

AMICUS THERAPEUTICS INC

Form 10-Q

October 31, 2007

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

**FORM 10-Q**

**(Mark One)**

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

**For the quarterly period ended September 30, 2007**

**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

20-0422823

(State or Other Jurisdiction of  
Incorporation or Organization)

(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 is a large accelerated filer, an accelerated filer, or a non-accelerated filer. See definition of "accelerated filer" and "large accelerated filer" in Rule 12b-2 of the Exchange Act. (Check one):

Large accelerated filer  Accelerated filer  Non-accelerated filer

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

The number of shares outstanding of the registrant's common stock, \$.01 par value per share, as of October 25, 2007 was 22,357,574 shares.

AMICUS THERAPEUTICS, INC  
Form 10-Q for the Quarterly Period Ended September 30, 2007

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<u>Certification of CEO</u>	
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<u>Certification of CEO and CFO</u>	
We have filed applications to register certain trademarks in the United States and abroad, including AMICUS™, AMICUS THERAPEUTICS™ (and design), AMIGAL™ and PLICERA™. Fabrazyme®, Cerezyme®, Myozyme®, Replagal®, and Zavesca® are the property of their respective owners.	



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**SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS**

This quarterly report on Form 10-Q contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts, included in this quarterly report on Form 10-Q regarding our strategy, future operations, future financial position, future revenues, projected costs, prospects, plans and objectives of management are forward-looking statements. The words anticipate, believe, estimate, expect, in, 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:

our plans to develop and commercialize Amigal, Plicera and AT2220;

our ongoing and planned discovery programs, preclinical studies and clinical trials;

our ability to enter into selective collaboration arrangements;

the timing of and our ability to obtain and maintain regulatory approvals for our product candidates;

the rate and degree of market acceptance and clinical utility of our products;

our ability to quickly and efficiently identify and develop product candidates;

the extent to which our scientific approach may potentially address a broad range of diseases across multiple therapeutic areas;

our commercialization, marketing and manufacturing capabilities and strategy;

our intellectual property position; and

our estimates regarding expenses, future revenues, capital requirements and needs for additional financing.

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 this quarterly report on Form 10-Q, particularly in the Risk Factors in section 1A of Part II of this quarterly report on Form 10-Q, 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 and the documents that we reference herein. We do not assume any obligation to update any forward-looking statements.

**Table of Contents****PART I. FINANCIAL INFORMATION****Item 1. Financial Statements (unaudited)**

**Amicus Therapeutics, Inc.**  
**(a development stage company)**  
**Consolidated Balance Sheets**  
*(Unaudited)*  
**(in thousands, except per share amounts)**

	<b>December 31, 2006</b>	<b>September 30, 2007</b>
<b>Assets:</b>		
Current assets:		
Cash and cash equivalents	\$ 12,127	\$ 19,933
Investments in marketable securities	42,572	99,475
Prepaid expenses and other current assets	321	1,245
Total current assets	55,020	120,653
Property and equipment, less accumulated depreciation and amortization of \$1,557 and \$2,478 at December 31, 2006 and September 30, 2007, respectively	4,358	3,913
Other non-current assets	267	267
<b>Total Assets</b>	<b>\$ 59,645</b>	<b>\$ 124,833</b>
<b>Liabilities and Stockholders Equity</b>		
Current liabilities:		
Accounts payable	\$ 1,195	\$ 535
Accrued expenses	7,704	7,515
Current portion of capital lease obligations	1,307	1,521
Total current liabilities	10,206	9,571
Warrant liability	609	
Capital lease obligations, less current portion	2,256	1,573
Series A redeemable convertible preferred stock, \$.01 par value, 444,443 shares authorized, issued and outstanding at December 31, 2006 (aggregate liquidation preference \$2,500 at December 31, 2006), no shares authorized, issued, or outstanding at September 30, 2007	2,476	
Series B redeemable convertible preferred stock, \$.01 par value, 4,936,730 shares authorized, 4,877,056 shares issued and outstanding at December 31, 2006 (aggregate liquidation preference \$31,000 at December 31, 2006), no shares authorized, issued, or outstanding at September 30, 2007	30,868	
Series C redeemable convertible preferred stock, \$.01 par value, 5,820,020 shares authorized, issued and outstanding at December 31, 2006 (aggregate liquidation preferences \$54,999 at December 31, 2006), no shares authorized, issued, or outstanding at September 30, 2007	54,869	

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Series D redeemable convertible preferred stock, \$.01 par value, 4,930,405 shares authorized, 2,953,878 issued and outstanding at December 31, 2006 (aggregate liquidation preference \$36,000), no shares authorized, issued, or outstanding at September 30, 2007	35,876	
Stockholders (deficiency) equity:		
Common stock, \$.01 par value, 21,333,333 shares authorized, 990,492 shares issued and outstanding at December 31, 2006, 50,000,000 shares authorized, 22,372,573 shares issued and outstanding at September 30, 2007	70	284
Additional paid-in capital	6,067	226,246
Accumulated other comprehensive income	15	220
Deficit accumulated during the development stage	(83,667)	(113,061)
Total stockholders (deficiency) equity	(77,515)	113,689
<b>Total Liabilities and Stockholders Equity</b>	<b>\$ 59,645</b>	<b>\$ 124,833</b>

*See accompanying notes to consolidated financial statements*

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**Amicus Therapeutics, Inc.**  
**(a development stage company)**  
**Consolidated Statements of Operations**  
*(Unaudited)*

**(in thousands, except share and per share amounts)**

	<b>Three Months</b>		<b>Nine Months</b>		<b>Period from</b>
					<b>February 4,</b>
					<b>2002</b>
					<b>(inception)</b>
					<b>to</b>
					<b>September</b>
					<b>30,</b>
					<b>2007</b>
	<b>Ended September 30,</b>	<b>2007</b>	<b>Ended September 30,</b>	<b>2007</b>	
	<b>2006</b>		<b>2006</b>		
Operating Expenses:					
Research and development	\$ 6,725	\$ 7,537	\$ 19,444	\$ 21,404	\$ 80,208
General and administrative	3,783	3,954	8,247	9,994	32,786
Impairment of leasehold improvements					1,030
Depreciation and amortization	245	315	661	924	2,481
In-process research and development					418
Total operating expenses	10,753	11,806	28,352	32,322	116,923
Loss from operations	(10,753)	(11,806)	(28,352)	(32,322)	(116,923)
Other income (expenses):					
Interest income	519	1,593	1,204	3,346	6,154
Interest expense	(75)	(90)	(200)	(269)	(1,351)
Change in fair value of warrant liability	(154)		(27)	(149)	(454)
Other expense	(1,180)		(1,180)		(1,182)
Loss before tax benefit	(11,643)	(10,303)	(28,555)	(29,394)	(113,756)
Income tax benefit					695
Net loss	(11,643)	(10,303)	(28,555)	(29,394)	(113,061)
Deemed dividend			(19,424)		(19,424)
Preferred stock accretion	(41)		(122)	(351)	(802)
Net loss attributable to common stockholders	\$ (11,684)	\$ (10,303)	\$ (48,101)	\$ (29,745)	\$ (133,287)
Net loss attributable to common stockholders per common share basic and diluted	\$ (15.01)	\$ (0.46)	\$ (70.72)	\$ (2.92)	
Weighted-average common shares outstanding basic and diluted	778,561	22,291,832	680,114	10,177,449	



*See accompanying notes to consolidated financial statements*

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

	<b>Nine Months Ended September 30,</b>		<b>Period from February 4, 2002 (inception) to September 30, 2007</b>
	<b>2006</b>	<b>2007</b>	<b>2007</b>
<b>Operating activities</b>			
Net loss	\$ (28,555)	\$ (29,394)	\$ (113,061)
Adjustments to reconcile net loss to net cash used in operating activities:			
Non-cash interest expense			525
Depreciation and amortization	661	924	2,481
Amortization of non-cash compensation			522
Stock-based compensation employees	1,773	2,712	5,528
Stock-based compensation non-employees	462	162	853
Stock-based license payments			1,220
Change in fair value of warrant liability	26	149	454
Impairment of leasehold improvements			1,030
Non-cash charge for in-process research and development			418
Beneficial conversion feature related to bridge financing			135
Changes in operating assets and liabilities:			
Prepaid expenses and other current assets	177	(924)	(1,245)
Other non-current assets	266		(291)
Accounts payable and accrued expenses	600	(849)	8,050
Net cash used in operating activities	(24,590)	(27,220)	(93,381)
<b>Investing activities</b>			
Sale and redemption of marketable securities	25,055	70,404	113,100
Purchases of marketable securities	(31,583)	(127,101)	(212,472)
Purchases of property and equipment	(1,587)	(479)	(7,422)
Net cash used in investing activities	(8,115)	(57,176)	(106,794)
<b>Financing activities</b>			
Proceeds from the issuance of preferred stock, net of issuance costs	63,447	24,053	143,022
Proceeds from the issuance of common stock, net of issuance costs		68,146	68,146
Proceeds from the issuance of convertible notes			5,000
Payments of capital lease obligations	(542)	(1,016)	(2,493)
Proceeds from exercise of stock options	158	375	558
Proceeds from exercise of warrants (common and preferred)	76	98	264
Proceeds from capital asset financing arrangement	2,199	546	5,611

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Net cash provided by financing activities	65,338	92,202	220,108
Net increase in cash and cash equivalents	32,633	7,806	19,933
Cash and cash equivalents at beginning of period	6,449	12,127	
Cash and cash equivalents at end of period	\$ 39,082	\$ 19,933	\$ 19,933
<b>Supplemental disclosures of cash flow information</b>			
Cash paid during the period for interest	\$ 200	\$ 268	\$ 1,056
<b>Non-cash activities</b>			
Conversion of notes payable to series B redeemable convertible preferred stock			\$ 5,000
Conversion of preferred stock to common stock	\$	\$ 148,591	\$ 148,591
Accretion of redeemable convertible preferred stock	\$ 122	\$ 351	\$ 802
Beneficial conversion feature related to issuance of the second tranche of Series C redeemable convertible preferred stock	\$ 19,424	\$	\$ 19,424

*See accompanying notes to consolidated financial statements*

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**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 for the purpose of creating a premier drug development company at the forefront of therapy for human genetic diseases initially based on intellectual property in-licensed from Mount Sinai School of Medicine. The Company's activities since inception have consisted principally of raising capital, establishing facilities, and performing research and development, including clinical trials. Accordingly, the Company is considered to be in the development stage.

The Company has an accumulated deficit of approximately \$113.1 million at September 30, 2007 and anticipates incurring losses through the year 2007 and beyond. The Company has not yet generated revenues and has been able to fund its operating losses to date through its initial public offering of common stock, its issuance of redeemable convertible preferred stock, its issuance of convertible notes, and other financing arrangements. Successful transition to attaining profitable operations is dependent upon achieving a level of revenues adequate to support the Company's cost structure, and until that time, the Company will have to continue to raise additional debt or equity financing. If adequate funds are not available, the Company may have to substantially reduce or eliminate expenditures relating to the development of its product candidates or cease operations.

Management believes that the Company's current cash position is sufficient to cover its cash flow requirements until at least September 30, 2008.

***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 notes thereto should be read in conjunction with the audited financial statements for the year ended December 31, 2006 included in the Company's Registration Statement on Form S-1, as amended, which was declared effective by the Securities and Exchange Commission ( SEC ) on May 30, 2007.

***Initial Public Offering***

In June 2007, the Company closed its initial public offering of 5,000,000 shares of its common stock at a public offering price of \$15.00 per share. Net proceeds to the Company were approximately \$68.1 million, after deducting underwriting discounts, commissions and offering expenses totaling approximately \$6.9 million.

In connection with the initial public offering, the outstanding shares of series A redeemable convertible preferred stock were converted into 444,443 shares of common stock, the outstanding shares of series B redeemable convertible preferred stock were converted into 4,877,056 shares of common stock, the outstanding shares of series C redeemable convertible preferred stock were converted into 5,820,020 shares of common stock and the outstanding shares of series D redeemable convertible preferred stock were converted into 4,930,405 shares of common stock. In connection with the initial public offering, the outstanding warrants to purchase series B redeemable convertible preferred stock were automatically exercised and the shares of series B redeemable convertible preferred stock automatically converted into 40,797 shares of the common stock. As a result, the Company no longer recognizes accretion expense for preferred stock or non-operating income or expense for changes in the fair value of the warrant liability.

**Table of Contents*****Income Taxes***

The Company uses the asset and liability method to account for income taxes, including the recognition of deferred tax assets and deferred tax liabilities for the anticipated future tax consequences attributable to differences between financial statements amounts and their respective tax bases. The Company reviews its deferred tax assets for recovery. A valuation allowance is established when the Company believes that it is more likely than not that its deferred tax assets will not be realized. Changes in valuation allowances from period to period are included in the Company's tax provision in the period of change.

In June 2006, the Financial Accounting Standards Board ( FASB ) issued Interpretation No. 48, *Accounting for Uncertainty in Income Taxes* an Interpretation of FASB Statement No. 109 ( FIN 48 ) to create a single model to address accounting for uncertainty in tax positions. FIN 48 clarifies the accounting for income taxes, by prescribing a minimum recognition threshold a tax position is required to meet before being recognized in the financial statements. FIN 48 also provides guidance on de-recognition, measurement, and classification of amounts relating to uncertain tax positions, accounting for and disclosure of interest and penalties, accounting in interim periods, disclosures and transition relating to the adoption of the new accounting standard. FIN 48 is effective for fiscal years beginning after December 15, 2006. The Company adopted FIN 48 as of January 1, 2007, as required and determined that the adoption of FIN 48 did not have a material impact on the Company's financial position and results of operations. The Company did not recognize interest or penalties related to income tax during the three and nine months ended September 30, 2006 or 2007 and did not accrue for interest or penalties as of September 30, 2007 or December 31, 2006. The Company does not have an accrual for uncertain tax positions as of September 30, 2007. Tax returns for all years 2002 and thereafter are subject to future examination by tax authorities.

