number of Shares (in thousands)</u>	<u>Weighted Average Grant Date Fair Value</u>
Unvested at December 31, 2010	—	\$ —
Granted	50.0	\$ 7.21
Vested	—	\$ —
Forfeited	—	\$ —
Unvested at June 30, 2011	<u>50.0</u>	\$ 7.21

As of June 30, 2011, the total unrecognized compensation cost related to unvested restricted stock awards was approximately \$0.3 million. This cost is expected to be recognized over a weighted average period of 1.3 years.



**Note 6. Short-Term Borrowings and Long-Term Debt**

In May 2009, the Company entered into a loan and security agreement with Silicon Valley Bank (SVB) that provides for up to \$4 million of equipment financing through October 2012. Borrowings under the loan agreement are collateralized by equipment purchased with the proceeds of the loan and bear interest at a fixed rate of approximately 9%. The loan agreement contains customary terms and conditions, including a financial covenant whereby the Company must maintain a minimum amount of liquidity measured at the end of each month where unrestricted cash, cash equivalents, and marketable securities is greater than \$20 million plus outstanding debt due to SVB. The Company has at all times been in compliance with this covenant during the term of the agreement.

At June 30, 2011, the current and long-term amounts due under the loan agreement were \$1.3 million and \$0.4 million, respectively. The carrying amount of the Company's borrowings approximates fair value at June 30, 2011.

**Note 7. Assets and Liabilities Measured at Fair Value**

The Company's financial assets and liabilities are measured at fair value and classified within the fair value hierarchy which is defined as follows:

*Level 1* — Quoted prices in active markets for identical assets or liabilities that the Company has the ability to access at the measurement date.

*Level 2* — Inputs other than quoted prices in active markets that are observable for the asset or liability, either directly or indirectly.

*Level 3* — Inputs that are unobservable for the asset or liability.

***Cash, Money Market Funds and Marketable Securities***

The Company classifies its cash and money market funds within the fair value hierarchy as Level 1 as these assets are valued using quoted prices in active market for identical assets at the measurement date. The Company considers its investments in marketable securities as available for sale and classifies these assets within the fair value hierarchy as Level 2 primarily utilizing broker quotes in a non-active market for valuation of these securities. No changes in valuation techniques or inputs occurred during the three months ended June 30, 2011. No transfers of assets between Level 1 and Level 2 of the fair value measurement hierarchy occurred during the six months ended June 30, 2011.

***Secured Debt***

As disclosed in Note 6, the Company has a loan and security agreement with Silicon Valley Bank. The carrying amount of the Company's borrowings approximates fair value at June 30, 2011. The Company's secured debt is classified as Level 2 and the fair value is estimated using quoted prices for similar liabilities in active markets, as well as inputs that are observable for the liability (other than quoted prices), such as interest rates that are observable at commonly quoted intervals.

**Warrants**

The Company allocated \$3.3 million of proceeds from its March 2010 registered direct offering to warrants issued in connection with the offering that was classified as a liability. The valuation of the warrants is determined using the Black-Scholes model. This model uses inputs such as the underlying price of the shares issued when the warrant is exercised, volatility, risk free interest rate and expected life of the instrument. The Company has determined that the warrant liability should be classified within Level 3 of the fair value hierarchy by evaluating each input for the Black Scholes model against the fair value hierarchy criteria and using the lowest level of input as the basis for the fair value classification. There are six inputs: closing price of Amicus stock on the day of evaluation; the exercise price of the warrants; the remaining term of the warrants; the volatility of Amicus' stock over that term; annual rate of dividends; and the riskless rate of return. Of those inputs, the exercise price of the warrants and the remaining term are readily observable in the warrant agreements. The annual rate of dividends is based on the Company's historical practice of not granting dividends. The closing price of Amicus stock would fall under Level 1 of the fair value hierarchy as it is a quoted price in an active market. The riskless rate of return is a Level 2, while the historical volatility is a Level 3 input in accordance with the fair value accounting guidance. Since the lowest level input is a Level 3, the Company determined the warrant liability is most appropriately classified within Level 3 of the fair value hierarchy. This liability is subject to fair value mark-to-market adjustment each period. As a result, for the six month period ended June 30, 2011, the Company recognized the change in the fair value of the warrant liability as a non-operating expense of \$1.4 million. The resulting fair value of the warrant liability at June 30, 2011, was \$6.1 million.

A summary of the fair value of the Company's assets and liabilities aggregated by the level in the fair value hierarchy within which those measurements fall as of June 30, 2011, are identified in the following table (in thousands):

	<u>Level 1</u>	<u>Level 2</u>	<u>Total</u>	
<b>Assets:</b>				
Cash/Money market funds	\$ 25,789	\$ —	\$ 25,789	
U.S. government agency securities	—	1,997	1,997	
Corporate debt securities	—	44,911	44,911	
Commercial paper	—	9,999	9,999	
Certificate of deposit	—	350	350	
	<u>\$ 25,789</u>	<u>\$ 57,257</u>	<u>\$ 83,046</u>	
	<u>Level 1</u>	<u>Level 2</u>	<u>Level 3</u>	<u>Total</u>
<b>Liabilities:</b>				
Secured debt	\$ —	\$ 1,671	\$ —	\$ 1,671
Warrants liability	—	—	6,066	6,066
	<u>\$ —</u>	<u>\$ 1,671</u>	<u>\$ 6,066</u>	<u>\$ 7,737</u>

