AMICUS THERAPEUTICS INC
Form S-1/A
June 19, 2006

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As filed with the Securities and Exchange Commission on June 19, 2006.
Registration No. 333-134191

UNITED STATES SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549
Amendment No. 1
to
Form S-1
REGISTRATION STATEMENT
UNDER
THE SECURITIES ACT OF 1933
AMICUS THERAPEUTICS, INC.
(Exact Name of Registrant as Specified in its Charter)

Delaware	**2834**	**20-0422823**
(State or Other Jurisdiction of Incorporation or Organization)	*(Primary Standard Industrial Classification Code Number)*	*(I.R.S. Employer Identification Number)*

6 Cedar Brook Drive
Cranbury, New Jersey 08512
(609) 662-2000
(Address, including zip code, and telephone number, including area code, of Registrant s principal executive offices)
John F. Crowley
Chief Executive Officer
Amicus Therapeutics, Inc.
6 Cedar Brook Drive
Cranbury, New Jersey 08512
(609) 662-2000
(Name, address, including zip code, and telephone number, including area code, of agent for service)
Copies to:

Julio E. Vega	**Patrick O Brien**
Bingham McCutchen LLP	**Ropes & Gray LLP**
150 Federal Street	**One International Place**
Boston, Massachusetts 02110-1726	**Boston, Massachusetts 02110-1726**
(617) 951-8000	**(617) 951-7000**

Approximate date of commencement of proposed sale to the public: As soon as practicable after this Registration Statement is declared effective.

If any of the securities being registered on this Form are to be offered on a delayed or continuous basis pursuant to Rule 415 under the Securities Act of 1933, as amended (the Securities Act), please check the following box. o

If this Form is filed to register additional securities for an offering pursuant to Rule 462(b) under the Securities Act, please check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering.

o _____

If this Form is a post-effective amendment filed pursuant to Rule 462(c) under the Securities Act, please check the following box and list the Securities Act registration statement number of the earlier effective registration statement

for the same offering. o _____

If this form is a post-effective amendment filed pursuant to Rule 462(d) under the Securities Act, please check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering. o _____

The Registrant hereby amends this Registration Statement on such date or dates as may be necessary to delay its effective date until the Registrant shall file a further amendment which specifically states that this Registration Statement shall thereafter become effective in accordance with Section 8(a) of the Securities Act or until this Registration Statement shall become effective on such date as the Commission, acting pursuant to said Section 8(a), may determine.

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The information contained in this prospectus is not complete and may be changed. We may not sell these securities until the registration statement filed with the Securities and Exchange Commission is effective. This prospectus is not an offer to sell these securities and we are not soliciting offers to buy these securities in any jurisdiction where the offer or sale is not permitted.

PROSPECTUS (Subject to Completion)
Issued June 19, 2006

Shares

COMMON STOCK

Amicus Therapeutics, Inc. is offering shares of its common stock. This is our initial public offering, and no public market currently exists for our shares. We anticipate that the initial public offering price will be between $ and $ per share.

We have applied to have our common stock approved for quotation on The Nasdaq National Market under the symbol AMTX.

Investing in our common stock involves risks. See Risk Factors beginning on page 8.

PRICE $ A SHARE

	Price to **Public**	**Underwriting Discounts and Commissions**	**Proceeds to Amicus**
Per Share	$	$	$
Total	$	$	$

We have granted the underwriters the right to purchase up to an additional shares of common stock to cover over-allotments.

The Securities and Exchange Commission and state securities regulators have not approved or disapproved these securities or determined if this prospectus is truthful or complete. Any representation to the contrary is a criminal offense.

Morgan Stanley & Co. Incorporated expects to deliver the shares to purchasers on , 2006.

MORGAN STANLEY *GOLDMAN, SACHS & CO.*

PACIFIC GROWTH EQUITIES, LLC

, 2006

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You should rely only on the information contained in this prospectus. We have not authorized anyone to provide you with information different from that contained in this prospectus. We are offering to sell, and seeking offers to buy, shares of our common stock only in jurisdictions where offers and sales are permitted. The information contained in this prospectus is accurate only as of the date of this prospectus, regardless of the time of delivery of this prospectus or of any sale of our common stock. In this prospectus, unless otherwise stated or the context otherwise requires, references to Amicus Therapeutics, Amicus, we, us, our and similar references refer to Amicus Therapeutics, In

Until , 2006, 25 days after the commencement of this offering, all dealers that buy, sell or trade shares of our common stock, whether or not participating in this offering, may be required to deliver a prospectus. This is in addition to the dealers obligation to deliver a prospectus when acting as underwriters and with respect to their unsold allotments or subscriptions.

For investors outside the United States: Neither we nor any of the underwriters have done anything that would permit this offering or possession or distribution of this prospectus in any jurisdiction where action for that purpose is required, other than in the United States. You are required to inform yourselves about and to observe any restrictions relating to this offering and the distribution of this prospectus.

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PROSPECTUS SUMMARY

This summary highlights selected information contained elsewhere in this prospectus. This summary may not contain all of the information that is important to you. Before investing in our common stock, you should read this prospectus carefully in its entirety, especially the risks of investing in shares of our common stock that we discuss in the Risk Factors section of this prospectus beginning on page 7 and our financial statements and related notes beginning on page F-1.

AMICUS THERAPEUTICS, INC.