***Investment in Marketable Securities***

Marketable securities consist of fixed income investments with a maturity of greater than three months and other highly liquid investments that can be readily purchased or sold using established markets. In accordance with FASB Statement of Financial Accounting Standards ( SFAS ) No. 115, *Accounting for Certain Investments in Debt and Equity Securities* ( SFAS No. 115 ), these investments are classified as available-for-sale and 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/(deficiency). 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. No other than temporary impairment charges have been recorded.

***New Accounting Standards***

In June 2007, the Emerging Issues Task Force ( EITF ) of the FASB reached a consensus on Issue No. 07-3, *Accounting for Nonrefundable Advance Payments for Goods or Services Received for Use in Future Research and Development Activities* (EITF 07-3). EITF 07-3 requires that non-refundable advance payments for goods or services that will be used or rendered for future research and development activities should be deferred and capitalized. As the related goods are delivered or the services are performed, or when the goods or services are no longer expected to be provided, the deferred amounts would be recognized as an expense. This Issue is effective for financial statements issued for fiscal years beginning after December 15, 2007. The Company is currently evaluating the impact, if any, that the adoption of EITF 07-3 will have on the Company's results of operations or financial condition.

In February 2007, the FASB issued SFAS No. 159, *The Fair Value Option for Financial Assets and Financial Liabilities Including an amendment of FASB Statement No. 115*, or SFAS No. 159, which is effective for fiscal years beginning after November 15, 2007. SFAS No. 159 permits the Company to choose to measure many financial instruments and certain other items at fair value. The objective is to improve financial reporting by providing entities with the opportunity to mitigate volatility in reported earnings caused by measuring related assets and liabilities differently without having to apply complex hedge accounting provisions. SFAS No. 159 is expected to expand the use of fair value measurement, which is consistent with the FASB's long-term measurement objectives for accounting for financial instruments. SFAS No. 159 is effective for fiscal year 2008 but early adoption is permitted.



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The Company is currently evaluating the impact, if any, that the adoption of SFAS No. 159 will have on the Company's financial statements.

In September 2006, the FASB issued SFAS No. 157, *Fair Value Measures* (SFAS No. 157). SFAS No. 157 defines fair value, establishes a framework for measuring fair value and enhances disclosures about fair value measures required under other accounting pronouncements, but does not change existing guidance as to whether or not an instrument is carried at fair value. SFAS No. 157 is effective as of the beginning of the Company's 2008 fiscal year. The Company does not expect this will have a significant impact on the financial statements of the Company.

**2. Stock-Based Compensation**

During the three and nine months ended September 30, 2007, the Company recorded compensation expense of approximately \$1.1 million and \$2.9 million, respectively. The stock-based compensation expense had no impact on the Company's cash flows from operations and financing activities. As of September 30, 2007, the total unrecognized compensation cost related to non-vested stock options granted was \$12.2 million and is expected to be recognized over a weighted average period of 2.0 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 September 30, 2006</b>	<b>Nine Months Ended September 30, 2006</b>	<b>Three Months Ended September 30, 2007</b>	<b>Nine Months Ended September 30, 2007</b>
Expected stock price volatility	80.0%	73.9%	78.9%	78.2%
Risk free interest rate	4.8%	4.7%	4.7%	4.6%
Expected life of options (years)	6.25	6.25	6.25	6.25
Expected annual dividend per share	\$ 0.00	\$ 0.00	\$ 0.00	\$ 0.00

A summary of option activities related to the Company's stock options for the nine months ended September 30, 2007 is as follows:

	<b>Number of Shares (in thousands)</b>	<b>Weighted Average Exercise Price</b>	<b>Weighted Average Remaining Contractual Life</b>	<b>Aggregate Intrinsic Value  (in millions)</b>
Balance at December 31, 2006	1,868.5	\$ 4.27		
Options granted	942.7	\$13.31		
Options exercised	(269.4)	\$ 1.52		
Options forfeited	(128.5)	\$ 8.57		
Balance at September 30, 2007	2,413.3	\$ 7.86	8.4 years	\$21.3
Vested and unvested expected to vest, September 30, 2007	2,174.2	\$ 7.61	8.3 years	\$19.8
Exercisable at September 30, 2007	652.4	\$ 4.64	7.3 years	\$ 7.9

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

The Company calculates net loss per share in accordance with SFAS No. 128, *Earnings Per Share*. The Company has determined that its series A, B, C, and D redeemable convertible preferred stock represented participating securities in accordance with EITF 03-6 *Participating Securities and the Two Class Method under FASB Statement No. 128*. However, because the Company operates at a loss, and losses are not allocated to the redeemable convertible preferred stock, the two-class method does not affect the Company's calculation of earnings

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per share. The Company has a net loss for all periods presented; accordingly, the inclusion of common stock options and warrants would be anti-dilutive. Therefore, the weighted average shares used to calculate both basic and diluted earnings per share are the same.

(In thousands, except per share amounts)	<b>Three Months Ended September 30,</b>		<b>Nine Months Ended September 30,</b>	
	<b>2006</b>	<b>2007</b>	<b>2006</b>	<b>2007</b>
<b>Statement of Operations</b>				
Net loss attributable to common stockholders	\$(11,684)	\$(10,303)	\$(48,101)	\$(29,745)
Net loss attributable to common stockholders per common share basic and diluted	\$ (15.01)	\$ (0.46)	\$ (70.72)	\$ (2.92)

**4. Comprehensive Loss**

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

	<b>Three Months Ended September 30,</b>		<b>Nine Months Ended September 30,</b>	
	<b>2006</b>	<b>2007</b>	<b>2006</b>	<b>2007</b>
Net loss	\$(11,643)	\$(10,303)	\$(28,555)	\$(29,394)
Change in unrealized net gain on marketable securities	16	107	38	205
Comprehensive loss	\$(11,627)	\$(10,196)	\$(28,517)	\$(29,189)

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

**5. Capital Structure*****Redeemable Convertible Preferred Stock***

In March 2007, the Company issued an additional 1,976,527 shares of its series D redeemable convertible preferred stock for gross proceeds of \$24.1 million.

On June 5, 2007, all outstanding shares of the Company's series A redeemable convertible preferred stock, series B redeemable convertible preferred stock, series C redeemable convertible preferred stock, and series D redeemable convertible preferred stock were automatically converted into shares of common stock at the closing of the Company's initial public offering.

	<b>Series A</b>		<b>Series B</b>		<b>Series C</b>		<b>Series D</b>	
	<b>Shares</b>	<b>Amount (in thousands)</b>	<b>Shares</b>	<b>Amount (in thousands)</b>	<b>Shares</b>	<b>Amount (in thousands)</b>	<b>Shares</b>	<b>Amount (in thousands)</b>
Balance at December 31, 2006	444,443	\$ 2,476	4,877,056	\$ 30,868	5,820,020	\$ 54,869	2,953,878	\$ 35,876
Issuance of Series D at \$12.15 per share							1,976,527	24,053
Series B warrant exercise			40,797	98				
Accretion to redemption value		24		126		130		71

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Conversion of preferred stock to common	(444,443)	(2,500)	(4,917,853)	(31,092)	(5,820,020)	(54,999)	(4,930,405)	(60,000)
<b>Balance at September 30, 2007</b>		\$		\$		\$		\$

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

**Overview**

We are a clinical-stage biopharmaceutical company focused on the discovery, development and commercialization of novel small molecule, orally-administered drugs, known as pharmacological chaperones, for the treatment of a range of human genetic diseases. Certain human diseases result from mutations in specific genes that, in many cases, lead to the production of proteins with reduced stability. Proteins with such mutations may not fold into their correct three-dimensional shape and are generally referred to as misfolded proteins. Misfolded proteins are often recognized by cells as having defects and, as a result, may be eliminated prior to reaching their intended location in the cell. The reduced biological activity of these proteins leads to impaired cellular function and ultimately to disease. 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, thereby increasing protein activity, improving cellular function and potentially reducing cell stress. We are currently conducting Phase II clinical trials of Amigal for Fabry disease, Phase II clinical trials of Plicera for Gaucher disease, and Phase I clinical trials of AT2220 for Pompe disease.

We have generated significant losses to date and expect to continue to generate losses as we continue the clinical development of Amigal, Plicera, and AT2220. From our inception in February 2002 through September 30, 2007, we have accumulated a deficit of \$113.1 million. Because we do not generate revenue from any of our product candidates, our losses will continue as we conduct our research and development activities. These activities are budgeted to expand over time and will require further resources if we are to be successful. As a result, our operating losses are likely to be substantial over the next several years. We will need to obtain additional funds to further develop our research and development programs and product candidates.

In June 2007, we completed our initial public offering 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 after deducting underwriting discounts, commissions and offering expenses payable by us. In connection with the closing of the initial public offering, all of the Company's shares of redeemable convertible preferred stock outstanding at the time of the offering were automatically converted into 16,112,721 shares of common stock.

**Financial Operations Overview**

***Revenue***

We have not generated any revenue since our inception. To date, we have funded our operations primarily through the sale of equity securities and equipment financings through capital leases. If our development efforts result in clinical success, regulatory approval and successful commercialization of any of our product candidates, we could generate revenue from sales of our product candidates.

***Research and Development Expenses***

We expect our research and development expense to increase as we continue to develop our product candidates. Research and development expense consists of:

internal costs associated with our research activities;

payments we make to third party contract research organizations, contract manufacturers, investigative sites, and consultants;

technology and intellectual property 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

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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 do not believe that allocating internal costs on the basis of estimates of time spent by our employees would accurately represent the actual costs of a project. We do, however, record and maintain information regarding external, out-of-pocket research and development expenses on a project specific basis.

We expense research and development costs as incurred, including payments made to date under our license agreements. We believe that significant investment in product development is a competitive necessity and plan to continue these investments in order to realize the potential of our product candidates. From our inception in February 2002 through September 30, 2007, we have incurred research and development expense in the aggregate of \$80.2 million, including stock-based compensation expense of \$3.4 million.

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

<b>Product Candidate</b>	<b>Three Months Ended</b>		<b>Nine Months Ended</b>		<b>Period from</b>
	<b>September 30, 2006</b>	<b>September 30, 2007</b>	<b>September 30, 2006</b>	<b>September 30, 2007</b>	<b>February 4, 2002</b>
					<b>(inception) to September 30, 2007</b>
Third party direct project expenses					
Amigal (Fabry Disease Phase II)	\$ 595	\$ 1,667	\$ 2,181	\$ 4,073	\$ 20,455
Plicera (Gaucher Disease Phase II)	1,861	978	5,669	3,548	15,278
AT2220 (Pompe Disease Phase I)	497	932	1,610	2,565	7,328
<b>Total third party direct project expenses</b>	<b>2,953</b>	<b>3,577</b>	<b>9,460</b>	<b>10,186</b>	<b>43,061</b>
Other project costs <sup>(1)</sup>					
Personnel costs	2,018	2,409	5,483	7,009	21,719
Other costs <sup>(2)</sup>	1,754	1,551	4,501	4,209	15,428
<b>Total other project costs</b>	<b>3,772</b>	<b>3,960</b>	<b>9,984</b>	<b>11,218</b>	<b>37,147</b>
<b>Total research and development costs</b>	<b>\$ 6,725</b>	<b>\$ 7,537</b>	<b>\$ 19,444</b>	<b>\$ 21,404</b>	<b>\$ 80,208</b>

(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.

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, or the period, if any, in which material net cash inflows may commence from Amigal, Plicera, AT2220 or any of our other preclinical product candidates. This uncertainty is due to the numerous risks and uncertainties associated with the duration and cost of clinical trials, which vary significantly over the life of a project as a result of differences arising 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; and

- the results of our clinical trials.

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

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timing associated with the development 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, 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. We expect that our general and administrative expenses will increase as we add personnel and are subject to the reporting obligations applicable to public companies. From our inception in February 2002 through September 30, 2007, we spent \$32.8 million, including stock-based compensation expense of \$4.3 million, on general and administrative expense.

### ***Interest Income and Interest Expense***

Interest income consists of interest earned on our cash and cash equivalents and marketable securities. Interest expense consists of interest incurred on our capital lease facility.

### **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 September 30, 2007 to the items that we disclosed as our significant accounting policies and estimates described in Note 2 to our audited consolidated financial statements for the year ended December 31, 2006 in our Registration Statement on Form S-1 (File No. 333-141700), we believe that the following accounting policies are the most critical to aid you in fully understanding and evaluating our financial condition and results of operations.

### ***Accrued Expenses***

As part of the process of preparing our financial statements, we are required to estimate accrued expenses. This process involves identifying services that have been performed on our behalf and estimating the level of service performed and the associated cost incurred for the service when we have not yet been invoiced or otherwise notified of actual cost. 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.