The following table summarizes the changes in Level 3 liability for the six months ended June 30, 2011 (in thousands):

	<u>Balance as of December 31, 2010</u>	<u>Increase/(decrease) in fair value</u>	<u>Balance as of June 30, 2011</u>
Warrants liability	\$ 4,712	\$ 1,354	\$ 6,066

## Note 8. Collaborative Agreements

### GSK

On October 28, 2010, the Company entered into the License and Collaboration Agreement with Glaxo Group Limited, an affiliate of GSK, to develop and commercialize Amigal. Under the terms of the License and Collaboration Agreement, GSK received an exclusive worldwide license to develop, manufacture and commercialize Amigal. In consideration of the license grant, the Company received an upfront, license payment of \$30 million from GSK and is eligible to receive further payments of approximately \$170 million upon the successful achievement of development and commercialization milestones, as well as tiered double-digit royalties on global sales of Amigal. GSK and the Company will jointly fund development costs in accordance with an agreed upon development plan. This plan provides that the Company will fund 50% of the development costs for 2011 and 25% of the development costs in 2012 and beyond. The Company's development costs are subject to annual and aggregate caps. Additionally, GSK purchased approximately 6.9 million shares of the Company's common stock at \$4.56 per share. The total value of this equity investment to the Company was approximately \$31 million and represents a 19.9% ownership position in the Company. Under the terms of the collaboration agreement, while the Company will collaborate with GSK, GSK will have decision-making authority over clinical, regulatory and commercial matters. Additionally, GSK will have primary responsibility for interactions with regulatory agencies and prosecuting applications for marketing and reimbursement approvals worldwide.

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

The total arrangement consideration which was allocated to the single unit of accounting identified above was \$33.2 million which consists of the upfront license payment of \$30 million and the premium over the closing market price of the common stock transaction of \$3.2 million. The Company will recognize this consideration as Collaboration Revenue on a straight-line basis over the development period of 5.2 years as included in the detailed development plan that was included in the collaboration agreement. The Company determined that the overall level of activity over the development period approximates a straight-line approach. At June 30, 2011, the Company recognized approximately \$4.2 million of the total arrangement consideration as Collaboration Revenue since the inception of the agreement.

The revenue associated with reimbursements for research and development costs under collaboration agreements is included in Research Revenue and the costs associated with these reimbursable amounts are included in research and development expenses. The Company records these reimbursements as revenue and not as a reduction of research and development expenses as the Company has not commenced its planned principal operations (i.e., selling commercial products) and is a development stage enterprise, therefore development activities are part of its ongoing central operations. During the six months ended June 30, 2011, the Company recorded \$6.7 million in Research Revenue. As of June 30, 2011, the Company recorded \$2.0 million of current receivable due from GSK related to reimbursed research and development costs.

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The Company evaluated the contingent milestones included in the collaboration agreement at the inception of the collaboration agreement and determined that the contingent milestones are substantive milestones and will be recognized as revenue in the period that the milestone is achieved. The Company determined that the research based milestones are commensurate with the enhanced value of each delivered item as a result of the Company's specific performance to achieve the milestones. There is considerable effort underway to meet the specified milestones and complete the development of Amigal. Additionally, there is considerable time and effort involved in evaluating the data from the clinical trials that are planned and underway and if acceptable, in preparing the documentation required for filing for approval with the applicable regulatory authorities. The research based milestones would relate to past performances when achieved and are reasonable relative to the other payment terms within the collaboration agreement, including the \$30 million upfront payment and the cost sharing arrangement.

### **Note 9. Restructuring Charges**

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

The following table summarizes the restructuring charges and utilization for the six months ended June 30, 2011 (in thousands):

	<b>Balance as of December 31, 2010</b>	<b>Charges</b>	<b>Cash Payments</b>	<b>Adjustments</b>	<b>Balance as of June 30, 2011</b>
Facilities consolidation	\$ 268	—	\$ (114)	—	\$ 154

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

**Overview**

Amicus Therapeutics, Inc. (Amicus) is a biopharmaceutical company focused on the discovery, development and commercialization of orally-administered, small molecule drugs known as pharmacological chaperones for the treatment of rare diseases. Pharmacological chaperones are a novel, first-in-class approach to treating a broad range of diseases including lysosomal storage disorders and diseases of neurodegeneration. Our current areas of focus include the following:

- the Phase 3 development of our lead product candidate, Amigal for Fabry disease;
- the preclinical and clinical development of pharmacological chaperones co-administered with enzyme replacement therapy; and
- the preclinical evaluation of the use of pharmacological chaperones for neurodegenerative diseases.

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

Our lead product candidate, Amigal (migalastat hydrochloride) for Fabry disease, is in Phase 3 development. We are developing and commercializing Amigal with an affiliate of GlaxoSmithKline PLC (GSK) pursuant to a License and Collaboration Agreement entered into in October 2010. Our partnership with GSK allows us to utilize GSK's significant expertise in clinical, regulatory, commercial and manufacturing matters in the development of Amigal. In addition, the cost-sharing arrangements and potential milestone and royalty payments under the License and Collaboration Agreement provide us with financial strength and allow us to continue the development of Amigal while also advancing our other programs for the treatment of other lysosomal storage disorders and neurodegenerative diseases. We also believe this collaboration is important in validating our status as a leader in the development of treatments for rare diseases given the increasing focus placed on the rare disease field.