Our Company

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. Since our founding in 2002, we have generated three product development programs: Amigal for Fabry disease, AT2101 for Gaucher disease and AT2220 for Pompe disease. Fabry, Gaucher and Pompe are relatively rare disorders but represent substantial commercial markets due to the severity of the symptoms and the chronic nature of the diseases. The worldwide net product sales for the four approved therapeutics to treat Fabry and Gaucher disease were more than $1.3 billion in 2005, as publicly reported by companies that market these therapeutics.

We are currently conducting Phase II clinical trials of Amigal and have observed results in the first four patients after 12 weeks of treatment that suggest that treatment with Amigal causes an increase in the activity of the enzyme deficient in Fabry disease. These results do not necessarily predict final results for our Phase II clinical trials, and subsequent results from these clinical trials or future clinical trials may not corroborate the results we have observed to date. We expect to complete enrollment in our current Phase II trials for Amigal by the end of 2006 and, assuming positive results, we intend to initiate a Phase III trial in 2007. We have initiated a Phase I trial for AT2101 and plan to file an investigational new drug application, or IND, for AT2220 by the end of 2006.

Human genetic diseases result from mutations in specific genes that, in many cases, lead to the production of proteins with reduced stability. Proteins with these mutations may not achieve their correct three-dimensional shape and are generally referred to as misfolded proteins. The cell ensures that proteins are folded into their correct shape before they can move from where they are made, the endoplasmic reticulum, or ER, to the appropriate destination in the cell, a process referred to as protein trafficking. Proteins that do not achieve their correct shape are often eliminated by the cell, resulting in reduced biological activity that can lead to impaired cellular function and ultimately to disease. In certain instances, misfolded proteins can accumulate in the ER instead of being eliminated. This accumulation of misfolded proteins may lead to various types of stress on cells, which may also contribute significantly to cellular dysfunction and disease.

Our novel approach to the treatment of human genetic diseases consists of using a pharmacological chaperone that selectively binds to the target protein, which increases the stability of the protein and helps it fold into its correct three-dimensional shape. This restores appropriate trafficking of the protein, thereby increasing protein activity, improving cellular function and reducing stress on cells.

The current standard of treatment for Fabry, Gaucher and Pompe is enzyme replacement therapy. This therapy compensates for the reduced level of activity of specialized proteins called enzymes through regular infusions. Instead of adding enzyme from an external source by intravenous infusion, our approach uses small molecule, orally-administered pharmacological chaperones to restore the function of the enzyme that is already made by the patient s own body. We believe our product candidates may have advantages relative to enzyme replacement therapy relating to biodistribution, treatment effect and ease of use,

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potentially improving treatment of these diseases. In addition, we believe our technology may be broadly applicable to other diseases that have been linked to misfolded proteins, including certain types of neurological disease, metabolic disease, cardiovascular disease and cancer.

Our Lead Programs

Our three most advanced product development programs target lysosomal storage disorders, which are chronic genetic diseases that frequently result in severe symptoms. Each of these disorders results from the deficiency of a single enzyme.

Amigal for Fabry disease. We are developing Amigal for the treatment of patients with Fabry disease, which commonly causes kidney failure, cardiac abnormalities and progressive neurological complications. We are currently conducting multiple Phase II clinical trials of Amigal. We expect to complete enrollment in our current Phase II trials by the end of 2006 and, assuming positive results, we intend to initiate a Phase III trial in 2007.

AT2101 for Gaucher disease. We are developing AT2101 for the treatment of Gaucher disease, which commonly causes an enlarged liver and spleen, low levels of red blood cells and platelets, bone pain and fractures. Some patients also present with neurological complications. In preclinical studies, administration of AT2101 resulted in a dose-related increase in the activity of the enzyme known to be deficient in Gaucher disease. We filed an IND for AT2101 in April 2006 and have initiated a Phase I trial. If our Phase I trials are successful, we plan to initiate a Phase II trial in the first half of 2007.

AT2220 for Pompe disease. We are developing AT2220 for the treatment of Pompe disease, which commonly causes progressive muscle weakness, particularly affecting breathing, mobility and heart function. In preclinical studies, administration of AT2220 resulted in an increase in the activity of the enzyme known to be deficient in Pompe disease. We plan to file an IND for AT2220 in the second half of 2006.

Initial Data from our Phase II Studies in Fabry Disease

We are currently conducting multiple open-label Phase II clinical trials in up to 48 adult male and female patients with Fabry disease. The initial data from the first four Fabry disease patients enrolled in one of our Phase II trials showed that after six weeks of treatment the activity of alpha-galactosidase A, or α-GAL, the enzyme deficient in Fabry disease, was on average more than five-fold higher in white blood cells than before treatment. The four patients had three different genetic mutations and we observed an increase in the level of α-GAL enzyme activity in all of these patients.

After the initial six weeks of treatment, in accordance with the protocol, the dose was decreased to the same dose used during the first two weeks of the study. Patients received this lower dose for an additional six weeks. The initial data obtained after 12 weeks of treatment show that α-GAL enzyme activity in white blood cells remained elevated at levels approximately four-fold higher than before treatment. In two of the four patients, α-GAL enzyme activity, as measured in biopsies of the skin, increased after 12 weeks of treatment, compared to levels before treatment. Results of α-GAL enzyme activity levels in skin biopsies of the other two patients were inconclusive due to insufficient sample size. GL-3 levels in patient plasma, urine and skin, both before and after 12 weeks of treatment, were in the normal range of healthy individuals. GL-3, a complex lipid called globotriaosylceramide, is the substrate broken down by α-GAL. In all four patients, the measures of cardiac, renal and central nervous system function before treatment were normal or near normal, and no clinically meaningful changes were observed after 12 weeks of treatment.