**Table of Contents****Stock-Based Compensation**

Effective January 1, 2006, we adopted SFAS No. 123(R), *Share-Based Payment*, using the fair value method, which requires a public entity to measure the cost of employee services received in exchange for an award of equity instruments based on the grant-date fair value of the award. Our financial statements as of and for the three and nine months ended September 30, 2006 and 2007 reflect the impact of SFAS No. 123(R). 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 requisite service period of the last separately vesting portion of each award. 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. The average expected life was determined using the SEC shortcut approach as described in Staff Accounting Bulletin, *Disclosure about Fair Value of Financial Instruments*, which is 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.

We account for equity instruments issued to non-employees in accordance with the provisions of Emerging Issues Task Force No. 96-18, *Accounting for Equity Instruments That Are Issued to Other Than Employees for Acquiring, or in Conjunction with Selling, Goods or Services*. The equity instruments, consisting of stock options, are valued using the Black-Scholes-Merton valuation model. The measurement of stock-based compensation is subject to periodic adjustments as the underlying equity instruments vest.

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

We calculated net loss per share in accordance with SFAS No. 128, *Earnings Per Share*. We have determined that the series A, B, C, and D redeemable convertible preferred stock represented participating securities in accordance with Emerging Issue Task Force, or EITF, 03-6 *Participating Securities and the Two Class Method under FASB Statement No. 128*. However, because we operate at a loss, and losses are not allocated to the redeemable convertible preferred stock, the two class method does not affect our calculation of earnings per share. 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)	Three Months Ended September 30,		Nine Months Ended September 30,	
	2006	2007	2006	2007
<b>Historical</b>				
Numerator:				
Net loss	\$ (11,643)	\$ (10,303)	\$ (28,555)	\$ (29,394)
Deemed dividend			(19,424)	
Accretion of redeemable convertible preferred stock	(41)		(122)	(351)
Net loss attributable to common stockholders	\$ (11,684)	\$ (10,303)	\$ (48,101)	\$ (29,745)
Denominator:				
Weighted average common shares outstanding basic and diluted	778,561	22,291,832	680,114	10,177,449

Dilutive common stock equivalents would include the dilutive effect of convertible securities, common stock options and warrants for common stock equivalents. Potentially dilutive common stock equivalents totaled

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approximately 17,334,242 and 24,791,239 for the three and nine months ended September 30, 2006 and 2007, 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 September 30, 2007 Compared to Three Months Ended September 30, 2006***

*Research and Development Expense.* Research and development expense was \$7.5 million for the three months ended September 30, 2007 representing an increase of \$0.8 million or 12% from \$6.7 million for the three months ended September 30, 2006. The variance was primarily attributable to higher personnel costs associated with headcount growth, partially offset by lower third party direct project costs.

*General and Administrative Expense.* General and administrative expense was \$4.0 million for the three months ended September 30, 2007, an increase of \$0.2 million or 5% from \$3.8 million from the three months ended September 30, 2006. The variance was primarily attributable to higher personnel costs associated with headcount growth, partially offset by lower consulting and professional fees.

*Interest Income and Interest Expense.* Interest income was \$1.6 million for the three months ended September 30, 2007, compared to \$0.5 million for the three months ended September 30, 2006. The increase of \$1.1 million or 220% was due to higher cash balances as a result of the issuance of the second tranche of our series D redeemable convertible preferred stock and our initial public offering. Interest expense was \$0.1 million for the three months ended September 30, 2007 and 2006.

***Nine Months Ended September 30, 2007 Compared to Nine Months Ended September 30, 2006***

*Research and Development Expense.* Research and development expense was \$21.4 million for the nine months ended September 30, 2007, representing an increase of \$2.0 million or 10% from \$19.4 million for the nine months ended September 30, 2006. The increase was primarily attributable to increases in third party direct project costs of \$0.7 million and an increase in personnel costs of \$1.5 million associated with headcount growth.

*General and Administrative Expense.* General and administrative expense was \$10.0 million for the nine months ended September 30, 2007, an increase of \$1.8 million or 22% from \$8.2 million for the nine months ended September 30, 2006. The increase resulted primarily from an increase in personnel costs attributable to headcount growth.

*Interest Income and Interest Expense.* Interest income was \$3.3 million for the nine months ended September 30, 2007, compared to \$1.2 million for the nine months ended September 30, 2006. The increase of \$2.1 million or 175% was due to higher cash balances as a result of the issuance of the second tranche of series D redeemable convertible preferred stock and our initial public offering. Interest expense was \$0.3 million for the nine months ended September 30, 2007, compared to \$0.2 million for the nine months ended September 30, 2006. The increase in interest expense resulted from additional capital lease borrowings during the second quarter of 2007.

**Table of Contents****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 and \$75.0 million of gross proceeds from our initial public offering in June 2007. The following table summarizes our funding sources as of September 30, 2007:

<b>Issue</b>	<b>Year</b>	<b>No. Shares</b>	<b>Approximate Amount <sup>(1)</sup> (in thousands)</b>
Series A Redeemable Convertible Preferred Stock	2002 2004, 2005, 2006,	444,443	\$ 2,500
Series B Redeemable Convertible Preferred Stock	2007 2005,	4,917,853	31,189
Series C Redeemable Convertible Preferred Stock	2006 2006,	5,820,020	54,999
Series D Redeemable Convertible Preferred Stock	2007	4,930,405	60,000
Common Stock	2007	5,000,000	75,000
		21,112,721	\$ 223,688

(1) Represents  
gross proceeds

As of September 30, 2007, we had cash and cash equivalents and marketable securities of \$119.4 million. We hold our cash and investment balances in a variety of interest-bearing instruments, including obligations of U.S. government agencies and money market accounts. We invest cash in excess of our immediate requirements with regard to liquidity and capital preservation. Wherever possible, we seek to minimize the potential effects of concentration and degrees of risk.

Also, we maintain cash balances with financial institutions in excess of insured limits. We do not anticipate any losses with respect to such cash balances.

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

Net cash used in operations for the nine months ended September 30, 2006 was \$24.6 million. The net loss for the nine months ended September 30, 2006 of \$28.6 million was offset primarily by non-cash charges for depreciation and amortization of \$0.7 million, stock-based compensation of \$2.2 million and changes in operating assets and liabilities of \$1.0 million.

Net cash used in operations for the nine months ended September 30, 2007 was \$27.2 million due to the net loss for the nine months ended September 30, 2007 of \$29.4 million and the change in operating assets and liabilities of \$1.7 million, offset primarily by non-cash charges for depreciation and amortization of \$0.9 million, stock-based compensation of \$2.9 million and the change in fair value of warrant liability of \$0.1 million.

***Net Cash Used in Investing Activities***

Net cash provided by investing activities for the nine months ended September 30, 2006 was \$8.1 million. Net cash provided by investing activities reflects \$31.6 million cash used for the purchase of marketable securities and \$1.6 million for the acquisition of property and equipment, offset by \$25.1 million of cash provided by the sale and

redemption of marketable securities.

Net cash used in investing activities for the nine months ended September 30, 2007 was \$57.2 million. Net cash used in investing activities reflects \$127.1 million for the purchase of marketable securities and \$0.5 million for the acquisition of property and equipment, partially offset by \$70.4 million for the sale and redemption of marketable securities.

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***Net Cash Provided by Financing Activities***

Net cash provided by financing activities for the nine months ended September 30, 2006 was \$65.3 million. Net cash provided by financing activities mainly reflects \$27.5 million of proceeds from the issuance of our series C redeemable convertible preferred stock, \$35.9 million of proceeds from the issuance of our series D redeemable convertible preferred stock, \$0.2 million of proceeds from the exercise of warrants and stock options; and \$2.2 million of proceeds from our capital asset financing arrangement, which are offset by \$0.5 million of payments of capital lease obligations.

Net cash provided by financing activities for the nine months ended September 30, 2007 was \$92.2 million, consisting primarily of \$24.1 million from the issuance of series D redeemable convertible preferred stock, \$68.1 million from the issuance of common stock, \$0.5 million from asset financing arrangements, and \$0.5 million proceeds from exercise of stock options and warrants offset by payments of equipment debt financing obligations of \$1.0 million.

***Funding Requirements***

We expect to incur losses from operations for the foreseeable future. We expect to incur increasing research and development expenses, including expenses related to the hiring of personnel and additional clinical trials. We expect that our general and administrative expenses will also increase as we expand our finance and administrative staff, add infrastructure, and incur additional costs related to being a public company, including directors and officers insurance, investor relations programs, and increased professional fees. Our future capital requirements will depend on a number of factors, including the continued progress of our research and development of products, the progress and results of our clinical trials, the duration and cost of discovery and preclinical development and laboratory testing and clinical trials for our product candidates, the timing and outcome of regulatory review of our product candidates, the number and development requirements of other product candidates that we pursue, the costs involved in preparing, filing, prosecuting, maintaining, defending, and enforcing patent claims and other intellectual property rights, the acquisition of licenses to new products or compounds, the status of competitive products, the availability of financing, our success in developing markets for our product candidates and the costs of commercialization activities, including product marketing, sales and distribution.

We believe that the net proceeds from our initial public offering in June 2007 together with our existing cash and cash equivalents and short-term investments, will be sufficient to enable us to fund our operating expenses and capital expenditure requirements at least until early 2010. We have based this estimate on assumptions that may prove to be wrong, and we could use our available capital resources sooner than we currently expect. Because of the numerous risks and uncertainties associated with the development and commercialization of our product candidates, we are unable to estimate the amounts of increased capital outlays and operating expenditures associated with our current and anticipated clinical trials.

We do not anticipate that we will generate product revenue for at least the next several years. In the absence of additional funding, we expect our continuing operating losses to result in increases in our cash used in operations over the next several quarters and years.

We will need to finance our future cash needs through public or private equity offerings, debt financings or corporate collaboration and licensing arrangements. We do not currently have any commitments for future external funding. We may need to raise additional funds more quickly if one or more of our assumptions prove to be incorrect or if we choose to expand our product development efforts more rapidly than we presently anticipate, and we may decide to raise additional funds even before we need them if the conditions for raising capital are favorable. We may seek to sell additional equity or debt securities or obtain a bank credit facility. The sale of additional equity or debt securities, if convertible, could result in dilution to our stockholders. The incurrence of indebtedness would result in increased fixed obligations and could also result in covenants that would restrict our operations.

Additional equity or debt financing, grants, or corporate collaboration and licensing arrangements may not be available on acceptable terms, if at all. If adequate funds are not available, we may be required to delay, reduce the scope of or eliminate our research and development programs, reduce our planned commercialization efforts or

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obtain funds through arrangements with collaborators or others that may require us to relinquish rights to certain product candidates that we might otherwise seek to develop or commercialize independently.

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

We have acquired rights to develop and commercialize our product candidates through licenses granted by various parties. Two of these agreements contain milestone payments that are due with respect to Plicera only if certain specified pre-commercialization events occur. Amigal and AT2220 do not trigger such milestone payments. 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. In general, potential milestone payments for Plicera may or may not be triggered under these licenses, and may vary in size, depending on a number of variables, almost all of which are currently uncertain.

The events that trigger these payments include:

completion of Phase II clinical trials;

commencement of Phase III clinical trials;

submission of an NDA to the FDA or foreign equivalents; and

receipt of marketing approval from the FDA or foreign equivalents.

Under our license agreements, if we owe royalties on net sales for one of our products to more than one of the above licensors, 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. We expect to pay royalties to all three licensors with respect to Plicera. To date, we have not made any royalty payments on sales of our products and believe we are several years away from selling any products that would require us to make any such royalty payments. 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**

The primary objective of our investment activities is to preserve our capital to fund operations. We also seek to maximize income from our investments without assuming significant risk. To achieve our objectives, we maintain a portfolio of cash equivalents and investments in a variety of securities of high credit quality. As of September 30, 2007, we had cash and cash equivalents and investments in marketable securities of \$119.4 million. A portion of our investments may be subject to interest rate risk and could fall in value if market interest rates increase. However, because our investments are short-term in duration, we believe that our exposure to interest rate risk is not significant and a 1% movement in market interest rates would not have a significant impact on the total value of our portfolio. We actively monitor changes in interest rates.

**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) was carried out under the supervision of our Chief Executive Officer and Chief Financial Officer, with the participation of our management. Based on that evaluation, the Chief Executive Officer and the Chief 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 Chief Executive Officer and Chief Financial Officer, as appropriate to allow timely decisions regarding required disclosure.

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During the fiscal quarter covered by this report, there has been no change in our internal control over financial reporting that occurred during the fiscal quarter that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

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**PART II OTHER INFORMATION**

**ITEM 1. LEGAL PROCEEDINGS**

We are not a party to any material legal proceedings.