Our Phase 3 clinical development program for the use of Amigal as monotherapy in Fabry disease includes two clinical trials: Study 011 and Study 012. We have enrolled a majority of the planned 60 patients for Study 011, and have commenced an additional Phase 3 study (Study 012). We plan to use the data from Study 011 to support the filing of a New Drug Application, or NDA, for marketing approval in the United States and the data from Study 012 to support the filing of an application for marketing authorization in Europe.

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

While our initial clinical efforts have focused on the use of pharmacological chaperones to treat lysosomal storage disorders, we believe that our technology may be applicable to the treatment of certain diseases of neurodegeneration. Our lead preclinical program in this area is focused on Parkinson's disease, where we expect to complete late-stage preclinical proof of concept studies, including IND-enabling activities, for our pharmacological chaperone molecule AT3375 during 2011. Our second preclinical program in this area is focused on Alzheimer's disease. Our preclinical work in both Parkinson's and Alzheimer's disease is presently focused on genetically-defined subpopulations of Parkinson's and Alzheimer's patients and leverages our expertise and knowledge in the rare disease field.

We have generated significant losses to date and expect to continue to generate losses as we continue the clinical development of our drug candidates, including Amigal, and conduct preclinical studies on other programs. These activities are budgeted to expand over time and will require further resources if we are to be successful. From our inception in February 2002 through June 30, 2011, we have accumulated a deficit of \$251.7 million. As we have not yet generated commercial sales revenue from any of our product candidates, our losses will continue and are likely to be substantial over at least the next couple of years.

In June 2007, we completed our initial public offering (IPO) of 5,000,000 shares of common stock at a public offering price of \$15.00 per share. Net cash proceeds from the initial public offering were approximately \$68.1 million. In March 2010, we sold 4.95 million shares of our common stock and warrants to purchase 1.85 million shares of common stock in a registered direct offering to a select group of institutional investors. The shares of common stock and warrants were sold in units consisting of one share of common stock and one warrant to purchase 0.375 shares of common stock at a price of \$3.74 per unit. The warrants have a term of four years and are exercisable any time on or after the six month anniversary of the date they were issued, at an exercise price of \$4.43 per share. The net proceeds of the offering were \$17.1 million.

### **Program Status**

#### *Amigal for Fabry Disease: Phase 3 Global Registration Program*

We and our partner GSK are conducting two Phase 3 registration studies to support the global approval of Amigal for the treatment of Fabry disease. Both studies are evaluating Fabry patients with genetic mutations that may be addressable with Amigal monotherapy.

Study 011 is a six-month, randomized, double-blind, placebo-controlled study to support marketing applications to the FDA and other regulatory agencies. Patient enrollment is ongoing at 37 centers worldwide, and the study is expected to achieve target enrollment in the fourth quarter of 2011.

During the third quarter of 2011, we and GSK expect to dose the first patient in Study 012 to support the global approval of Amigal. Study 012 is a randomized, open-label, 18-month Phase 3 study to compare the safety and efficacy of Amigal and ERT. Approximately 50 male or female patients that are currently on ERT will be randomized (30 to switch to Amigal and 20 to remain on ERT). The primary outcome of efficacy will be renal function as measured by glomerular filtration rate (GFR).

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

The broader use of pharmacological chaperones co-administered with ERT represents an important extension of the Company's chaperone technology platform. Results of preclinical studies in animal models of Fabry and Pompe diseases have consistently demonstrated that a pharmacological chaperone can selectively bind to and stabilize the infused ERT, prevent the loss of activity of ERT in the circulation, increase tissue uptake of the ERT, and increase substrate reduction over ERT alone.

Two Phase 2 PC-ERT co-administration studies are currently underway to build from these preclinical proof-of-concept results and potentially create a platform for expansion into other lysosomal storage disorders that are currently treated with ERT.

We plan to announce preliminary results in the fourth quarter of 2011 from a Phase 2 study (Study 013) being conducted with GSK investigating drug-drug interactions between Amigal and ERT. After an initial ERT infusion, patients will return two weeks later to receive Amigal at one of two oral dose levels, prior to a second ERT infusion. The primary outcome measures will be safety and a comparison of the ERT activity in plasma, with and without co-administration of Amigal, in up to 18 male patients with Fabry disease.

We have also initiated sites for a Phase 2 study (Study 010) of AT2220 co-administered with ERT (Myozyme/Lumizyme) in individuals with Pompe disease. This study is on track to dose the first patient in the third quarter of 2011 and follows the same principle as Study 013 to investigate drug-drug interactions between AT2220 and ERT. The primary outcome measures will be safety and a comparison of the ERT activity in plasma, with and without co-administration of AT2220.

#### *Pharmacological Chaperones for the Treatment of Parkinson's and Alzheimer's Disease*

Based on genetic links between Parkinson's and Gaucher disease, we are developing AT3375 for Parkinson's disease. AT3375 is a pharmacological chaperone targeted at glucocerebrosidase (GCase), the enzyme deficient in Gaucher disease. Mutations in the GBA1 gene for GCase are the most common genetic risk factor known for Parkinson's disease. Gaucher carriers, who have one mutant copy of GCase, are approximately five times more frequent in the Parkinson's disease population. In addition, Gaucher patients, who have two mutant copies of GCase, have an estimated 20-fold increased risk of developing Parkinson's disease.