Amigal was well-tolerated with no reported serious adverse events. Adverse events in the first four patients were mostly mild and reported by the investigators as unlikely to be related to Amigal. We note that a fifth patient with a history of hypertension withdrew from the trial due to increased blood pressure, which was reported by the investigator as possibly related to the study drug.

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We believe the results from the 12 weeks of treatment of the first four Fabry patients support the continuation of our current Phase II clinical trials. It is generally believed that even small increases in lysosomal enzyme activity may have clinical benefits. These results do not necessarily predict final results for our Phase II clinical trials. The results from additional patients in our current Phase II clinical trials or additional data from these first four patients may cause the final results of our Phase II clinical trials to differ from or be less favorable than the results observed to date in the first four patients.

Our Strategy

Our goal is to become a leading biopharmaceutical company focused on the discovery, development and commercialization of pharmacological chaperone therapies for the treatment of a wide range of human genetic diseases. The introduction of pharmacological chaperones as a treatment option has the potential to address significant unmet medical needs and improve the quality of life for patients.

To achieve this goal, we intend to:

focus our initial efforts on developing pharmacological chaperones for severe genetic diseases;

rapidly advance our lead programs;

leverage our proprietary approach to discover and develop additional small molecules; and

build a targeted sales and marketing infrastructure.

Our success in achieving our goal, however, depends in part on the risks and uncertainties described in this prospectus in the section entitled Risk Factors, including, without limitation, those relating to our ability to conduct preclinical and clinical trials that demonstrate safety and efficacy of our product candidates, our ability to obtain regulatory approvals and our ability to attract and retain effective sales and marketing personnel.

Risks Associated with Our Business

Our business is subject to a number of risks of which you should be aware before making an investment decision. We discuss these risks more fully in the Risk Factors section of this prospectus immediately following this prospectus summary. We have a limited operating history and have not yet commercialized any products. We have incurred substantial operating losses in each year since inception. Our net loss attributable to common stockholders was $19.8 million and $7.7 million for the year ended December 31, 2005 and three month period ended March 31, 2006, respectively. As of March 31, 2006, we had an accumulated deficit of $44.7 million. We expect to incur significant and increasing net losses for at least the next several years. It is uncertain whether any of our product candidates under development will become effective treatments. All of our product candidates are undergoing clinical trials or are in earlier stages of development, and failure in the development of new drugs is common and can occur at any stage of development. None of our product candidates has received regulatory approval for commercialization, and we do not expect that any drugs resulting from our research and development efforts will be commercially available for a number of years, if at all. We may never generate any revenues or achieve profitability.

Our Corporate Information

We were incorporated under the laws of the State of Delaware on February 4, 2002. Our principal executive offices are located at 6 Cedar Brook Drive, Cranbury, New Jersey 08512, and our telephone number is (609) 662-2000. Our website address is *www.amicustherapeutics.com*. The information on, or that can be accessed through, our website is not part of this prospectus. We have included our website address in this prospectus solely as an inactive textual reference.

The names Amigal and Amicus and the Amicus logo are our trademarks. Fabrazyme®, Cerezyme®, Myozyme®, Replagal and Zavesca® are the property of their respective owners.

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THE OFFERING

Common stock we are offering	shares
Common stock to be outstanding after this offering	shares
Over-allotment option	shares
Use of proceeds	We estimate that the net proceeds from this offering will be approximately $ million, or approximately $ million if the underwriters exercise their over-allotment option in full, assuming an initial public offering price of $ per share, after deducting estimated underwriting discounts and commissions and offering expenses payable by us. We expect to use most of the net proceeds from this offering to fund clinical trial activities and preclinical research and development activities, and the balance for other general corporate purposes. See Use of Proceeds.
Risk factors	You should read the Risk Factors section of this prospectus for a discussion of the factors to consider carefully before deciding to purchase any shares of our common stock.
Proposed Nasdaq National Market symbol	AMTX

The number of shares of common stock to be outstanding immediately after the offering is based on 5,507,024 shares of common stock outstanding as of May 3, 2006, and gives effect as of May 3, 2006 to the automatic exercise upon the closing of this offering of outstanding warrants to purchase 465,486 shares of series B redeemable convertible preferred stock, and the issuance of 84,009,190 shares of common stock issuable upon the automatic conversion of all outstanding shares of our redeemable convertible preferred stock upon the closing of this offering. The number of shares of common stock to be outstanding after this offering excludes:

- 13,552,120 shares of common stock issuable upon the exercise of stock options outstanding as of May 3, 2006, with a weighted average exercise price of $0.48 per share;
- 40,000 shares of common stock issuable upon exercise of a warrant to purchase common stock at an exercise price of $0.75 per share;
- an aggregate of shares of common stock reserved for future issuance under our 2006 equity incentive plan as of the closing of this offering; and
- an aggregate of shares of common stock reserved for future issuance under our 2006 employee stock purchase plan as of the closing of this offering.

Unless otherwise noted, all information in this prospectus assumes:

- no exercise of the outstanding options or warrant described above; and
- no exercise by the underwriters of their option to purchase shares of common stock to cover over-allotments.

We expect to complete a one-for- reverse stock split of our common stock before completion of this offering. All share numbers will be adjusted to give effect to this reverse stock split.

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SUMMARY FINANCIAL DATA

The following is a summary of our financial data. You should read the summary financial data together with our financial statements and the related notes appearing at the end of this prospectus, and Management s Discussion and Analysis of Financial Condition and Results of Operations and other financial information appearing elsewhere in this prospectus.