**ITEM 1A RISK FACTORS**

*Investing in our common stock involves a high degree of risk. You should carefully consider the following risk factors, as well as other information in this report, before deciding to invest in shares of our common stock. The occurrence of any of the following risks could harm our business, financial condition, results of operations and/or growth prospects. In that case, the trading price of our common stock could decline, and you may lose all or part of your investment.*

**Risks Related to Our Financial Position and Need for Additional Capital**

**We have incurred significant operating losses since our inception. We currently do not, and since inception never have had, any products available for commercial sale. We expect to incur operating losses for the foreseeable future and may never achieve or maintain profitability.**

Since inception, we have incurred significant operating losses. Our net loss attributable to common stockholders was \$11.7 million and \$10.3 million for the three months ended September 30, 2006 and 2007, respectively and \$48.1 million and \$29.7 million for the nine months ended September 30, 2006 and 2007, respectively. As of September 30, 2007, we had an accumulated deficit of \$113.1 million. To date, we have financed our operations primarily through private placements of our redeemable convertible preferred stock and through proceeds from our initial public offering. We have devoted substantially all of our efforts to research and development, including our preclinical development activities and clinical trials. We have not completed development of any drugs. We expect to continue to incur significant and increasing operating losses for at least the next several years and we are unable to predict the extent of any future losses. We anticipate that our expenses will increase substantially as we:

continue our ongoing Phase II clinical trials of Amigal for the treatment of Fabry disease and potentially conduct later-stage clinical trials of Amigal;

continue our ongoing Phase II clinical trials of Plicera for the treatment of Gaucher disease and potentially conduct later-stage clinical trials of Plicera;

continue our ongoing Phase I clinical trials of AT2220 for the treatment of Pompe disease and potentially conduct later-stage clinical trials of AT2220;

continue the research and development of additional product candidates;

seek regulatory approvals for our product candidates that successfully complete clinical trials;

establish a sales and marketing infrastructure to commercialize products for which we may obtain regulatory approval; and

add operational, financial and management information systems and personnel, including personnel to support our product development efforts and our obligations as a public company.

To become and remain profitable, we must succeed in developing and commercializing drugs with significant market potential. This will require us to be successful in a range of challenging activities, including the discovery of product candidates, successful completion of preclinical testing and clinical trials of our product candidates, obtaining regulatory approval for these product candidates and manufacturing, marketing and selling those products for which we may obtain regulatory approval. We are only in the preliminary stages of these activities. We may never succeed in these activities and may never generate revenues that are large enough to achieve profitability. Even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual



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basis. Our failure to become or remain profitable could depress the market price of our common stock and could impair our ability to raise capital, expand our business, diversify our product offerings or continue our operations. **We will need substantial funding and may be unable to raise capital when needed, which would force us to delay, reduce or eliminate our product development programs or commercialization efforts.**

We expect our research and development expenses to increase in connection with our ongoing activities, particularly as we continue our Phase II clinical trials of Amigal, our Phase II clinical trials of Plicera and our Phase I clinical trials of AT2220, and for any later-stage clinical trials of our product candidates. In addition, subject to obtaining regulatory approval of any of our product candidates, we expect to incur significant commercialization expenses for product sales and marketing, securing commercial quantities of product from our manufacturers and product distribution. We currently have no additional commitments or arrangements for any additional financing to fund the research and development and commercial launch of our product candidates.

We believe that the net proceeds from our initial public offering, together with our existing cash and cash equivalents and marketable securities, will be sufficient to enable us to fund our operating expenses and capital expenditure requirements until at least early 2010. Capital may not be available when needed on terms that are acceptable to us, or at all. If adequate funds are not available to us on a timely basis, we may be required to reduce or eliminate research development programs or commercial efforts.

Our future capital requirements will depend on many factors, including:

the progress and results of our clinical trials of Amigal, Plicera and AT2220;

the scope, progress, results and costs of preclinical development, laboratory testing and clinical trials for our other product candidates;

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.

**Any capital that we obtain may not be on terms favorable to us or our stockholders or may require us to relinquish valuable rights.**

Until such time, if ever, as we generate product revenue to finance our operations, we expect to finance our cash needs through public or private equity offerings and debt financings, corporate collaboration and licensing arrangements and grants from patient advocacy groups, foundations and government agencies. If we raise capital by issuing equity securities, our stockholders will experience dilution. Debt financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends and may include rights that are senior to the holders of our common stock. Any debt financing or additional equity that we raise may contain terms, such as liquidation and other preferences, which are not favorable to us or our stockholders. If we raise capital through collaboration and licensing arrangements with third parties, it may be necessary to relinquish valuable rights to our



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technologies, future revenue streams, research programs or product candidates or to grant licenses on terms that may not be favorable to us or our stockholders.

**Our short operating history may make it difficult to evaluate the success of our business to date and to assess our future viability.**

We are a development stage company. We commenced operations in February 2002. Our operations to date have been limited to organizing and staffing our company, acquiring and developing our technology and undertaking preclinical studies and limited clinical trials of our most advanced product candidates. We have not yet demonstrated our ability to successfully complete large-scale, clinical trials, obtain regulatory approvals, manufacture a commercial-scale product or arrange for a third party to do so on our behalf, or conduct sales and marketing activities necessary for successful product commercialization. Consequently, any predictions about our future success or viability may not be as accurate as they could be if we had a longer operating history.

In addition, as a new business, we may encounter unforeseen expenses, difficulties, complications, delays and other known and unknown factors. If we are successful in obtaining marketing approval for any of our lead product candidates, we will need to transition from a company with a research focus to a company capable of supporting commercial activities. We may not be successful in such a transition.

**Risks Related to the Development and Commercialization of Our Product Candidates**

**We depend heavily on the success of our most advanced product candidates, Amigal, Plicera and AT2220. All of our product candidates are still in either preclinical or clinical development. Clinical trials of our product candidates may not be successful. If we are unable to commercialize Amigal, Plicera or AT2220, or experience significant delays in doing so, our business will be materially harmed.**

We have invested a significant portion of our efforts and financial resources in the development of our most advanced product candidates, Amigal, Plicera and AT2220. Our ability to generate product revenue, which we do not expect will occur for at least the next several years, if ever, will depend heavily on the successful development and commercialization of these product candidates. The successful commercialization of our product candidates will depend on several factors, including the following:

obtaining supplies of Amigal, Plicera and AT2220 for completion of our clinical trials on a timely basis;

successful completion of preclinical studies and clinical trials;

obtaining marketing approvals from the United States Food and Drug Administration, or FDA, and similar regulatory authorities outside the United States;

establishing commercial-scale manufacturing arrangements with third party manufacturers whose manufacturing facilities are operated in compliance with current good manufacturing practice, or cGMP, regulations;

launching commercial sales of the product, whether alone or in collaboration with others;

acceptance of the product by patients, the medical community and third party payors;

competition from other companies and their therapies;

successful protection of our intellectual property rights from competing products in the United States and abroad;  
and

a continued acceptable safety and efficacy profile of our product candidates following approval.

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**If the market opportunities for our product candidates are smaller than we believe they are, then our revenues may be adversely affected and our business may suffer.**

Each of the diseases that our product candidates are being developed to address is relatively rare. Our projections of both the number of people who have these diseases, as well as the subset of people with these diseases who have the potential to benefit from treatment with our product candidates, are based on estimates.

Currently, most reported estimates of the prevalence of these diseases are based on studies of small subsets of the population of specific geographic areas, which are then extrapolated to estimate the prevalence of the diseases in the broader world population. In addition, as new studies are performed the estimated prevalence of these diseases may change. In fact, as a result of some recent studies, we believe that previously reported studies do not accurately account for the prevalence of Fabry disease and that the prevalence of Fabry disease could be many times higher than previously reported. There can be no assurance that the prevalence of Fabry disease, Gaucher disease or Pompe disease in the study populations, particularly in these newer studies, accurately reflects the prevalence of these diseases in the broader world population.

We estimate the number of potential patients in the broader world population who have those diseases and may respond to treatment with our product candidates by further extrapolating estimates of the prevalence of specific types of genetic mutations giving rise to these diseases. For example, we base our estimate of the percentage of Fabry patients who may respond to treatment with Amigal on the frequency of missense and other similar mutations that cause Fabry disease reported in the Human Gene Mutation Database. As a result of recent studies that estimate that the prevalence of Fabry disease could be many times higher than previously reported, we believe that the number of patients diagnosed with Fabry disease will increase and estimate that the number of Fabry patients who may benefit from the use of Amigal is significantly higher than some previously reported estimates of Fabry disease generally. If our estimates of the prevalence of Fabry disease, Gaucher disease or Pompe disease or of the number of patients who may benefit from treatment with our product candidates prove to be incorrect, the market opportunities for our product candidates may be smaller than we believe they are, our prospects for generating revenue may be adversely affected and our business may suffer.

**Initial results from a clinical trial do not ensure that the trial will be successful and success in early stage clinical trials does not ensure success in later-stage clinical trials.**

We will only obtain regulatory approval to commercialize a product candidate if we can demonstrate to the satisfaction of the FDA or the applicable non-United States regulatory authority, in well-designed and conducted clinical trials, that the product candidate is safe and effective and otherwise meets the appropriate standards required for approval for a particular indication. Clinical trials are lengthy, complex and extremely expensive processes with uncertain results. A failure of one or more of our clinical trials may occur at any stage of testing. We have limited experience in conducting and managing the clinical trials necessary to obtain regulatory approvals, including approval by the FDA.

Our efforts to develop all of our product candidates are at an early stage. Success in preclinical testing and early clinical trials does not ensure that later clinical trials will be successful, and initial results from a clinical trial do not necessarily predict final results. For example, results to date in our Phase II clinical trials of Amigal for the treatment of Fabry disease caused by missense mutations are based on enzyme level data from only eleven patients and kidney biopsy data from only three patients. Additional data from these eleven patients and data from additional patients in these trials may be less favorable than the results to date. No definitive conclusions as to the safety or efficacy of any drug candidate can be drawn from such a small number of patients. We cannot be assured that these trials will ultimately be successful.

Patients may not be compliant with their dosing regimen or trial protocols or they may withdraw from the study at any time for any reason. We note that a patient who started dosing in March 2006 in the ongoing Phase II clinical trials for Amigal for the treatment of Fabry disease elected to withdraw from the study in March 2006, shortly after initial dosing. This patient had a history of hypertension and discontinued study treatment due to increased blood pressure, which was reported by the investigator as possibly related to the study drug.

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Even if our early stage clinical trials are successful, we will need to conduct additional clinical trials with larger numbers of patients receiving the drug for longer periods for all of our product candidates before we are able to seek approvals to market and sell these product candidates from the FDA and regulatory authorities outside the United States. In addition, each of our product candidates is based on our pharmacological chaperone technology. To date, we are not aware that any product based on chaperone technology has been approved by the FDA. As a result, we cannot be sure what endpoints the FDA will require us to measure in later-stage clinical trials of our product candidates. We are aware that the currently available enzyme replacement therapy for the treatment of Fabry disease was approved by the FDA based on an endpoint measuring GL-3 levels in a specific type of kidney cell. We cannot be certain that the FDA will permit the use of this endpoint in our Phase III trials of Amigal. If the FDA requires different endpoints than the endpoints we anticipate using, it may be more difficult for us to obtain, or we may be delayed in obtaining, FDA approval of our product candidates. If we are not successful in commercializing any of our lead product candidates, or are significantly delayed in doing so, our business will be materially harmed.

**We have limited experience in conducting and managing the preclinical development activities and clinical trials necessary to obtain regulatory approvals, including approval by the FDA.**

We have limited experience in conducting and managing the preclinical development activities and clinical trials necessary to obtain regulatory approvals, including approval by the FDA. To date, we have only three lead product candidates: Amigal, Plicera and AT2220. We have not obtained regulatory approval nor commercialized any of these or any other product candidates. We are currently conducting Phase II clinical trials for Amigal and Plicera and a Phase I clinical trial for AT2220 but have not yet initiated a Phase III clinical trial, or even completed a Phase II clinical trial, for any of our product candidates. Our limited experience might prevent us from successfully designing or implementing a clinical trial. We have limited experience in conducting and managing the application process necessary to obtain regulatory approvals and we might not be able to demonstrate that our product candidates meet the appropriate standards for regulatory approval. If we are not successful in conducting and managing our preclinical development activities or clinical trials or obtaining regulatory approvals, we might not be able to commercialize our lead product candidates, or might be significantly delayed in doing so, which will materially harm our business.

**We may find it difficult to enroll patients in our clinical trials.**

Each of the diseases that our lead product candidates are intended to treat is relatively rare and we expect only a subset of the patients with these diseases to be eligible for our clinical trials. Given that each of our product candidates is in the early stages of required testing, we may not be able to initiate or continue clinical trials for each or all of our product candidates if we are unable to locate a sufficient number of eligible patients to participate in the clinical trials required by the FDA or other non-United States regulatory agencies. The requirements of our clinical testing mandate that a patient cannot be involved in another clinical trial for the same indication. We are aware that our competitors have ongoing clinical trials for products that are competitive with our product candidates and patients who would otherwise be eligible for our clinical trials may be involved in such testing, rendering them unavailable for testing of our product candidates. Additionally, many patients with Fabry disease, Gaucher disease and Pompe disease may already be receiving existing therapies, such as enzyme replacement therapy, which would render them ineligible for our current clinical trials if they are not willing to stop receiving such therapies. Further, if we are required to include patients in our clinical trials who have never received enzyme replacement therapy, we may experience yet further difficulty and delay enrolling patients in our trials. Our inability to enroll a sufficient number of patients for any of our current or future clinical trials would result in significant delays or may require us to abandon one or more clinical trials altogether.