We believe that AT3375 has the potential to address Parkinson's patients who are Gaucher carriers. We continue to evaluate AT3375 in preclinical studies for Parkinson's disease, and plan to report late-stage preclinical proof-of-concept results during the second half of 2011.

We are also currently researching novel approaches to treating patients with Genetic (Familial) Alzheimer's through a Presenilin-1 target and those with Sporadic Alzheimer's, with a focus on a lysosomal enzyme target. Our work in Alzheimer's also builds on the understanding of pharmacological chaperones we have developed over the past several years examining treatment of lysosomal storage disorders and our work in Parkinson's disease. We expect to continue preclinical proof-of-concept studies in Alzheimer's disease during the remainder of 2011.

#### **Collaboration with GSK**

On October 28, 2010, the Company entered into the License and Collaboration Agreement with Glaxo Group Limited, an affiliate of GSK, to develop and commercialize Amigal. Under the terms of the License and Collaboration Agreement, GSK received an exclusive worldwide license to develop, manufacture and commercialize Amigal. In consideration of the license grant, the Company received an upfront, license payment of \$30 million from GSK and is eligible to receive further payments of approximately \$170 million upon the successful achievement of development and commercialization milestones, as well as tiered double-digit royalties on global sales of Amigal. GSK and the Company will jointly fund development costs in accordance with an agreed upon development plan. This plan provides that the Company will fund 50% of the development costs for 2011 and 25% of the development costs in 2012 and beyond. The Company's development costs are subject to annual and aggregate caps. Additionally, GSK purchased approximately 6.9 million shares of the Company's common stock at a price of \$4.56 per share. The total value of this equity investment to the Company is approximately \$31 million and represents a 19.9% ownership position in the Company. Under the terms of the collaboration agreement, while we will collaborate with GSK, GSK will have decision-making authority over clinical, regulatory and commercial matters related to Amigal. Additionally, GSK will have primary responsibility for interactions with regulatory agencies and prosecuting applications for marketing and reimbursement approvals worldwide.

## **Financial Operations Overview**

### ***Revenue***

In November 2010, GSK paid us an initial, non-refundable license fee of \$30 million and a premium of \$3.2 million related to GSK's purchase of an equity investment in Amicus. The total upfront consideration received of \$33.2 million will be recognized as Collaboration Revenue on a straight-line basis over the development period of the collaboration agreement which is approximately 5.2 years. For the six months ended June 30, 2011, we recognized approximately \$3.3 million of the total upfront consideration as Collaboration Revenue. For the six months ended June 30, 2011, we recognized \$6.7 million of Research Revenue for reimbursed research and development costs. We have not generated any commercial sales revenue since our inception.

### ***Research and Development Expenses***

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

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

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

We expense research and development costs as incurred, including payments made to date under our license agreements. We believe that significant investment in product development is a competitive necessity and plan to continue these investments in order to realize the potential of our product candidates. From our inception in February 2002 through June 30, 2011, we have incurred research and development expense in the aggregate of \$237.5 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		Six Months Ended		Period from
	June 30,		June 30,		February 4, 2002
	2010	2011	2010	2011	(inception) to
					June 30,
					2011
Third party direct project expenses					
Amigal (Fabry Disease — Phase 3)	\$ 2,557	\$ 4,166	\$ 5,120	\$ 8,228	\$ 54,258
Plicera (Gaucher Disease — Phase 2*)	81	(1)	334	(201)	26,026
AT2220 (Pompe Disease — Phase 2)	135	41	311	45	13,179
Neurodegenerative Diseases (Preclinical)	57	498	406	949	7,348
Total third party direct project expenses	<u>2,830</u>	<u>4,704</u>	<u>6,171</u>	<u>9,021</u>	<u>100,811</u>
Other project costs (1)					
Personnel costs	4,002	4,801	8,183	9,724	84,161
Other costs (2)	1,305	2,113	2,672	3,998	52,535
Total other project costs	<u>5,307</u>	<u>6,914</u>	<u>10,855</u>	<u>13,722</u>	<u>136,696</u>
Total research and development costs	<u>\$ 8,137</u>	<u>\$ 11,618</u>	<u>\$ 17,026</u>	<u>\$ 22,743</u>	<u>\$ 237,507</u>

(1) Other project costs are leveraged across multiple projects.

(2) Other costs include facility, supply, overhead, and licensing costs that support multiple clinical and preclinical projects.

\* We do not plan to advance Plicera 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 Amigal 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. In addition, GSK has considerable influence over and decision-making authority related to our Amigal program. A change in the outcome of any of the foregoing variables with respect to the development of a product candidate could mean a significant change in the costs and timing associated with the development, regulatory approval and commercialization of that product candidate. For example, if the FDA or other regulatory authorities were to require us to conduct clinical trials beyond those which we currently anticipate, or if we experience significant delays in enrollment in any of our clinical trials, we could be required to expend significant additional financial resources and time on the completion of clinical development. Drug development may take several years and millions of dollars in development costs.