The pro forma net loss per share data for the year ended December 31, 2005, and three month period ended March 31, 2006, give effect, as of the beginning of each such period, to the issuance on April 17, 2006 of 21,825,131 shares of our series C redeemable convertible preferred stock, the automatic or voluntary exercise upon the closing of this offering of all outstanding warrants to purchase 555,003 shares of our series B redeemable convertible preferred stock, and the automatic conversion of all outstanding shares of our redeemable convertible preferred stock into 84,009,190 shares of common stock upon the closing of this offering. The pro forma balance sheet data set forth below also give effect, as of March 31, 2006, to the foregoing events.

The pro forma as adjusted balance sheet data gives further effect to our issuance and sale of shares of common stock in this offering at an assumed initial public offering price of $ per share, which is the midpoint of the price range listed on the cover page of this prospectus, after deducting estimated underwriting discounts and commissions and offering expenses payable by us.

	Year Ended December 31,			Three Months Ended March 31,		Period from February 4, 2002 (inception) to March 31, 2006
	2003	2004	2005	2005	2006	
				(unaudited)	(unaudited) (Restated)	(unaudited) (Restated)
	(in thousands, except shares and per share data)					
Statement of Operations Data:						
Revenue	$	$	$	$	$	$
Operating expenses:						
Research and development	4,433	6,301	13,652	2,238	5,546	30,720
General and administrative	1,005	2,081	6,878	1,178	2,065	12,582
Impairment of leasehold improvements	1,030					1,030
Depreciation and amortization	132	146	303	47	199	804
In-process research and development						418
Total operating expenses	6,600	8,528	20,833	3,463	7,810	45,554
Loss from operations	(6,600)	(8,528)	(20,833)	(3,463)	(7,810)	(45,554)

Other income (expenses):						
Interest income	5	190	610	57	238	1,056
Interest expense	(172)	(550)	(82)	(4)	(59)	(869)
Loss before tax benefit	(6,768)	(8,888)	(20,305)	(3,410)	(7,631)	(45,367)
Income tax benefit		83	612			695
Net loss	(6,768)	(8,805)	(19,693)	(3,410)	(7,631)	(44,672)
Preferred stock accretion	(17)	(126)	(139)	(32)	(41)	(333)
Net loss attributable to common stockholders	$ (6,785)	$ (8,931)	$ (19,832)	$ (3,442)	$ (7,672)	$ (45,005)
Net loss attributable to common stockholders per common share basic and diluted	$ (2.94)	$ (3.87)	$ (6.45)	$ (1.49)	$ (1.81)	
Weighted-average common shares outstanding basic and diluted	2,306,541	2,306,541	3,076,649	2,314,804	4,228,564	
Unaudited pro forma net loss			$ (19,692)		$ (7,631)	
Unaudited pro forma basic and diluted net loss per share			$ (0.23)		$ (0.09)	
Unaudited shares used to compute pro forma basic and diluted net loss per share			87,085,839		88,237,754	

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	As of March 31, 2006		
	Actual	Pro Forma	Pro Forma as Adjusted
		(unaudited) (in thousands)	
Balance Sheet Data:			
Cash and cash equivalents and marketable securities	$ 19,552	$ 47,524	
Working capital	16,006	43,978	
Total assets	23,995	51,967	
Total liabilities	5,819	5,819	
Redeemable convertible preferred stock[(1)]	60,509		
Deficit accumulated during the development stage	(44,671)	(44,671)	
Total stockholders equity (deficiency)	(42,334)	46,148	

(1) In April 2006, we completed the sale of an additional 21,825,131 shares of series C redeemable convertible preferred stock for proceeds of $27.5 million. After evaluating the fair value of the common stock issuable upon conversion by our preferred stockholders, we determined that the issuance of the series C redeemable convertible preferred stock sold in April 2006 resulted in a beneficial conversion feature of approximately $19.4 million which will be recorded as a deemed dividend to preferred stockholders during the second quarter of 2006.

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RISK FACTORS

Investing in our common stock involves a high degree of risk. You should carefully consider the risks and uncertainties described below together with all of the other information included in this prospectus, including the financial statements and related notes appearing at the end of this prospectus, before deciding to invest in our common stock. If any of the following risks actually occur, they would materially harm our business, prospects, financial condition and results of operations. In this event, the market price of our common stock could decline and you could lose part or all of your investment.

Risks Related to Our Financial Position and Need for Additional Capital

We have incurred significant losses since our inception. We currently do not, and since inception never have had, any products available for commercial sale. We expect to incur 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 $19.8 million and $7.7 million for the year ended December 31, 2005 and the three months ended March 31, 2006, respectively. As of March 31, 2006, we had an accumulated deficit of $44.7 million. To date, we have financed our operations primarily through private placements of our redeemable convertible preferred stock. 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 I clinical trial of AT2101 for the treatment of Gaucher disease, initiate another Phase I clinical trial and potentially conduct later-stage clinical trials of AT2101;
- continue our ongoing preclinical development activities of AT2220 for the treatment of Pompe disease and potentially conduct 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 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. A decline in the market price of our common stock would also cause you to lose a part or all of your investment.

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We will need substantial additional 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 I clinical trials of AT2101 and our ongoing preclinical studies of AT2220 and potentially file an IND for AT2220. 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 this 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 (see Use of Proceeds). Additional funds may not be available when we need them 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 preclinical development activities and clinical trials of Amigal, AT2101 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 additional funds 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 additional funds 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 additional funds through collaboration and licensing arrangements with third parties, it may be necessary to relinquish valuable rights to our 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.