**If our preclinical studies do not produce positive results, if our clinical trials are delayed or if serious side effects are identified during drug development, we may experience delays, incur additional costs and ultimately be unable to commercialize our product candidates.**

Before obtaining regulatory approval for the sale of our product candidates, we must conduct, at our own expense, extensive preclinical tests to demonstrate the safety of our product candidates in animals, and clinical trials to demonstrate the safety and efficacy of our product candidates in humans. Preclinical and clinical testing is expensive, difficult to design and implement and can take many years to complete. A failure of one or more of our



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preclinical studies or clinical trials can occur at any stage of testing. We may experience numerous unforeseen events during, or as a result of, preclinical testing and the clinical trial process that could delay or prevent our ability to obtain regulatory approval or commercialize our product candidates, including:

our preclinical tests or clinical trials may produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional preclinical testing or clinical trials or we may abandon projects that we expect to be promising;

regulators or institutional review boards may not authorize us to commence a clinical trial or conduct a clinical trial at a prospective trial site;

conditions imposed on us by the FDA or any non-United States regulatory authority regarding the scope or design of our clinical trials or may require us to resubmit our clinical trial protocols to institutional review boards for re-inspection due to changes in the regulatory environment;

the number of patients required for our clinical trials may be larger than we anticipate or participants may drop out of our clinical trials at a higher rate than we anticipate;

our third party contractors or clinical investigators may fail to comply with regulatory requirements or fail to meet their contractual obligations to us in a timely manner;

we might have to suspend or terminate one or more of our clinical trials if we, the regulators or the institutional review boards determine that the participants are being exposed to unacceptable health risks;

regulators or institutional review boards may require that we hold, suspend or terminate clinical research for various reasons, including noncompliance with regulatory requirements;

the cost of our clinical trials may be greater than we anticipate;

the supply or quality of our product candidates or other materials necessary to conduct our clinical trials may be insufficient or inadequate or we may not be able to reach agreements on acceptable terms with prospective clinical research organizations; and

the effects of our product candidates may not be the desired effects or may include undesirable side effects or the product candidates may have other unexpected characteristics.

If we are required to conduct additional clinical trials or other testing of our product candidates beyond those that we currently contemplate, if we are unable to successfully complete our clinical trials or other testing, if the results of these trials or tests are not positive or are only modestly positive or if there are safety concerns, we may:

be delayed in obtaining, or may not be able to obtain, marketing approval for one or more of our product candidates;

obtain approval for indications that are not as broad as intended or entirely different than those indications for which we sought approval; or

have the product removed from the market after obtaining marketing approval.

Our product development costs will also increase if we experience delays in testing or approvals. We do not know whether any preclinical tests or clinical trials will be initiated as planned, will need to be restructured or will be completed on schedule, if at all. Significant preclinical or clinical trial delays also could shorten the patent protection period during which we may have the exclusive right to commercialize our product candidates. Such delays could allow our competitors to bring products to market before we do and impair our ability to commercialize our products

or product candidates.



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**The commercial success of any product candidates that we may develop, including Amigal, Plicera and AT2220, will depend upon the degree of market acceptance by physicians, patients, third party payors and others in the medical community.**

Any products that we bring to the market, including Amigal, Plicera and AT2220, if they receive marketing approval, may not gain market acceptance by physicians, patients, third party payors and others in the medical community. If these products do not achieve an adequate level of acceptance, we may not generate significant product revenue and we may not become profitable. The degree of market acceptance of our product candidates, if approved for commercial sale, will depend on a number of factors, including:

- the prevalence and severity of any side effects, including any limitations or warnings contained in a product's approved labeling;
- the efficacy and potential advantages over alternative treatments;
- the pricing of our product candidates;
- relative convenience and ease of administration;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- the strength of marketing and distribution support and timing of market introduction of competitive products;
- publicity concerning our products or competing products and treatments; and
- sufficient third party insurance coverage or reimbursement.

Even if a potential product displays a favorable efficacy and safety profile in preclinical and clinical trials, market acceptance of the product will not be known until after it is launched. Our efforts to educate the medical community and third party payors on the benefits of our product candidates may require significant resources and may never be successful. Such efforts to educate the marketplace may require more resources than are required by the conventional technologies marketed by our competitors.

**If we are unable to obtain adequate reimbursement from governments or third party payors for any products that we may develop or if we are unable to obtain acceptable prices for those products, our prospects for generating revenue and achieving profitability will suffer.**

Our prospects for generating revenue and achieving profitability will depend heavily upon the availability of adequate reimbursement for the use of our approved product candidates from governmental and other third party payors, both in the United States and in other markets. Reimbursement by a third party payor may depend upon a number of factors, including the third party payor's determination that use of a product is:

- a covered benefit under its health plan;
- safe, effective and medically necessary;
- appropriate for the specific patient;
- cost-effective; and
- neither experimental nor investigational.

Obtaining reimbursement approval for a product from each government or other third party payor is a time consuming and costly process that could require us to provide supporting scientific, clinical and cost effectiveness

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data for the use of our products to each payor. We may not be able to provide data sufficient to gain acceptance with respect to reimbursement or we might need to conduct post-marketing studies in order to demonstrate the cost-effectiveness of any future products to such payors' satisfaction. Such studies might require us to commit a significant amount of management time and financial and other resources. Even when a payor determines that a product is eligible for reimbursement, the payor may impose coverage limitations that preclude payment for some uses that are approved by the FDA or non-United States regulatory authorities. In addition, there is a risk that full reimbursement may not be available for high priced products. Moreover, eligibility for coverage does not imply that any product will be reimbursed in all cases or at a rate that allows us to make a profit or even cover our costs. Interim payments for new products, if applicable, may also not be sufficient to cover our costs and may not be made permanent. A primary trend in the United States healthcare industry and elsewhere is toward cost containment. We expect recent changes in the Medicare program and increasing emphasis on managed care to continue to put pressure on pharmaceutical product pricing. For example, the Medicare Prescription Drug Improvement and Modernization Act of 2003 provides a new Medicare prescription drug benefit that began in 2006 and mandates other reforms. While we cannot predict the full outcome of the implementation of this legislation, it is possible that the new Medicare prescription drug benefit, which will be managed by private health insurers and other managed care organizations, will result in additional government reimbursement for prescription drugs, which may make some prescription drugs more affordable but may further exacerbate industry wide pressure to reduce prescription drug prices. If one or more of our product candidates reaches commercialization, such changes may have a significant impact on our ability to set a price we believe is fair for our products and may affect our ability to generate revenue and achieve or maintain profitability.

**Governments outside the United States tend to impose strict price controls and reimbursement approval policies, which may adversely affect our prospects for generating revenue.**

In some countries, particularly European Union countries, the pricing of prescription pharmaceuticals is subject to governmental control. In these countries, pricing negotiations with governmental authorities can take considerable time (6 to 12 months or longer) after the receipt of marketing approval for a product. To obtain reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost effectiveness of our product candidate to other available therapies. If reimbursement of our products is unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, our prospects for generating revenue, if any, could be adversely affected and our business may suffer.

**If we are unable to establish sales and marketing capabilities or enter into agreements with third parties to market and sell our product candidates, we may be unable to generate product revenue.**

At present, we have no sales or marketing personnel. In order to commercialize any of our product candidates, we must either acquire or internally develop sales, marketing and distribution capabilities, or enter into collaborations with partners to perform these services for us. We may not be able to establish sales and distribution partnerships on acceptable terms or at all, and if we do enter into a distribution arrangement, our success will be dependent upon the performance of our partner.

In the event that we attempt to acquire or develop our own in-house sales, marketing and distribution capabilities, factors that may inhibit our efforts to commercialize our products without strategic partners or licensees include:

- our inability to recruit and retain adequate numbers of effective sales and marketing personnel;

- the inability of sales personnel to obtain access to or persuade adequate numbers of physicians to prescribe our products;

- the lack of complementary products to be offered by our sales personnel, which may put us at a competitive disadvantage against companies with broader product lines;

- unforeseen costs associated with creating our own sales and marketing team or with entering into a partnering agreement with an independent sales and marketing organization; and



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efforts by our competitors to commercialize products at or about the time when our product candidates would be coming to market.

We may co-promote our product candidates in various markets with pharmaceutical and biotechnology companies in instances where we believe that a larger sales and marketing presence will expand the market or accelerate penetration. If we do enter into arrangements with third parties to perform sales and marketing services, our product revenues will be lower than if we directly sold and marketed our products and any revenues received under such arrangements will depend on the skills and efforts of others.

We may not be successful in entering into distribution arrangements and marketing alliances with third parties. Our failure to enter into these arrangements on favorable terms could delay or impair our ability to commercialize our product candidates and could increase our costs of commercialization. Dependence on distribution arrangements and marketing alliances to commercialize our product candidates will subject us to a number of risks, including:

we may not be able to control the amount and timing of resources that our distributors may devote to the commercialization of our product candidates;

our distributors may experience financial difficulties;

business combinations or significant changes in a distributor's business strategy may also adversely affect a distributor's willingness or ability to complete its obligations under any arrangement; and

these arrangements are often terminated or allowed to expire, which could interrupt the marketing and sales of a product and decrease our revenue.

If we are unable to establish adequate sales, marketing and distribution capabilities, whether independently or with third parties, we may not be able to generate product revenue and may not become profitable.

**Product liability lawsuits against us could cause us to incur substantial liabilities and to limit commercialization of any products that we may develop.**

We face an inherent risk of product liability exposure related to the testing of our product candidates in human clinical trials and will face an even greater risk if we commercially sell any products that we may develop and which are approved for sale. We may be exposed to product liability claims and product recalls, including those which may arise from misuse or malfunction of, or design flaws in, such products, whether or not such problems directly relate to the products and services we have provided. If we cannot successfully defend ourselves against claims that our product candidates or products caused injuries, we will incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

decreased demand for any product candidates or products that we may develop;

damage to our reputation;

regulatory investigations that could require costly recalls or product modifications;

withdrawal of clinical trial participants;

costs to defend the related litigation;

substantial monetary awards to trial participants or patients, including awards that substantially exceed our product liability insurance, which we would then be required to pay from other sources, if available, and would damage our ability to obtain liability insurance at reasonable costs, or at all, in the future;

loss of revenue;

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the diversion of management's attention from managing our business; and

the inability to commercialize any products that we may develop.

We have liability insurance policies for our clinical trials in the geographies in which we are conducting trials. The aggregate annual limit of coverage amount under these policies expressed in United States dollars is approximately \$31.4 million, and these policies are also subject to per claim deductibles. The amount of insurance that we currently hold may not be adequate to cover all liabilities that we may incur. Insurance coverage is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost and we may not be able to obtain insurance coverage that will be adequate to satisfy any liability that may arise. On occasion, large judgments have been awarded in class action lawsuits based on drugs that had unanticipated side effects. A successful product liability claim or a series of claims brought against us could cause our stock price to fall and, if judgments exceed our insurance coverage, could decrease our available cash and adversely affect our business.

**We face substantial competition which may result in others discovering, developing or commercializing products before or more successfully than we do.**

The development and commercialization of new drugs is highly competitive and competition is expected to increase. We face competition with respect to our current product candidates and any products we may seek to develop or commercialize in the future from major pharmaceutical companies, specialty pharmaceutical companies and biotechnology companies worldwide. For example, several large pharmaceutical and biotechnology companies currently market and sell products for the treatment of Fabry disease. These products include Genzyme Corporation's Fabrazyme and Shire PLC's Replagal. In addition, Genzyme Corporation and Actelion, Ltd. market and sell Cerezyme and Zavesca, respectively, for the treatment of Gaucher disease, and Genzyme Corporation markets and sells Myozyme for the treatment of Pompe disease. We are also aware of other enzyme replacement and substrate reduction therapies in development by third parties.

Potential competitors also include academic institutions, government agencies and other public and private research organizations that conduct research, seek patent protection and establish collaborative arrangements for research, development, manufacturing and commercialization. Our competitors may develop products that are more effective, safer, more convenient or less costly than any that we are developing or that would render our product candidates obsolete or noncompetitive. Our competitors may also obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for ours. We may also face competition from off-label use of other approved therapies. There can be no assurance that developments by others that will not render our product candidates obsolete or noncompetitive either during the research phase or once the products reach commercialization.

We believe that many competitors, including academic institutions, government agencies, public and private research organizations, large pharmaceutical companies and smaller more focused companies, are attempting to develop therapies for many of our target indications.

Many of our competitors have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals, prosecuting intellectual property rights and marketing approved products than we do. Smaller and other early stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These third parties compete with us in recruiting and retaining qualified scientific and management personnel, establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to or necessary for our programs or advantageous to our business. In addition, if we obtain regulatory approvals for our products, manufacturing efficiency and marketing capabilities are likely to be significant competitive factors. We currently have no commercial manufacturing capability, sales force or marketing infrastructure. Further, many of our competitors have substantial resources and expertise in conducting collaborative arrangements, sourcing in-licensing arrangements and acquiring new business lines or businesses that are greater than our own.

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**Our business activities involve the use of hazardous materials, which require compliance with environmental and occupational safety laws regulating the use of such materials. If we violate these laws, we could be subject to significant fines, liabilities or other adverse consequences.**

Our research and development programs involve the controlled use of hazardous materials, including microbial agents, corrosive, explosive and flammable chemicals and other hazardous compounds in addition to certain biological hazardous waste. Ultimately, the activities of our third party product manufacturers when a product candidate reaches commercialization will also require the use of hazardous materials. Accordingly, we are subject to federal, state and local laws governing the use, handling and disposal of these materials. Although we believe that our safety procedures for handling and disposing of these materials comply in all material respects with the standards prescribed by local, state and federal regulations, we cannot completely eliminate the risk of accidental contamination or injury from these materials. In addition, our collaborators may not comply with these laws. In the event of an accident or failure to comply with environmental laws, we could be held liable for damages that result, and any such liability could exceed our assets and resources or we could be subject to limitations or stoppages related to our use of these materials which may lead to an interruption of our business operations or those of our third party contractors. While we believe that our existing insurance coverage is generally adequate for our normal handling of these hazardous materials, it may not be sufficient to cover pollution conditions or other extraordinary or unanticipated events. Furthermore, an accident could damage or force us to shut down our operations. Changes in environmental laws may impose costly compliance requirements on us or otherwise subject us to future liabilities and additional laws relating to the management, handling, generation, manufacture, transportation, storage, use and disposal of materials used in or generated by the manufacture of our products or related to our clinical trials. In addition, we cannot predict the effect that these potential requirements may have on us, our suppliers and contractors or our customers.