### ***General and Administrative Expense***

General and administrative expense consists primarily of salaries and other related costs, including stock-based compensation expense, for persons serving in our executive, finance, accounting, legal, information technology and human resource functions. Other general and administrative expense includes facility-related costs not otherwise included in research and development expense, promotional expenses, costs associated with industry and trade shows, and professional fees for legal services, including patent-related expense and accounting services. From our inception in February 2002 through June 30, 2011, we spent \$104.5 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 capital lease facility and our equipment financing agreement.

### ***Critical Accounting Policies and Significant Judgments and Estimates***

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

While there were no significant changes during the quarter ended June 30, 2011 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, 2010, we believe that the following accounting policies are the most critical to aid you in fully understanding and evaluating our

### ***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) estimated selling price (BESP) if neither VSOE nor TPE is available. We would establish the VSOE of selling price using the price charged for a deliverable when sold separately. The TPE of selling price would be established by evaluating largely similar and interchangeable competitor products or services in standalone sales to similarly situated customers. The best estimate of selling price would be established considering internal factors such as an internal pricing analysis or an income approach using a discounted cash flow model.

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

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

### ***Accrued Expenses***

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

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

### ***Stock-Based Compensation***

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

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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 the mid-point between the vesting date and the end of the contractual term. The risk-free interest rate is based on U.S. Treasury, zero-coupon issues with a remaining term equal to the expected life assumed at the date of grant. Forfeitures are estimated based on voluntary termination behavior, as well as a historical analysis of actual option forfeitures. The weighted average assumptions used in the Black-Scholes option pricing model are as follows:

	<b>Three Months Ended June 30, 2010</b>	<b>Six Months Ended June 30, 2010</b>	<b>Three Months Ended June 30, 2011</b>	<b>Six Months Ended June 30, 2011</b>
Expected stock price volatility	80.0%	80.5%	78.4%	78.8%
Risk free interest rate	2.4%	2.5%	2.1%	2.2%
Expected life of options (years)	6.25	6.25	6.25	6.25
Expected annual dividend per share	\$ 0.00	\$ 0.00	\$ 0.00	\$ 0.00

**Warrants**

The warrants issued in connection with the March 2010 registered direct offering are classified as a liability. The fair value of the warrants liability is evaluated at each balance sheet date using the Black-Scholes valuation model. This model uses inputs such as the underlying price of the shares issued when the warrant is exercised, volatility, risk free interest rate and expected life of the instrument. Any changes in the fair value of the warrants liability is recognized in the consolidated statement of operations. The weighted average assumptions used in the Black-Scholes valuation model for the warrants June 30, 2011 and 2010 are as follows:

	<u>June 30, 2010</u>	<u>June 30, 2011</u>
Expected stock price volatility	79.4%	76.6%
Risk free interest rate	1.3%	.74%
Expected life of warrants (years)	3.67	2.67
Expected annual dividend per share	\$ 0.00	\$ 0.00

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

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

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

(In thousands, except per share amount)	<u>Three Months Ended</u> <u>June 30,</u>		<u>Six Months Ended</u> <u>June 30,</u>	
	<u>2010</u>	<u>2011</u>	<u>2010</u>	<u>2011</u>
<b>Historical</b>				
Numerator:				
Net loss attributable to common stockholders	<u>\$ (11,315)</u>	<u>\$ (12,641)</u>	<u>\$ (24,491)</u>	<u>\$ (25,991)</u>
Denominator:				
Weighted average common shares outstanding — basic and diluted	<u>27,623,297</u>	<u>34,530,693</u>	<u>25,956,366</u>	<u>34,514,947</u>

Dilutive common stock equivalents would include the dilutive effect of common stock options and warrants for common stock equivalents. Potentially dilutive common stock equivalents totaled approximately 7.1 million and 7.6 million for the six months ended June 30, 2010 and 2011, respectively. Potentially dilutive common stock equivalents were excluded from the diluted earnings per share denominator for all periods because of their anti-dilutive effect.

## Results of Operations

### *Three Months Ended June 30, 2011 Compared to Three Months Ended June 30, 2010*

*Revenue.* In November 2010, GSK paid us an initial, non-refundable license fee of \$30 million and a premium of \$3.2 million related to GSK's purchase of an equity investment in Amicus. The total upfront consideration received of \$33.2 million will be recognized as Collaboration Revenue on a straight-line basis over the development period of the collaboration agreement which is approximately 5.2 years. For the three months ended June 30, 2011, we recognized \$1.7 million of the total upfront consideration as Collaboration Revenue. For the three months ended June 30, 2011, we recognized \$2.4 million of Research Revenue for reimbursed research and development costs. We have not generated any commercial sales revenue since our inception.

*Research and Development Expense.* Research and development expense was \$11.6 million for the three months ended June 30, 2011, representing an increase of \$3.5 million or 43% from \$8.1 million for the three months ended June 30, 2010. The variance was primarily attributable to higher personnel costs, an increase in consulting costs and an increase in contract research and manufacturing costs due to the increased activity within the Fabry program.

*General and Administrative Expense.* General and administrative expense was \$6.7 million for the three months ended June 30, 2011, representing an increase of \$2.7 million or 68% from \$4.0 million for the three months ended June 30, 2010. The variance was primarily due to additional stock option compensation expense recognized as a result of the change in the terms of the Chief Executive Officer's stock options resulting from his resignation and subsequent reappointment to the Chief Executive Officer position.