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Our short operating history may make it difficult for you 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 you make 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, AT2101 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, AT2101 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, AT2101 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, AT2101 and AT2220 for completion of our preclinical activities and clinical studies 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.

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

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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 reflect the prevalence of these diseases in the broader world population.

We estimate the number of potential patients in the broader world population who 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 report 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-U.S. 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. We are currently conducting Phase II clinical trials of Amigal for Fabry disease. We expect to complete enrollment in these Phase II clinical trials by the end of 2006. We are currently conducting a Phase I clinical trial of AT2101 for Gaucher disease in healthy volunteers and expect to commence another Phase I clinical trial in the second half of 2006. We expect to file an IND for AT2220 for Pompe disease by the end of 2006. 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 data from only four patients. Data from additional patients enrolled in these trials may be less favorable than the results to date. We cannot assure you 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 fifth patient in the ongoing Phase II clinical trials for Amigal for the treatment of Fabry disease elected to withdraw from the study. 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. In addition, we have only initial data from these four continuing patients. We will obtain additional data regarding the safety and efficacy of Amigal from these four patients as well as additional patients that enroll in our ongoing Phase II studies and any additional data may be less favorable than the data observed to date.

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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 and it may be difficult for us to obtain 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, AT2101 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, Phase I clinical trials for AT2101 and preclinical studies 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-U.S. regulatory agencies. The requirements of our clinical testing mandates 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. 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,

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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 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-U.S. 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, AT2101 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, AT2101 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 ability to offer our product candidates for sale at competitive prices;
- 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 studies, 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

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.

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

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

the inability to commercialize any products that we may develop.

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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 $16.4 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;

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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; and

the possible termination or nonrenewal 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, 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

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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.

We rely on third parties to conduct our 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 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 clinical data management 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

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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 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;

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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 assure you 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 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.

The U.S. patents that we own or have licensed relating to Amigal and AT2220 expire in 2018 and the U.S. patents that we own or have licensed relating to AT2101 expire between 2015 and 2018. 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 that 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 U.S. 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 U.S. 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 U.S. 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

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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 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 U.S. patents, and corresponding international counterparts, owned by third parties that contain claims related to treating protein misfolding. If any of these 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 U.S. 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.

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Others may sue us for infringing their patent rights or file nullity, opposition or interference proceedings against our patents, even if such claims are without merit, which would similarly harm our business. 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, AT2101 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;

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the results of clinical trials may not meet the level of statistical significance required by the FDA or 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 presents an advantage over 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-U.S. 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.

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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 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 and for AT2101 for the treatment of Gaucher disease on January 10, 2006. We also obtained orphan drug designation from the European Medicines Agency, or EMEA, for Amigal on May 22, 2006. We anticipate filing for orphan drug designation from the EMEA for AT2101 for the treatment of Gaucher disease and from the FDA and EMEA 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 AT2101 may be important to each of the product candidate s success. Even if we obtain orphan drug exclusivity for Amigal or AT2101 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.

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;
- fines;

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 principal members of our management team and scientific staff. These executives each have significant pharmaceutical industry experience, including our President and Chief Executive Officer, John F. 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. Mr. Crowley is a commissioned officer in the U.S. Navy (Reserve) and may be called to active duty service at any time. The loss of Mr. Crowley for protracted military duty would 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 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.

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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 52 employees as of April 30, 2006. Of these employees, 35 work in research and development and 17 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 have grown to a total of 90-100 employees by the end of 2006 and to a total of 140-150 employees by the end of 2007. 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 and This Offering

After this offering, our executive officers, directors and principal stockholders will maintain the ability to control all matters submitted to our stockholders for approval.

When this offering is completed, our executive officers, directors and stockholders who owned more than 5% of our outstanding common stock before this offering will, in the aggregate, beneficially own shares representing approximately % 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 your interests or 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 will become effective upon the closing of this 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 you might otherwise receive a premium for your 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 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;

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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.

If you purchase shares of common stock in this offering, you will suffer immediate dilution of your investment.

We expect the initial public offering price of our common stock to be substantially higher than the net tangible book value per share of our common stock. Therefore, if you purchase shares of our common stock in this offering, you will pay a price per share that substantially exceeds our net tangible book value per share after this offering. To the extent outstanding options or warrants are exercised, you will incur further dilution.

Based on an assumed initial public offering price of $ per share, which is the midpoint of the price range listed on the cover page of this prospectus, you will experience immediate dilution of $ per share, representing the difference between our pro forma net tangible book value per share after giving effect to this offering and the assumed initial public offering price. In addition, purchasers of common stock in this offering will have contributed approximately % of the aggregate price paid by all purchasers of our common stock but will own only approximately % of our common stock outstanding after this offering.

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

This is our initial public offering of equity securities and prior to this offering, there has been no public market for our common stock. The initial public offering price for our common stock will be determined through negotiations with the underwriters. Although we have applied to have our common stock approved for quotation on The Nasdaq National Market, an active trading market for our common stock may never develop or be sustained following this offering. If an active market for our common stock does not develop, it may be difficult for you to sell shares you purchase in this offering without depressing the market price for our common stock.

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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 likely to be 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. As a result of this volatility, investors may not be able to sell their shares of our common stock at or above the initial public offering price. 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;
- 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 you should consider an investment in our common stock as risky and invest only if you can withstand a significant loss and wide fluctuations in the marked value of your investment.

We have broad discretion in the use of the net proceeds from this offering and may not use them effectively.