**Risks Related to Our Dependence on Third Parties**

**Use of third parties to manufacture our product candidates may increase the risk that we will not have sufficient quantities of our product candidates or such quantities at an acceptable cost, and clinical development and commercialization of our product candidates could be delayed, prevented or impaired.**

We do not own or operate manufacturing facilities for clinical or commercial production of our product candidates. We have limited personnel with experience in drug manufacturing and we lack the resources and the capabilities to manufacture any of our product candidates on a clinical or commercial scale. We currently outsource all manufacturing and packaging of our preclinical and clinical product candidates and products to third parties. The manufacture of pharmaceutical products requires significant expertise and capital investment, including the development of advanced manufacturing techniques and process controls. Manufacturers of pharmaceutical products often encounter difficulties in production, particularly in scaling up initial production. These problems include difficulties with production costs and yields and quality control, including stability of the product candidate.

We do not currently have any agreements with third party manufacturers for the long-term commercial supply of any of our product candidates. We may be unable to enter into agreements for commercial supply with third party manufacturers, or may be unable to do so on acceptable terms. Even if we enter into these agreements, the manufacturers of each product candidate will be single source suppliers to us for a significant period of time.

Reliance on third party manufacturers entails risks, to which we would not be subject if we manufactured product candidates or products ourselves, including:

reliance on the third party for regulatory compliance and quality assurance;

limitations on supply availability resulting from capacity and scheduling constraints of the third parties;

impact on our reputation in the marketplace if manufacturers of our products, once commercialized, fail to meet the demands of our customers;

the possible breach of the manufacturing agreement by the third party because of factors beyond our control;

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and

the possible termination or non-renewal of the agreement by the third party, based on its own business priorities, at a time that is costly or inconvenient for us.

The failure of any of our contract manufacturers to maintain high manufacturing standards could result in injury or death of clinical trial participants or patients using products. Such failure could also result in product liability claims, product recalls, product seizures or withdrawals, delays or failures in testing or delivery, cost overruns or other problems that could seriously harm our business or profitability.

Our contract manufacturers will be required to adhere to FDA regulations setting forth current good manufacturing processes, or cGMP. These regulations cover all aspects of the manufacturing, testing, quality control and recordkeeping relating to our product candidates and any products that we may commercialize. Our manufacturers may not be able to comply with cGMP regulations or similar regulatory requirements outside the United States. Our manufacturers are subject to unannounced inspections by the FDA, state regulators and similar regulators outside the United States. Our failure, or the failure of our third party manufacturers, to comply with applicable regulations could result in sanctions being imposed on us, including fines, injunctions, civil penalties, failure of regulatory authorities to grant marketing approval of our product candidates, delays, suspension or withdrawal of approvals, license revocation, seizures or recalls of product candidates or products, operating restrictions and criminal prosecutions, any of which could significantly and adversely affect regulatory approval and supplies of our product candidates.

Our product candidates and any products that we may develop may compete with other product candidates and products for access to manufacturing facilities. There are a limited number of manufacturers that operate under cGMP regulations and that are both capable of manufacturing for us and willing to do so. If the third parties that we engage to manufacture products for our preclinical tests and clinical trials should cease to continue to do so for any reason, we likely would experience delays in advancing these trials while we identify and qualify replacement suppliers and we may be unable to obtain replacement supplies on terms that are favorable to us. Later relocation to another manufacturer will also require notification, review and other regulatory approvals from the FDA and other regulators and will subject our production to further cost and instability in the availability of our product candidates. In addition, if we are not able to obtain adequate supplies of our product candidates or the drug substances used to manufacture them, it will be more difficult for us to develop our product candidates and compete effectively.

Our current and anticipated future dependence upon others for the manufacture of our product candidates may adversely affect our future profit margins and our ability to develop product candidates and commercialize any products that obtain regulatory approval on a timely and competitive basis.

**Materials necessary to manufacture our product candidates may not be available on commercially reasonable terms, or at all, which may delay the development and commercialization of our product candidates.**

We rely on the manufacturers of our product candidates to purchase from third party suppliers the materials necessary to produce the compounds for our preclinical and clinical studies and will rely on these other manufacturers for commercial distribution if we obtain marketing approval for any of our product candidates. Suppliers may not sell these materials to our manufacturers at the time we need them or on commercially reasonable terms and all such prices are susceptible to fluctuations in price and availability due to transportation costs, government regulations, price controls and changes in economic climate or other foreseen circumstances. We do not have any control over the process or timing of the acquisition of these materials by our manufacturers. Moreover, we currently do not have any agreements for the commercial production of these materials. If our manufacturers are unable to obtain these materials for our preclinical and clinical studies, product testing and potential regulatory approval of our product candidates would be delayed, significantly impacting our ability to develop our product candidates. If our manufacturers or we are unable to purchase these materials after regulatory approval has been obtained for our product candidates, the commercial launch of our product candidates would be delayed or there would be a shortage in supply, which would materially affect our ability to generate revenues from the sale of our product candidates.

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**We rely on third parties to conduct certain preclinical development activities and our clinical trials and those third parties may not perform satisfactorily, including failing to meet established deadlines for the completion of such activities and trials.**

We do not independently conduct certain preclinical development activities of our product candidates, such as long-term safety studies in animals, or clinical trials for our product candidates. We rely on, or work in conjunction with, third parties, such as contract research organizations, medical institutions and clinical investigators, to perform this function. Our reliance on these third parties for preclinical and clinical development activities reduces our control over these activities. We are responsible for ensuring that each of our preclinical development activities and our clinical trials is conducted in accordance with the applicable general investigational plan and protocols, however, we have no direct control over these researchers or contractors (except by contract), as they are not our employees. Moreover, the FDA requires us to comply with standards, commonly referred to as Good Clinical Practices, or GCP, for conducting, recording and reporting the results of our preclinical development activities and our clinical trials to assure that data and reported results are credible and accurate and that the rights, safety and confidentiality of trial participants are protected. Our reliance on third parties that we do not control does not relieve us of these responsibilities and requirements. Furthermore, these third parties may also have relationships with other entities, some of which may be our competitors. If these third parties do not successfully carry out their contractual duties, meet expected deadlines or conduct our preclinical development activities or our clinical trials in accordance with regulatory requirements or our stated protocols, we will not be able to obtain, or may be delayed in obtaining, regulatory approvals for our product candidates and will not be able to, or may be delayed in our efforts to, successfully commercialize our product candidates. Moreover, these third parties may be bought by other entities or they may go out of business, thereby preventing them from meeting their contractual obligations.

We also rely on other third parties to store and distribute drug supplies for our preclinical development activities and our clinical trials. Any performance failure on the part of our existing or future distributors could delay clinical development or regulatory approval of our product candidates or commercialization of our products, producing additional losses and depriving us of potential product revenue.

Extensions, delays, suspensions or terminations of our preclinical development activities and our clinical trials as a result of the performance of our independent clinical investigators and contract research organizations will delay, and make more costly, regulatory approval for any product candidates that we may develop. Any change in a contract research organization during an ongoing preclinical development activity or clinical trial could seriously delay that trial and potentially compromise the results of the activity or trial.

**We may not be successful in maintaining or establishing collaborations, which could adversely affect our ability to develop and, particularly in international markets, commercialize products.**

For each of our product candidates, we are collaborating with physicians, patient advocacy groups, foundations and government agencies in order to assist with the development of our products. We plan to pursue similar activities in future programs and plan to evaluate the merits of retaining commercialization rights for ourselves or entering into selective collaboration arrangements with leading pharmaceutical or biotechnology companies. We also may seek to establish collaborations for the sales, marketing and distribution of our products outside the United States. If we elect to seek collaborators in the future but are unable to reach agreements with suitable collaborators, we may fail to meet our business objectives for the affected product or program. We face, and will continue to face, significant competition in seeking appropriate collaborators. Moreover, collaboration arrangements are complex and time consuming to negotiate, document and implement. We may not be successful in our efforts, if any, to establish and implement collaborations or other alternative arrangements. The terms of any collaborations or other arrangements that we establish, if any, may not be favorable to us.

Any collaboration that we enter into may not be successful. The success of our collaboration arrangements, if any, will depend heavily on the efforts and activities of our collaborators. It is likely that any collaborators of ours will have significant discretion in determining the efforts and resources that they will apply to these collaborations. The risks that we may be subject to in possible future collaborations include the following:

our collaboration agreements are likely to be for fixed terms and subject to termination by our collaborators





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in the event of a material breach or lack of scientific progress by us;

our collaborators are likely to have the first right to maintain or defend our intellectual property rights and, although we would likely have the right to assume the maintenance and defense of our intellectual property rights if our collaborators do not, our ability to do so may be compromised by our collaborators' acts or omissions; and

our collaborators may utilize our intellectual property rights in such a way as to invite litigation that could jeopardize or invalidate our intellectual property rights or expose us to potential liability.

Collaborations with pharmaceutical companies and other third parties often are terminated or allowed to expire by the other party. Such terminations or expirations may adversely affect us financially and could harm our business reputation in the event we elect to pursue collaborations that ultimately expire or are terminated.

**Risks Related to Our Intellectual Property**

**If we are unable to obtain and maintain protection for the intellectual property relating to our technology and products, the value of our technology and products will be adversely affected.**

Our success will depend in large part on our ability to obtain and maintain protection in the United States and other countries for the intellectual property covering or incorporated into our technology and products. The patent situation in the field of biotechnology and pharmaceuticals generally is highly uncertain and involves complex legal, technical, scientific and factual questions. We may not be able to obtain additional issued patents relating to our technology or products. Even if issued, patents issued to us or our licensors may be challenged, narrowed, invalidated, held to be unenforceable or circumvented, which could limit our ability to stop competitors from marketing similar products or reduce the term of patent protection we may have for our products. Changes in either patent laws or in interpretations of patent laws in the United States and other countries may diminish the value of our intellectual property or narrow the scope of our patent protection.

The degree of future protection for our proprietary rights is uncertain, and we cannot ensure that:

we or our licensors were the first to make the inventions covered by each of our pending patent applications;

we or our licensors were the first to file patent applications for these inventions;

others will not independently develop similar or alternative technologies or duplicate any of our technologies;

any patents issued to us or our licensors will provide a basis for commercially viable products, will provide us with any competitive advantages or will not be challenged by third parties;

we will develop additional proprietary technologies that are patentable;

we will file patent applications for new proprietary technologies promptly or at all;

our patents will not expire prior to or shortly after commencing commercialization of a product; or

the patents of others will not have a negative effect on our ability to do business.

In addition, we cannot be assured that any of our pending patent applications will result in issued patents. In particular, we have filed patent applications in the European Patent Office and other countries outside the United States that have not been issued as patents. These pending applications include, among others, the patent applications we license pursuant to a license agreement with Mount Sinai School of Medicine of New York University. If patents are not issued in respect of our pending patent applications, we may not be able to stop competitors from marketing similar products in Europe and other countries in which we do not have issued patents.

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The patents and patent applications that we own or have licensed relating to use of Amigal expire in 2018 in the United States and 2019 outside of the United States, and the foreign counterparts, if issued, would expire in 2019. Patents that we own or have licensed relating to Plicera expire between 2015 and 2016 in the United States and in 2015 outside of the United States for composition of matter, and in 2018 in the United States for methods of use. We currently have no issued patents or pending applications covering methods of using Plicera outside of the United States. Patents and patent applications that we own or have licensed relating to the use of AT2220 expire in 2018 in the United States. Further, we currently do not have composition of matter or method of use protection for AT2220 outside of the United States. Where we lack patent protection outside of the United States, we intend to seek orphan medicinal product designation and to rely on statutory data exclusivity provisions in jurisdictions outside the United States where such protections are available, including Europe. If we are unable to obtain such protection outside the United States, our competitors may be free to use and sell Plicera and/or AT2220 outside of the United States and there will be no liability for infringement or any other barrier to competition. The patent rights that we own or have licensed relating to our product candidates are limited in ways that may affect our ability to exclude third parties from competing against us if we obtain regulatory approval to market these product candidates. In particular:

We do not hold composition of matter patents covering Amigal and AT2220, two of our three lead product candidates. Composition of matter patents can provide protection for pharmaceutical products to the extent that the specifically covered compositions are important. For our product candidates for which we do not hold composition of matter patents, competitors who obtain the requisite regulatory approval can offer products with the same composition as our products so long as the competitors do not infringe any method of use patents that we may hold.

For some of our product candidates, the principal patent protection that covers or those we expect will cover, our product candidate is a method of use patent. This type of patent only protects the product when used or sold for the specified method. However, this type of patent does not limit a competitor from making and marketing a product that is identical to our product that is labeled for an indication that is outside of the patented method, or for which there is a substantial use in commerce outside the patented method.

Moreover, physicians may prescribe such a competitive identical product for indications other than the one for which the product has been approved, or off-label indications, that are covered by the applicable patents. Although such off-label prescriptions may infringe or induce infringement of method of use patents, the practice is common and such infringement is difficult to prevent or prosecute.