*Interest Income and Interest Expense.* Interest income was \$0.05 million for the three months ended June 30, 2011, and 2010. Interest expense was approximately \$0.05 million for the three months ended June 30, 2011 compared to \$0.1 for the three months ended June 30, 2010. The decrease was due to less outstanding debt during the period on the secured loan.

*Change in Fair Value of Warrant Liability.* In connection with the sale of our common stock and warrants from the registered direct offering in March 2010, we recorded the warrants as a liability at their fair value using a Black-Scholes model and remeasure the fair value at each reporting date until exercised or expired. Changes in the fair value of the warrants are reported in the statements of operations as non-operating income or expense. For the three months ended June 30, 2011, we reported a gain of \$2.1 million related to the decrease in fair value of these warrants from issuance dates compared to a gain of \$1.4 million for the three month period ending June 30, 2010. The market price for our common stock has been and may continue to be volatile. Consequently, future fluctuations in the price of our common stock may cause significant increases or decreases in the fair value of these warrants.

## Results of Operations

### *Six Months Ended June 30, 2011 Compared to Six Months Ended June 30, 2010*

*Revenue.* In November 2010, GSK paid us an initial, non-refundable license fee of \$30 million and a premium of \$3.2 million related to GSK's purchase of an equity investment in Amicus. The total upfront consideration received of \$33.2 million will be recognized as Collaboration Revenue on a straight-line basis over the development period of the collaboration agreement which is approximately 5.2 years. For the six months ended June 30, 2011, we recognized \$3.3 million of the total upfront consideration as Collaboration Revenue. For the six months ended June 30, 2011, we recognized \$6.7 million of Research Revenue for reimbursed research and development costs. We have not generated any commercial sales revenue since our inception.

*Research and Development Expense.* Research and development expense was \$22.7 million for the six months ended June 30, 2011, representing an increase of \$5.7 million or 34% from \$17.0 million for the six months ended June 30, 2010. The variance was primarily attributable to higher personnel costs, an increase in consulting costs and an increase in contract research and manufacturing costs due to the increased activity within the Fabry program.

*General and Administrative Expense.* General and administrative expense was \$11.1 million for the six months ended June 30, 2011, representing an increase of \$3.2 million or 41% from \$7.9 million for the six months ended June 30, 2010. The variance was primarily due an increase in consulting fees as well as additional stock option compensation expense recognized as a result of the change in the terms of the Chief Executive Officer's stock options resulting from his resignation and subsequent reappointment to the Chief Executive Officer position.

*Interest Income and Interest Expense.* Interest income was \$0.1 million for the six months ended June 30, 2011, and 2010. Interest expense was approximately \$0.1 million for the six months ended June 30, 2011, and 2010.

*Change in Fair Value of Warrant Liability.* In connection with the sale of our common stock and warrants from the registered direct offering in March 2010, we recorded the warrants as a liability at their fair value using a Black-Scholes model and remeasure the fair value at each reporting date until exercised or expired. Changes in the fair value of the warrants are reported in the statements of operations as non-operating income or expense. For the six months ended June 30, 2011, we reported a loss of \$1.4 million related to the increase in fair value of these warrants from issuance dates compared to a gain of \$1.6 million for the six months ended June 30, 2010. The market price for our common stock has been and may continue to be volatile. Consequently, future fluctuations in the price of our common stock may cause significant increases or decreases in the fair value of these warrants.

*Other Income/Expense.* Other income increased due to funds received from the U.S. Treasury Department in February 2011 of \$0.07 million under the Qualified Therapeutic Discovery Projects tax credit and grant program.

## Liquidity and Capital Resources

### Source of Liquidity

As a result of our significant research and development expenditures and the lack of any approved products to generate product sales revenue, we have not been profitable and have generated operating losses since we were incorporated in 2002. We have funded our operations principally with \$148.7 million of proceeds from redeemable convertible preferred stock offerings, \$75.0 million of gross proceeds from our IPO in June 2007, \$18.5 million of gross proceeds from our Registered Direct Offering in March 2010, \$80.0 million from the non-refundable license fees from the collaboration agreements and \$31.0 million from GSK's investment in the Company at the time the collaboration was formed. In the future, we expect to fund our operations, in part, through the receipt of cost-sharing and milestone payments from GSK. The following table summarizes our significant funding sources as of June 30, 2011:

<u>Funding</u>	<u>Year</u>	<u>No. Shares</u>	<u>Approximate Amount<sup>(1)</sup> (in thousands)</u>
Series A Redeemable Convertible Preferred Stock	2002	444,443	\$ 2,500
Series B Redeemable Convertible Preferred Stock	2004, 2005, 2006, 2007	4,917,853	31,189
Series C Redeemable Convertible Preferred Stock	2005, 2006	5,820,020	54,999
Series D Redeemable Convertible Preferred Stock	2006, 2007	4,930,405	60,000
Common Stock	2007	5,000,000	75,000
Upfront License Fee from Shire	2007	—	50,000
Registered Direct Offering	2010	4,946,525	18,500
Upfront License Fee from GSK	2010	—	30,000
Common Stock GSK	2010	6,866,245	31,285
		<u>32,925,491</u>	<u>\$ 353,473</u>

(1) Represents gross proceeds

In addition, in conjunction with the GSK collaboration agreement, we received reimbursement of research and development expenditures from the date of the agreement (October 28, 2010) through June 30, 2011 of \$4.6 million. We also received \$31.1 million in reimbursement of research and development expenditures from the prior Shire collaboration from the date of the agreement (November 7, 2007) through year-end 2009.