Our management will have broad discretion in the application of the net proceeds from this offering and could spend the proceeds in ways that do not improve our results of operations or enhance the value of our common stock. The failure by our management to apply these funds effectively could result in financial losses that could have a material adverse effect on our business, cause the price of our common stock to decline and delay the development of our product candidates. Pending the application of these funds, we may invest the net proceeds from this offering in a manner that does not produce income or that loses value.

We intend to use the proceeds from this offering for clinical activities, including clinical supplies, preclinical research and development activities, general and administrative expenses, working capital needs and other general corporate purposes, including capital expenditures. Because of the number and variability of factors that will determine our use of the proceeds from this offering, their ultimate use may vary substantially from their currently intended use. For a further description of our intended use of the proceeds of this offering, see the Use of Proceeds section of this prospectus.

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We have never paid cash dividends on our capital stock and we do not anticipate paying any cash dividends in the foreseeable future. You should not invest in us if you 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 your 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 this offering, we will have outstanding shares of common stock based on the number of shares outstanding as of , 2006. Of these shares, may be resold in the public market immediately and the remaining shares are currently restricted under securities laws or as a result of lock-up agreements but will be able to be sold after the offering as described in the Shares Eligible for Future Sale section of this prospectus. Moreover, after this offering, holders of an aggregate of 84,049,190 shares of our common stock will 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 intend to register all shares of common stock that we may issue under our equity compensation plans. Once we register these shares, they can be freely sold in the public market upon issuance, subject to the 180 day lock-up periods under the lock-up agreements described in the Underwriters section of this prospectus.

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. We do not currently have and may never obtain research coverage by securities and industry analysts. If no securities or industry analysts commence coverage of us the trading price for our common stock would be negatively affected. In the event we obtain securities or industry 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.

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SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This prospectus contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts, included in this prospectus regarding our strategy, future operations, future financial position, future revenues, projected costs, prospects, plans and objectives of management are forward-looking statements. The words anticipate, believe, estimate, expect, intend, may, plan, predi 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 prospectus include, among other things, statements about:

- our plans to develop and commercialize Amigal, AT2101 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 prospectus, particularly in the Risk Factors section, 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 prospectus and the documents that we reference in this prospectus and have filed as exhibits to the registration statement of which this prospectus is a part completely and with the understanding that our actual future results may be materially different from what we expect. We do not assume any obligation to update any forward-looking statements.

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USE OF PROCEEDS

We estimate that the net proceeds from the sale of shares of common stock in this offering will be approximately $ million, or $ million if the underwriters exercise their over-allotment option in full, based on an assumed initial public offering price of $ per share, which is the midpoint of the price range listed on the cover page of this prospectus, and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us.

We intend to use the net proceeds from this offering to fund the growth of our business, including:

- $ to $ million for clinical development of Amigal for the treatment of Fabry disease;
- $ to $ million for clinical development of AT2101 for the treatment of Gaucher disease;
- $ to $ million for development of AT2220 for the treatment of Pompe disease;
- $ to $ million for research and development activities relating to additional preclinical programs; and
- the balance, if any, to fund working capital and other general corporate purposes, which may include the acquisition or licensing of complementary technologies, products or businesses, the expansion of our current corporate offices and laboratory space in Cranbury, NJ, and the leasing of additional space at one or more different facilities.

The expected use of net proceeds of this offering represents our intentions based on our current plans and business conditions. The amount and timing of our actual expenditures will depend on numerous factors, including the progress of our research and development activities and clinical trials, the number and breadth of our product development programs, whether or not we establish corporate collaborations and other arrangements, and the amount of cash, if any, generated by our operations and any unforeseen cash needs. As a result, we will retain broad discretion in the allocation and use of the remaining net proceeds of this offering. We do not expect the net proceeds from this offering and our other available funds to be sufficient to fund the completion of the development of our lead product candidates, and we expect that we will need to raise additional funds prior to being able to market any products. We have no current plans, agreements or commitments for any material acquisitions or licenses of any technologies, products or businesses.

Pending application of the net proceeds, as described above, we intend to invest any remaining proceeds in a variety of short-term, investment-grade, interest-bearing securities.

DIVIDEND POLICY

We have never declared or paid any dividends on our capital stock. We currently intend to retain any future earnings to finance our research and development efforts, the further development of our pharmacological chaperone technology, and the expansion of our business. We do not intend to declare or pay cash dividends to our stockholders in the foreseeable future.

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CAPITALIZATION

The following table sets forth our capitalization as of March 31, 2006:

- on an actual basis;
- on a pro forma basis to give effect, as of March 31, 2006, to our issuance on April 17, 2006 of 21,825,131 shares of series C redeemable convertible preferred stock, the automatic or voluntary exercise upon the completion of this offering of all outstanding warrants to purchase 555,003 shares of series B redeemable convertible preferred stock, and the automatic conversion of all outstanding shares of our redeemable convertible preferred stock into an aggregate of 84,009,190 shares of common stock upon the completion of this offering; and
- on a pro forma as adjusted basis to give further effect to our issuance and sale of shares of common stock in this offering at an assumed initial public offering price of $ per share, which is the midpoint of the price range listed on the cover page of this prospectus, after deducting estimated underwriting discounts and commissions and offering expenses payable by us.

The pro forma information below is illustrative only and our capitalization following the closing of this offering will be adjusted based on the actual initial public offering price and other terms of this offering determined at pricing. You should read this table together with Management s Discussion and Analysis of Financial Condition and Results of Operations and our financial statements and the related notes appearing at the end of this prospectus.