Our patents also may not afford us protection against competitors with similar technology. Because patent applications in the United States and many other jurisdictions are typically not published until 18 months after filing, or in some cases not at all, and because publications of discoveries in the scientific literature often lag behind the actual discoveries, neither we nor our licensors can be certain that we or they were the first to make the inventions claimed in our or their issued patents or pending patent applications, or that we or they were the first to file for protection of the inventions set forth in these patent applications. If a third party has also filed a United States patent application covering our product candidates or a similar invention, we may have to participate in an adversarial proceeding, known as an interference, declared by the United States Patent and Trademark Office to determine priority of invention in the United States. The costs of these proceedings could be substantial and it is possible that our efforts could be unsuccessful, resulting in a loss of our United States patent position.

**If we fail to comply with our obligations in our intellectual property licenses with third parties, we could lose license rights that are important to our business.**

We are a party to a number of license agreements including agreements with the Mount Sinai School of Medicine of New York University, the University of Maryland, Baltimore County and Novo Nordisk A/S, pursuant to which we license key intellectual property relating to our lead product candidates. We expect to enter into additional licenses in the future. Under our existing licenses, we have the right to enforce the licensed patent rights. Our existing licenses impose, and we expect that future licenses will impose, various diligence, milestone payment, royalty, insurance and other obligations on us. If we fail to comply with these obligations, the licensor may have the



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right to terminate the license, in which event we might not be able to market any product that is covered by the licensed patents.

**If we are unable to protect the confidentiality of our proprietary information and know-how, the value of our technology and products could be adversely affected.**

We seek to protect our know-how and confidential information, in part, by confidentiality agreements with our employees, corporate partners, outside scientific collaborators, sponsored researchers, consultants and other advisors. We also have confidentiality and invention or patent assignment agreements with our employees and our consultants. If our employees or consultants breach these agreements, we may not have adequate remedies for any of these breaches. In addition, our trade secrets may otherwise become known to or be independently developed by others. Enforcing a claim that a party illegally obtained and is using our trade secrets is difficult, expensive and time consuming, and the outcome is unpredictable. In addition, courts outside the United States may be less willing to protect trade secrets. Costly and time consuming litigation could be necessary to seek to enforce and determine the scope of our proprietary rights, and failure to obtain or maintain trade secret protection could adversely affect our competitive business position.

**If we infringe or are alleged to infringe the intellectual property rights of third parties, it will adversely affect our business.**

Our research, development and commercialization activities, as well as any product candidates or products resulting from these activities, may infringe or be accused of infringing one or more claims of an issued patent or may fall within the scope of one or more claims in a published patent application that may subsequently issue and to which we do not hold a license or other rights. Third parties may own or control these patents or patent applications in the United States and abroad. These third parties could bring claims against us that would cause us to incur substantial expenses and, if successful against us, could cause us to pay substantial damages. Further, if a patent infringement suit were brought against us, we or they could be forced to stop or delay research, development, manufacturing or sales of the product or product candidate that is the subject of the suit.

No assurance can be given that patents do not exist, have not been filed, or could not be filed or issued, which contain claims covering our products, technology or methods. Because of the number of patents issued and patent applications filed in our field, we believe there is a risk that third parties may allege they have patent rights encompassing our products, technology or methods.

We are aware, for example, of United States patents, and corresponding international counterparts, owned by third parties that contain claims related to treating protein misfolding. We have received written notice from one of these third parties indicating that it believes we may need a license to certain of these patents in order to avoid infringing such patents. If any of these third party patents were to be asserted against us we do not believe that our proposed products would be found to infringe any valid claim of these patents. If we were to challenge the validity of any issued United States patent in court, we would need to overcome a presumption of validity that attaches to every patent. This burden is high and would require us to present clear and convincing evidence as to the invalidity of the patent's claims. There is no assurance that a court would find in our favor on infringement or validity.

In order to avoid or settle potential claims with respect to any of the patent rights described above or any other patent rights of third parties, we may choose or be required to seek a license from a third party and be required to pay license fees or royalties or both. These licenses may not be available on acceptable terms, or at all. Even if we or our future collaborators were able to obtain a license, the rights may be nonexclusive, which could result in our competitors gaining access to the same intellectual property. Ultimately, we could be prevented from commercializing a product, or be forced to cease some aspect of our business operations, if, as a result of actual or threatened patent infringement claims, we are unable to enter into licenses on acceptable terms. This could harm our business significantly.

Others may sue us for infringing their patent or other intellectual property rights or file nullity, opposition or interference proceedings against our patents, even if such claims are without merit, which would similarly harm our business. For example, by letter dated April 10, 2007, we received a notice from a third party alleging trademark

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infringement in connection with our use of The NASDAQ Global Market ticker symbol FOLD for our common stock. Furthermore, during the course of litigation, confidential information may be disclosed in the form of documents or testimony in connection with discovery requests, depositions or trial testimony. Disclosure of our confidential information and our involvement in intellectual property litigation could materially adversely affect our business.

There has been substantial litigation and other proceedings regarding patent and other intellectual property rights in the pharmaceutical and biotechnology industries. In addition to infringement claims against us, we may become a party to other patent litigation and other proceedings, including interference proceedings declared by the United States Patent and Trademark Office and opposition proceedings in the European Patent Office, regarding intellectual property rights with respect to our products and technology. Even if we prevail, the cost to us of any patent litigation or other proceeding could be substantial.

Some of our competitors may be able to sustain the costs of complex patent litigation more effectively than we can because they have substantially greater resources. In addition, any uncertainties resulting from any litigation could significantly limit our ability to continue our operations. Patent litigation and other proceedings may also absorb significant management time.

Many of our employees were previously employed at universities or other biotechnology or pharmaceutical companies, including our competitors or potential competitors. We try to ensure that our employees do not use the proprietary information or know-how of others in their work for us. However, we may be subject to claims that we or these employees have inadvertently or otherwise used or disclosed intellectual property, trade secrets or other proprietary information of any such employee's former employer. Litigation may be necessary to defend against these claims and, even if we are successful in defending ourselves, could result in substantial costs to us or be distracting to our management. If we fail to defend any such claims, in addition to paying monetary damages, we may jeopardize valuable intellectual property rights, disclose confidential information or lose personnel.

**Risks Related to Regulatory Approval of Our Product Candidates**

**If we are not able to obtain and maintain required regulatory approvals, we will not be able to commercialize our product candidates, and our ability to generate revenue will be materially impaired.**

Our product candidates, including Amigal, Plicera and AT2220, and the activities associated with their development and commercialization, including their testing, manufacture, safety, efficacy, recordkeeping, labeling, storage, approval, advertising, promotion, sale and distribution, are subject to comprehensive regulation by the FDA and other regulatory agencies in the United States and by comparable authorities in other countries. Failure to obtain regulatory approval for a product candidate will prevent us from commercializing the product candidate in the jurisdiction of the regulatory authority. We have not obtained regulatory approval to market any of our product candidates in any jurisdiction. We have only limited experience in filing and prosecuting the applications necessary to obtain regulatory approvals and expect to rely on third party contract research organizations to assist us in this process.

Securing FDA approval requires the submission of extensive preclinical and clinical data and supporting information to the FDA for each therapeutic indication to establish the product candidate's safety and efficacy. Securing FDA approval also requires the submission of information about the product manufacturing process to, and inspection of manufacturing facilities by, the FDA. Our future products may not be effective, may be only moderately effective or may prove to have undesirable or unintended side effects, toxicities or other characteristics that may preclude our obtaining regulatory approval or prevent or limit commercial use.

Our product candidates may fail to obtain regulatory approval for many reasons, including:

our failure to demonstrate to the satisfaction of the FDA or comparable regulatory authorities that a product candidate is safe and effective for a particular indication;

the results of clinical trials may not meet the level of statistical significance required by the FDA or

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comparable regulatory authorities for approval;

our inability to demonstrate that a product candidate's benefits outweigh its risks;

our inability to demonstrate that the product candidate is at least as effective as existing therapies;

the FDA's or comparable regulatory authorities' disagreement with the manner in which we interpret the data from preclinical studies or clinical trials;

the FDA's or comparable regulatory authorities' failure to approve the manufacturing processes, quality procedures or manufacturing facilities of third party manufacturers with which we contract for clinical or commercial supplies; and

a change in the approval policies or regulations of the FDA or comparable regulatory authorities or a change in the laws governing the approval process.

The process of obtaining regulatory approvals is expensive, often takes many years, if approval is obtained at all, and can vary substantially based upon a variety of factors, including the type, complexity and novelty of the product candidates involved. Changes in regulatory approval policies during the development period, changes in or the enactment of additional statutes or regulations, or changes in regulatory review for each submitted product application may cause delays in the approval or rejection of an application. The FDA and non-United States regulatory authorities have substantial discretion in the approval process and may refuse to accept any application or may decide that our data is insufficient for approval and require additional preclinical, clinical or other studies. In addition, varying interpretations of the data obtained from preclinical and clinical testing could delay, limit or prevent regulatory approval of a product candidate. Any regulatory approval we ultimately obtain may be limited or subject to restrictions or post approval commitments that render the approved product not commercially viable. Any FDA or other regulatory approval of our product candidates, once obtained, may be withdrawn, including for failure to comply with regulatory requirements or if clinical or manufacturing problems follow initial marketing.

**Our product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval or commercialization.**

Undesirable side effects caused by our product candidates could interrupt, delay or halt clinical trials and could result in the denial of regulatory approval by the FDA or other regulatory authorities for any or all targeted indications, and in turn prevent us from commercializing our product candidates and generating revenues from their sale. For example, in a clinical trial of Amigal for Fabry disease, one patient with a history of hypertension experienced increased blood pressure during the course of the trial which was reported by the investigator as possibly related to the drug. Further, Amigal has been shown to cause reversible infertility effects in mice.

In addition, if any of our product candidates receive marketing approval and we or others later identify undesirable side effects caused by the product:

regulatory authorities may require the addition of restrictive labeling statements;

regulatory authorities may withdraw their approval of the product; and

we may be required to change the way the product is administered or conduct additional clinical trials.

Any of these events could prevent us from achieving or maintaining market acceptance of the affected product or could substantially increase the costs and expenses of commercializing the product candidate, which in turn could delay or prevent us from generating significant revenues from its sale or adversely affect our reputation.

**We may not be able to obtain orphan drug exclusivity for our product candidates. If our competitors are able to obtain orphan drug exclusivity for their products that are the same drug as our product candidates, we**

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**may not be able to have competing products approved by the applicable regulatory authority for a significant period of time.**

Regulatory authorities in some jurisdictions, including the United States and Europe, may designate drugs for relatively small patient populations as orphan drugs. We obtained orphan drug designations from the FDA for Amigal for the treatment of Fabry disease on February 25, 2004, for the active ingredient in Plicera for the treatment of Gaucher disease on January 10, 2006 and for AT2220 for the treatment of Pompe disease on June 18, 2007. We also obtained orphan drug designation in the European Union for Amigal on May 22, 2006 and on September 12, 2007, the European Medicines Agency's Committee for Orphan Medicinal Products recommended orphan designation for Plicera for the treatment of Gaucher disease. We anticipate filing for orphan drug designation in the European Union for AT2220 for the treatment of Pompe disease. Generally, if a product with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, the product is entitled to a period of marketing exclusivity, which precludes the applicable regulatory authority from approving another marketing application for the same drug for that time period. The applicable period is seven years in the United States and ten years in Europe. For a drug composed of small molecules, the FDA defines "same drug" as a drug that contains the same active molecule and is intended for the same use. Obtaining orphan drug exclusivity for Amigal and Plicera may be important to each of the product candidate's success. Even if we obtain orphan drug exclusivity for Amigal or Plicera for these indications, we may not be able to maintain it. For example, if a competitive product that is the same drug as our product candidate is shown to be clinically superior to our product candidate, any orphan drug exclusivity we have obtained will not block the approval of such competitive product and we may effectively lose what had previously been orphan drug exclusivity.

**Any product for which we obtain marketing approval could be subject to restrictions or withdrawal from the market and we may be subject to penalties if we fail to comply with regulatory requirements or if we experience unanticipated problems with our products, when and if any of them are approved.**

Any product for which we obtain marketing approval, along with the manufacturing processes, post approval clinical data, labeling, advertising and promotional activities for such product, will be subject to continual requirements of and review by the FDA and comparable regulatory authorities. These requirements include submissions of safety and other post marketing information and reports, registration requirements, cGMP requirements relating to quality control, quality assurance and corresponding maintenance of records and documents, requirements regarding the distribution of samples to physicians and recordkeeping. Even if we obtain regulatory approval of a product, the approval may be subject to limitations on the indicated uses for which the product may be marketed or to the conditions of approval, or contain requirements for costly post marketing testing and surveillance to monitor the safety or efficacy of the product. We also may be subject to state laws and registration requirements covering the distribution of our products. Later discovery of previously unknown problems with our products, manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may result in actions such as:

restrictions on such products, manufacturers or manufacturing processes;

warning letters;

withdrawal of the products from the market;

refusal to approve pending applications or supplements to approved applications that we submit;

voluntary or mandatory recall;

finest;

suspension or withdrawal of regulatory approvals or refusal to approve pending applications or supplements to approved applications that we submit;



refusal to permit the import or export of our products;  
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product seizure or detentions;

injunctions or the imposition of civil or criminal penalties; and

adverse publicity.

If we, or our suppliers, third party contractors, clinical investigators or collaborators are slow to adapt, or are unable to adapt, to changes in existing regulatory requirements or adoption of new regulatory requirements or policies, we or our collaborators may lose marketing approval for our products when and if any of them are approved, resulting in decreased revenue from milestones, product sales or royalties.