As of June 30, 2011, we had cash, cash equivalents and marketable securities of \$83.0 million. We invest cash in excess of our immediate requirements with regard to liquidity and capital preservation in a variety of interest-bearing instruments, including obligations of U.S. government agencies and money market accounts. Wherever possible, we seek to minimize the potential effects of concentration and degrees of risk. Although we maintain cash balances with financial institutions in excess of insured limits, we do not anticipate any losses with respect to such cash balances.

### Net Cash Used in Operating Activities

Net cash used in operations for the six months ended June 30, 2010 of \$25.3 million was primarily comprised of the net loss for the six months ended June 30, 2010 of \$24.5 million and the change in other operating assets and liabilities of \$3.5 million.

Net cash used in operations for the six months ended June 30, 2011 was \$23.9 million due to the net loss for the six months ended June 30, 2011 of \$26.0 million, an increase in a receivable of \$1.9 million related to amounts due from GSK from the collaboration agreement and a decrease in other operating liabilities of \$2.6 million.



***Net Cash Provided By Investing Activities***

Net cash provided by investing activities for the six months ended June 30, 2010 was \$14.3 million. Net cash provided by investing activities reflects \$52.5 million for the sale and redemption of marketable securities, partially offset by \$38.1 million for the purchase of marketable securities and \$0.1 million for the acquisition of property and equipment.

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

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

Net cash provided by financing activities for the six months ended June 30, 2010 was \$16.3 million, consisting of \$17.1 million from the issuance of common stock primarily offset by the payments of our secured loan agreement and capital lease obligations of \$0.6 million and \$0.2 million, respectively.

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

***Funding Requirements***

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

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

We do not anticipate that we will generate revenue from commercial sales until at least 2013, if at all. In the absence of additional funding, we expect our continuing operating losses to result in increases in our cash used in operations over the next several quarters and years. However, we believe that our existing cash and cash equivalents and short-term investments, including anticipated payments from GSK in connection with the collaboration, is expected to be sufficient to fund our operating expenses and capital expenditure requirements through at least the end of 2012.

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

#### *Milestone Payments*

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

#### *Royalties*

Under our license agreements, if we owe royalties on net sales for one of our products to more than one licensor, then we have the right to reduce the royalties owed to one licensor for royalties paid to another. The amount of royalties to be offset is generally limited in each license and can vary under each agreement. For Amigal and AT2220, we will owe royalties only to Mt. Sinai School of Medicine (MSSM). We would expect to pay royalties to all three licensors with respect to Plicera should we advance Plicera to commercialization. To date, we have not made any royalty payments on sales of our products and believe we are at least a couple years away from selling any products that would require us to make any such royalty payments.

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

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

### **ITEM 3. Quantitative and Qualitative Disclosures about Market Risk**

Market risk is the risk of change in fair value of a financial instrument due to changes in interest rates, equity prices, creditworthiness, financing, exchange rates or other factors. Our primary market risk exposure relates to changes in interest rates in our cash, cash equivalents and marketable securities. We place our investments in high-quality financial instruments, primarily money market funds, corporate debt securities, asset backed securities and U.S. government agency notes with maturities of less than one year, which we believe are subject to limited interest rate and credit risk. The securities in our investment portfolio are not leveraged, are classified as available-for-sale and, due to the short-term nature, are subject to minimal interest rate risk. We currently do not hedge interest rate exposure and consistent with our investment policy, we do not use derivative financial instruments in our investment portfolio. At June 30, 2011, we held \$83.0 million in cash, cash equivalents and available for sale securities and due to the short-term maturities of our investments, we do not believe that a 10% change in average interest rates would have a significant impact on our interest income. Our outstanding debt has a fixed interest rate and therefore, we have no exposure to interest rate fluctuations.

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

**ITEM 4. CONTROLS AND PROCEDURES**

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

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

## **PART II. OTHER INFORMATION**

### **ITEM 1. LEGAL PROCEEDINGS**

We are not a party to any material legal proceedings.

### **ITEM 1A. RISK FACTORS**

There have been no material changes with respect to the Risk Factors disclosed in our Annual Report on Form 10-K for the year ended December 31, 2010.

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

#### **Recent Sales of Unregistered Securities**

None.

#### **Use of Proceeds**

##### *Initial Public Offering*

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

After deducting expenses of approximately \$6.9 million, we received net offering proceeds of approximately \$68.1 million from our initial public offering. As of June 30, 2011, approximately \$12.1 million of the net proceeds from our initial public offering were maintained in money market funds and in investment-grade, interest bearing instruments, pending their use. We have used the remaining proceeds of approximately \$56.0 million for clinical development of our projects, research and development activities relating to additional preclinical projects and to fund working capital and other general corporate purposes.