	As of March 31, 2006		
	Actual	Pro Forma	Pro Forma As Adjusted
		(unaudited) (in thousands)	
Capital lease obligations	$ 2,846	$ 2,846	
Series A redeemable convertible preferred stock, par value $0.01 per share; 3,333,334 shares authorized, issued and outstanding, actual; no shares authorized, issued or outstanding, pro forma and pro forma as adjusted	2,470		
Series B redeemable convertible preferred stock, par value $0.01 per share; 37,025,594 shares authorized, actual, 36,470,591 shares issued and outstanding, actual; no shares authorized, issued or outstanding, pro forma and pro forma as adjusted	30,696		
Series C redeemable convertible preferred stock, par value $0.01 per share; 43,650,262 shares authorized, issued and outstanding, actual; no shares authorized, issued or outstanding, pro forma and pro forma as adjusted	27,343		
Stockholders equity:			
Common stock, par value $0.01 per share; 115,000,000 shares authorized, actual and pro forma; 4,635,231 shares issued and outstanding, actual; 88,644,421 shares issued and outstanding, pro forma; shares authorized and shares issued and outstanding, pro forma as adjusted	46	886	

Additional paid-in capital[1]	2,297	89,938
Accumulated other comprehensive loss	(5)	(5)
Deficit accumulated during the development stage	(44,671)	(44,671)
Total stockholders equity (deficiency)[1]	$ (42,334)	$ 46,148
Total capitalization[1]	$ 21,021	$ 48,994

(1) A $1.00 increase (decrease) in the assumed initial public offering price of $ per share would increase (decrease) each of cash, and cash equivalents and short-term investments, additional paid-in capital, total stockholders equity and total capitalization by approximately $ million, assuming the number of shares offered by us, as set forth on the cover page of

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this prospectus, remains the same and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us.

The table above does not include:

- 14,573,975 shares of common stock issuable upon exercise of options outstanding as of March 31, 2006 at a weighted average exercise price of $0.43 per share;
- 40,000 shares of common stock issuable upon exercise of a warrant to purchase common stock at an exercise price of $0.75 per share;
- an aggregate of shares of common stock reserved for future issuance under our 2006 equity incentive plan as of the closing of this offering; and
- an aggregate of shares of common stock reserved for future issuance under our 2006 employee stock purchase plan as of the closing of this offering.

We expect to complete a one-for- reverse stock split of our common stock before the completion of this offering. All share numbers have been retroactively adjusted to give effect to this reverse stock split.

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DILUTION

If you invest in our common stock in this offering, your interest will be diluted to the extent of the difference between the public offering price per share of our common stock and the pro forma net tangible book value per share of our common stock after this offering.

The historical net tangible book value of our common stock as of March 31, 2006 was approximately $ million or $ per share, based on shares of common stock outstanding, as adjusted to reflect the one-for- reverse split of our common stock to be effected prior to the completion of this offering. Historical net tangible book value per share represents the amount of our total tangible assets less total liabilities, divided by the number of shares of common stock outstanding. Our pro forma net tangible book value as of March 31, 2006 was approximately $ million, or $ per share of common stock. Pro forma net tangible book value per share represents the amount of our total tangible assets reduced by the amount of our total liabilities, divided by the pro forma number of shares of common stock outstanding after giving effect, as of March 31, 2006, to the issuance on April 17, 2006 of 21,825,131 shares of our series C redeemable convertible preferred stock, the automatic or voluntary exercise upon completion of this offering of all outstanding warrants to purchase 555,003 shares of series B redeemable convertible preferred stock, and the automatic conversion of all outstanding shares of our redeemable convertible preferred stock into an aggregate of 84,009,190 shares of common stock upon completion of this offering.

After giving effect to our issuance and sale of shares of our common stock in this offering at an assumed initial public offering price of $ per share (the midpoint of the price range set forth on the cover page of this prospectus) less the estimated underwriting discounts and commissions and estimated offering expenses payable by us, our pro forma net tangible book value as of , 2006 would have been approximately $ million, or $ per share of our common stock. This represents an immediate increase in pro forma net tangible book value of $ per share to our existing stockholders and an immediate dilution in pro forma net tangible book value of $ per share to new investors purchasing shares in this offering at the initial public offering price. Dilution per share to new investors is determined by subtracting pro forma net tangible book value per share after this offering from the initial public offering price per share paid by a new investor.

The following table illustrates this per share dilution:

Assumed initial public offering price per share	$
Historical net tangible book value per shares as of March 31, 2006	$
Increase attributable to the conversion of outstanding preferred stock	
Pro forma net tangible book value per share before this offering	
Increase per share attributable to new investors	
Pro forma net tangible book value per share after this offering	
Dilution per share to new investors	$

A $1.00 increase (decrease) in the assumed initial public offering price of $ per share would increase (decrease) our pro forma net tangible book value after this offering by approximately $ million, our pro forma net tangible book value per share after this offering by approximately $ by $ per share and dilution per share to new investors in this offering by approximately $ assuming that the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same and after deducting the estimated underwriting discounts and commissions.

If the underwriters exercise their over-allotment option or if any shares are issued in connection with outstanding options or warrants, you will experience further dilution.

The following table sets forth, as of March 31, 2006, on a pro forma basis to give effect to our issuance on April 17, 2006 of 21,825,131 shares of series C redeemable convertible preferred stock, the automatic or voluntary exercise upon completion of this offering of all outstanding warrants to purchase 555,003 shares of series B redeemable convertible preferred stock, and the automatic conversion of all

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outstanding shares of our redeemable convertible preferred stock into an aggregate of 84,009,190 shares of common stock upon the closing of this offering, the total consideration paid investors in this offering and the average price per share paid, or to be paid, to us by existing stockholders and by new investors in this offering at an assumed initial public offering price of $ per share, which is the midpoint of the price range listed on the cover page of this prospectus, before deducting estimated underwriting discounts and commissions.