**Failure to obtain regulatory approval in international jurisdictions would prevent us from marketing our products abroad.**

We intend to have our products marketed outside the United States. In order to market our products in the European Union and many other jurisdictions, we must obtain separate regulatory approvals and comply with numerous and varying regulatory requirements. The approval procedures vary among countries and can involve additional testing and clinical trials. The time required to obtain approval may differ from that required to obtain FDA approval. The regulatory approval process outside the United States may include all of the risks associated with obtaining FDA approval. In addition, in many countries outside the United States, it is required that the product be approved for reimbursement by government-backed healthcare regulators or insurance providers before the product can be approved for sale in that country. We may not obtain approvals from regulatory authorities outside the United States on a timely basis, if at all. Approval by the FDA does not ensure approval by regulatory authorities in other countries or jurisdictions, and approval by one regulatory authority outside the United States does not ensure approval by regulatory authorities in other countries or jurisdictions or by the FDA. We may not be able to file for regulatory approvals and may not receive necessary approvals to commercialize our products in any market.

**Risks Related to Employee Matters and Managing Growth**

**Our future success depends on our ability to retain our Chief Executive Officer and other key executives and to attract, retain and motivate qualified personnel.**

We are highly dependent on John F. Crowley, our President and Chief Executive Officer, Matthew R. Patterson, our Chief Operating Officer, James E. Dentzer, our Chief Financial Officer, and David J. Lockhart, Ph.D., our Chief Scientific Officer. These executives each have significant pharmaceutical industry experience, including Mr. Crowley, with whom we have entered into an employment agreement that runs for successive one year terms until either we or Mr. Crowley elect to terminate the agreement. We may terminate Mr. Crowley's employment without cause at any time, or we may decide not to extend Mr. Crowley's agreement at the end of any term, or he may terminate his employment for good reason at any time, in each case subject to certain severance payments and benefits.

Mr. Crowley is a commissioned officer in the United States Navy (Reserve). The United States recently called Mr. Crowley to service, which he fulfilled, from September 11, 2006 to March 5, 2007, and he may be called to active duty service again at any time. The loss of Mr. Crowley for protracted military duty could materially adversely affect our business. We are also parties to employment agreements with each of Messrs. Patterson and Dentzer and Dr. Lockhart. These employment agreements each provide for an initial term of two years, and will continue thereafter for successive two-year periods until we provide the executive with written notice of the end of the agreement in accordance with its terms. We may terminate any of these executives without cause at any time, or one of these executives may quit for good reason within six months of the occurrence of certain corporate changes, in each case subject to certain severance payments and benefits. The loss of the services of any of these executives might impede the achievement of our research, development and commercialization objectives and materially adversely affect our business. We do not maintain key person insurance on Mr. Crowley or on any of our other executive officers.

Recruiting and retaining qualified scientific personnel, clinical personnel and sales and marketing personnel will also be critical to our success. Our industry has experienced a high rate of turnover in recent years. We may not

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be able to attract and retain these personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar personnel, particularly in New Jersey and surrounding areas. Although we believe we offer competitive salaries and benefits, we may have to increase spending in order to retain personnel.

We also experience competition for the hiring of scientific and clinical personnel from universities and research institutions. In addition, we rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development and commercialization strategy. Our consultants and advisors may be employed by employers other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us.

### **We expect to expand our development, regulatory and sales and marketing capabilities, and as a result, we may encounter difficulties in managing our growth, which could disrupt our operations.**

We are a development stage company with 83 full-time employees as of September 30, 2007. Of these employees, 52 work primarily in research and development and 31 provide administrative services. We expect to experience significant growth in the number of our employees and the scope of our operations, particularly in the areas of drug development, regulatory affairs and sales and marketing. Assuming our plans and business conditions progress consistent with our current projections, we plan to grow to a total of approximately 100 employees by the end of 2007 and to a total of 100-120 employees by the end of 2008. To manage our anticipated future growth, we must continue to implement and improve our managerial, operational and financial systems, expand our facilities and continue to recruit and train additional qualified personnel. Due to our limited resources, we may not be able to effectively manage the expansion of our operations or recruit and train additional qualified personnel. The physical expansion of our operations may lead to significant costs and may divert our management and business development resources. Any inability on the part of our management to manage growth could delay the execution of our business plans or disrupt our operations.

### **Risks Related to Our Common Stock**

#### **Our executive officers, directors and principal stockholders maintain the ability to control all matters submitted to our stockholders for approval.**

Our executive officers, directors and principal stockholders beneficially own shares representing 78% of our common stock. As a result, if these stockholders were to choose to act together, they would be able to control all matters submitted to our stockholders for approval, as well as our management and affairs. For example, these persons, if they choose to act together, will control the election of directors and approval of any merger, consolidation, sale of all or substantially all of our assets or other business combination or reorganization. This concentration of voting power could delay or prevent an acquisition of us on terms that other stockholders may desire. The interests of this group of stockholders may not always coincide with the interests of other stockholders, and they may act, whether by meeting or written consent of stockholders, in a manner that advances their best interests and not necessarily those of other stockholders, including obtaining a premium value for their common stock, and might affect the prevailing market price for our common stock.

#### **Provisions in our corporate charter documents and under Delaware law could make an acquisition of us, which may be beneficial to our stockholders, more difficult and may prevent attempts by our stockholders to replace or remove our current management.**

Provisions in our corporate charter and our bylaws that became effective upon our initial public offering may discourage, delay or prevent a merger, acquisition or other change in control of us that stockholders may consider favorable, including transactions in which our stockholders might otherwise receive a premium for their shares. These provisions could also limit the price that investors might be willing to pay in the future for shares of our common stock, thereby depressing the market price of our common stock. In addition, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our board of directors. Because our board of directors is responsible

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for appointing the members of our management team, these provisions could in turn affect any attempt by our stockholders to replace current members of our management team. Among others, these provisions:

establish a classified board of directors, and, as a result, not all directors are elected at one time;

allow the authorized number of our directors to be changed only by resolution of our board of directors;

limit the manner in which stockholders can remove directors from our board of directors;

establish advance notice requirements for stockholder proposals that can be acted on at stockholder meetings and nominations to our board of directors;

require that stockholder actions must be effected at a duly called stockholder meeting and prohibit actions by our stockholders by written consent;

limit who may call stockholder meetings;

authorize our board of directors to issue preferred stock, without stockholder approval, which could be used to institute a poison pill that would work to dilute the stock ownership of a potential hostile acquirer, effectively preventing acquisitions that have not been approved by our board of directors; and

require the approval of the holders of at least 67% of the votes that all our stockholders would be entitled to cast to amend or repeal certain provisions of our charter or bylaws.

Moreover, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, which prohibits a person who owns in excess of 15% of our outstanding voting stock from merging or combining with us for a period of three years after the date of the transaction in which the person acquired in excess of 15% of our outstanding voting stock, unless the merger or combination is approved in a prescribed manner.

**An active trading market for our common stock may not develop.**

We completed our initial public offering of equity securities in June 2007, and prior to this offering, there was no public market for our common stock. Although we have been listed on The NASDAQ Global Market, an active trading market for our common stock may never develop or be sustained. If an active market for our common stock does not develop or is not sustained, it may be difficult for our stockholders to sell shares since our initial public offering without depressing the market price for our common stock.

**If the price of our common stock is volatile, purchasers of our common stock could incur substantial losses.**

The price of our common stock is volatile. The stock market in general and the market for biotechnology companies in particular have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. The market price for our common stock may be influenced by many factors, including:

results of clinical trials of our product candidates or those of our competitors;

our entry into or the loss of a significant collaboration;

regulatory or legal developments in the United States and other countries, including changes in the health care payment systems;

variations in our financial results or those of companies that are perceived to be similar to us;

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changes in the structure of healthcare payment systems;

market conditions in the pharmaceutical and biotechnology sectors and issuance of new or changed securities analysts' reports or recommendations;

general economic, industry and market conditions;

results of clinical trials conducted by others on drugs that would compete with our product candidates;

developments or disputes concerning patents or other proprietary rights;

public concern over our product candidates or any products approved in the future;

litigation;

future sales or anticipated sales of our common stock by us or our stockholders; and

the other factors described in this "Risk Factors" section.

For these reasons and others, potential purchasers of our common stock should consider an investment in our common stock as risky and invest only if they can withstand a significant loss and wide fluctuations in the marked value of their investment.

**We have never paid cash dividends on our capital stock and we do not anticipate paying any cash dividends in the foreseeable future. Potential purchasers of our common stock should not invest in us if they require dividend income. Any income from an investment in us would only come from a rise in the market price of our common stock, which is uncertain and unpredictable.**

We have paid no cash dividends on our capital stock to date. We currently intend to retain our future earnings, if any, to fund the development and growth of our business and do not foresee payment of a dividend in any upcoming fiscal period. In addition, the terms of existing or any future debt agreements may preclude us from paying dividends. As a result, capital appreciation, if any, of our common stock will be the sole source of gain for the foreseeable future. **A significant portion of our total outstanding shares of common stock is restricted from immediate resale but may be sold into the market in the near future. This could cause the market price of our common stock to drop significantly, even if our business is doing well.**

Sales of a substantial number of shares of our common stock in the public market could occur at any time. These sales, or the perception in the market that the holders of a large number of shares of common stock intend to sell shares, could reduce the market price of our common stock. After our initial public offering, we had outstanding 22,234,426 shares of common stock. Of these shares, 5,005,333 may be resold in the public market immediately and the remaining 17,229,093 shares are currently restricted under securities laws or as a result of lock-up agreements. Moreover, after the offering, holders of an aggregate of 16,570,855 shares of our common stock have rights, subject to some conditions, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders. We also registered all 1,366,667 shares of common stock under our equity compensation plans on Form S-8 filed on August 9, 2007. These shares can be freely sold in the public market upon issuance, subject to the 180 day lock-up periods under the lock-up agreements. **If securities or industry analysts do not publish research or reports or publish unfavorable research about our business, the price of our common stock and trading volume could decline.**

The trading market for our common stock will depend in part on the research and reports that securities or industry analysts publish about us or our business. If securities or industry analysts do not continue coverage of us, the trading price for our common stock would be negatively affected. In the event we obtain securities or industry

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analyst coverage, if one or more of the analysts who covers us downgrades our common stock, the price of our common stock would likely decline. If one or more of these analysts ceases to cover us or fails to publish regular reports on us, interest in the purchase of our common stock could decrease, which could cause the price of our common stock or trading volume to decline.

**We will incur increased costs as a result of being a public company.**

As a public company, we will incur significant legal, accounting, reporting and other expenses that we did not incur as a private company, including costs related to compliance with the regulations of the Sarbanes-Oxley Act of 2002. We expect these rules and regulations to increase our legal and financial compliance costs and to make some activities more time-consuming and costly. We also expect these new rules and regulations may make it more difficult and more expensive for us to obtain director and officer liability insurance and we may be required to accept reduced policy limits and coverage or incur substantially higher costs to obtain the same or similar coverage. As a result, we may experience more difficulty attracting and retaining qualified individuals to serve on our board of directors or as executive officers. We cannot predict or estimate the amount of additional costs we may incur as a result of these requirements or the timing of such costs.

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

**Recent Sales of Unregistered Securities**

None.

**Use of Proceeds**

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 on May 30, 2007, which 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.

We paid to the underwriters underwriting discounts totaling approximately \$5.3 million in connection with the offering. In addition, we incurred additional costs of approximately \$1.6 million in connection with the offering, which when added to the underwriting discounts paid by us, amounts to total expenses of approximately \$6.9 million. Thus, the net offering proceeds to us, after deducting underwriting discounts and offering expenses, were approximately \$68.1 million. No offering expenses were paid directly or indirectly to any of our directors or officers (or their associates) or persons owning ten percent or more of any class of our equity securities or to any other affiliates.

As of October 26, 2007, we had invested the \$68.1 million in net proceeds from the offering in money market funds and in investment-grade, interest bearing instruments, pending their use. Through October 26, 2007, we have not used the net proceeds from the offering. We intend to use the proceeds for clinical development of our drug candidates, for research and development activities relating to additional preclinical programs and to fund working capital and other general corporate purposes, which may include the acquisition or licensing of complementary technologies, products or businesses.

**ITEM 3. DEFAULTS UPON SENIOR SECURITIES**

None.

**ITEM 4. SUBMISSION OF MATTERS TO A VOTE OF SECURITY HOLDERS**

None.

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**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 Bylaws
31.1*	Certification of Chief 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 Chief 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 Chief Executive Officer pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
32.2*	Certification of Chief 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 (Commission File No. 333-141700).

(2) Incorporated by reference to Exhibit 3.4 to our Registration Statement on Form S-1 (Commission File No. 333-141700)

\* These certifications are being furnished solely to accompany this quarterly report pursuant to 18 U.S.C. Section 1350, and are not being filed for purposes of

Section 18 of the Securities Exchange Act of 1934 and are not to be incorporated by reference into any filing of Amicus Therapeutics, Inc., whether made before or after the date hereof, regardless of any general incorporation language in such filing.



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**SIGNATURES**

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

AMICUS THERAPEUTICS, INC.

Date: October 31, 2007

By: /s/ JOHN F. CROWLEY

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

Date: October 31, 2007

By: /s/ JAMES E. DENTZER

James E. Dentzer  
Chief Financial Officer  
(Principal Financial and Accounting Officer)

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**INDEX TO EXHIBITS**

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