##### *March 2010 Registered Direct Offering*

In March 2010, we sold 4,946,524 million shares of our common stock and warrants to purchase 1,854,946 million shares of common stock in a registered direct offering to a select group of institutional investors through a Registration Statement on Form S-3 (File No. 333-158405) that was declared effective by the SEC on May 27, 2009. The shares of common stock and warrants were sold in units consisting of one share of common stock and one warrant to purchase 0.375 shares of common stock at a price of \$3.74 per unit. The warrants have a term of four years and are exercisable any time on or after the six month anniversary of the date they were issued, at an exercise price of \$4.43 per share. The aggregate offering proceeds were \$18.5 million. Leerink Swann LLC served as sole placement agent for the offering. Following the sale of the common stock and warrants, the public offering terminated.

We paid Leerink Swann a placement agency fee equal to 5.7% of the aggregate offering proceeds, approximately \$1.05 million. The net proceeds of the offering were approximately \$17.1 million after deducting the placement agency fee and all other estimated offering expenses. No offering expenses were paid directly or indirectly to any of our directors or officers (or their associates) or persons owning ten percent or more of any class of our equity securities or to any other affiliates.

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As of June 30, 2011, we had invested the \$17.1 million in net proceeds from our registered direct offering in money market funds and in investment-grade, interest bearing instruments, pending their use. Through June 30, 2011, we have not used the net proceeds from this offering. We intend to use the proceeds from this offering to further advance the development of our lead product candidate, Amigal, and the completion of certain activities required for the submission of a license application globally, as well as for general corporate matters.

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

### **Issuer Purchases of Equity Securities**

There were no purchases of our common stock for the six months ended June 30, 2011.

### **ITEM 3. DEFAULTS UPON SENIOR SECURITIES**

None.

### **ITEM 5. OTHER INFORMATION**

None.

**ITEM 6. EXHIBITS**

<b>Exhibit Number</b>	<b>Description</b>
3.1(1)	Restated Certificate of Incorporation
3.2(2)	Amended and Restated By-laws
10.1(3)	Employment Agreement dated April 18, 2011 between Amicus Therapeutics, Inc. and John F. Crowley
10.2(3)	Letter Agreement dated April 18, 2011 between Amicus Therapeutics, Inc. and Matthew R. Patterson
10.3(3)	Restricted Stock Award Agreement dated April 18, 2011 between Amicus Therapeutics, Inc. and Matthew R. Patterson
10.4(4)	Amended and Restated 2007 Equity Incentive Plan
10.5(5)	Employment Agreement dated June 28, 2011 between Amicus Therapeutics, Inc. and John F. Crowley
31.1*	Certification of Principal Executive Officer pursuant to Rules 13a-14 and 15d-14 promulgated pursuant to the Securities Exchange Act of 1934, as amended
31.2*	Certification of Principal Financial Officer pursuant to Rules 13a-14 and 15d-14 promulgated pursuant to the Securities Exchange Act of 1934, as amended
32.1*	Certification of Principal Executive Officer and Principal Financial Officer pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
(1)	Incorporated by reference to Exhibit 3.2 to our Registration Statement on Form S-1
(2)	Incorporated by reference to Exhibit 3.4 to our Registration Statement on Form S-1
(3)	Incorporated by reference to Exhibits 10.1, 10.2, and 10.3 to our Current Report on Form 8-K filed May 25, 2011
(4)	Incorporated by reference to Exhibit 10.1 to our Current Report on Form 8-K filed May 25, 2011
(5)	Incorporated by reference to Exhibit 10.1 to our Current Report on Form 8-K filed on June 30, 2011
*	These certifications are being furnished solely to accompany this quarterly report pursuant to 18 U.S.C. Section 1350, and are not being filed for purposes of Section 18 of the Securities Exchange Act of 1934 and are not to be incorporated by reference into any filing of Amicus Therapeutics, Inc., whether made before or after the date hereof, regardless of any general incorporation language in such filing.

**SIGNATURES**

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

AMICUS THERAPEUTICS, INC.

Date: August 11, 2011

By: /s/ JOHN F. CROWLEY  
John F. Crowley  
Chairman and Chief Executive Officer  
(Principal Executive Officer)

Date: August 11, 2011

By: /s/ DAPHNE QUIMI  
Daphne Quimi  
Corporate Controller  
(Principal Financial and Accounting Officer)

## INDEX TO EXHIBITS

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

I, John F. Crowley, certify that:

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

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

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

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

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

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

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

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

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

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

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

Date: August 11, 2011

/s/ John F. Crowley

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John F. Crowley

**Chairman and Chief Executive Officer**

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

I, Daphne Quimi, certify that:

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

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

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

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

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

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

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

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

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

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

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

Date: August 11, 2011

/s/ Daphne Quimi

\_\_\_\_\_  
Daphne Quimi

**Corporate Controller**

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

Each of the undersigned hereby certifies, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, in his capacity as an officer of Amicus Therapeutics, Inc. (the "Company"), that, to his knowledge, the Quarterly Report of the Company on Form 10-Q for the period ended June 30, 2011, fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934 (15 U.S.C. 78m or 78o(d)) and that the information contained in such report fairly presents, in all material respects, the financial condition and results of operations of the Company. This written statement is being furnished to the Securities and Exchange Commission as an exhibit to such Form 10-Q. A signed original of this statement has been provided to the Company and will be retained by the Company and furnished to the Securities and Exchange Commission or its staff upon request.

Date: August 11, 2011

By: /s/ John F. Crowley  
John F. Crowley  
**Chairman and Chief Executive Officer**

Date: August 11, 2011

By: /s/ Daphne Quimi  
Daphne Quimi  
**Corporate Controller**