	Shares Purchased		Total Consideration		Average Price Per Share
	Number	Percent	Amount	Percent	
Existing stockholders		%		%	$
New investors[1]					
Total		100%		100%	

(1) A $1.00 increase (decrease) in the assumed initial public offering price of $ per share would increase (decrease) the total consideration paid by new investors by $ million and increase (decrease) the percentage of total consideration paid by new investors by approximately %, assuming that the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same.

The discussion and tables above exclude:

- 14,573,975 shares of common stock issuable upon exercise of stock options outstanding as of March 31, 2006 at a weighted average exercise price of $0.43 per share;
- 40,000 shares of common stock issuable upon exercise of a warrant to purchase common stock at an exercise price of $0.75 per share;
- an aggregate of shares of common stock reserved for future issuance under our 2006 equity incentive plan as of the closing of this offering; and
- an aggregate of shares of common stock reserved for future issuance under our 2006 employee stock purchase plan as of the closing of this offering.

If the underwriters exercise their over-allotment option in full, the following will occur:

- the percentage of shares of common stock held by existing stockholders will decrease to approximately % of the total number of shares of our common stock outstanding after this offering; and
- the pro forma as adjusted number of shares held by new investors will be increased to , or approximately %, of the total pro forma as adjusted number of shares of our common stock outstanding after this offering.

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SELECTED FINANCIAL DATA

You should read the following selected financial data together with our financial statements and the related notes appearing at the end of this prospectus and the Management s Discussion and Analysis of Financial Condition and Results of Operations section of this prospectus. We have derived the statements of operations data for the years ended December 31, 2003, 2004 and 2005 and the balance sheet data at December 31, 2004 and 2005 from our audited financial statements, which are included in this prospectus. We have derived the statement of operations for the period of February 4, 2002 (inception) to December 31, 2002, and the balance sheet data at December 31, 2002 and 2003, from our audited financial statements, which are not included in this prospectus. We have derived the statements of operations data for the three months ended March 31, 2005 and 2006, the period February 4, 2002 (inception) to March 31, 2006, and the balance sheet data at March 31, 2006, from our unaudited financial statements. The unaudited financial statements include, in the opinion of management, all adjustments, consisting of only recurring adjustments, that management considers necessary for the fair presentation of the financial information set forth in those statements. Our historical results for any prior period are not necessarily indicative of results to be expected for any future period.

	Period from February 4, 2002 (inception) to December 31, 2002	Year Ended December 31,			Three Months Ended March 31,		Period from February 4, 2002 (inception) to March 31, 2006
		2003	2004	2005	2005	2006	
					(unaudited)	(unaudited) (Restated)	(unaudited) (Restated)
	(in thousands, except shares and per share data)						
Statement of Operations Data:							
Revenue	$	$	$	$	$	$	$
Operating expenses:							
Research and development	1,147	4,433	6,301	13,652	2,238	5,546	30,720
General and administrative	197	1,005	2,081	6,878	1,178	2,065	12,582
Impairment of leasehold improvements		1,030					1,030
Depreciation and amortization	21	132	146	303	47	199	804
In-process research and development	418						418
Total operating	1,783	6,600	8,528	20,833	3,463	7,810	45,554

expenses							
Loss from operations	(1,783)	(6,600)	(8,528)	(20,833)	(3,463)	(7,810)	(45,554)
Other income (expenses):							
Interest income	13	5	190	610	57	238	1,056
Interest expense	(6)	(172)	(550)	(82)	(4)	(59)	(869)
Loss before tax benefit	(1,776)	(6,768)	(8,888)	(20,305)	(3,410)	(7,631)	(45,367)
Income tax benefit			83	612			695
Net loss	(1,776)	(6,768)	(8,805)	(19,693)	(3,410)	(7,631)	(44,672)
Preferred stock accretion	(10)	(17)	(126)	(139)	(32)	(41)	(333)
Net loss attributable to common stockholders	$ (1,786)	$ (6,785)	$ (8,931)	$ (19,832)	$ (3,442)	$ (7,672)	$ (45,005)
Net loss attributable to common stockholders per common share basic and diluted		$ (2.94)	$ (3.87)	$ (6.45)	$ (1.49)	$ (1.81)	
Weighted-average common shares outstanding basic and diluted		2,306,541	2,306,541	3,076,649	2,314,804	4,228,564	
Unaudited pro forma net loss				$ (19,692)		$ (7,631)	
Unaudited pro forma basic and diluted net loss per share				$ (0.23)		$ (0.09)	
Unaudited shares used to compute pro forma basic and diluted net loss per share				87,085,839		88,237,754	

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	As of December 31,				As of March 31,
	2002	2003	2004	2005	2006
					(unaudited)
			(in thousands)		
Balance Sheet Data:					
Cash and cash equivalents and marketable securities	$ 1,341	$ 15	$ 4,336	$ 24,418	$ 19,552
Working capital	947	(5,588)	3,569	22,267	16,006
Total assets	1,919	501	5,073	28,670	23,995
Total liabilities	752	5,776	922	3,327	5,819
Redeemable convertible preferred stock[(1)]	2,416	2,432	20,014	60,469	60,509
Deficit accumulated during the development stage	(1,775)	(8,503)	(17,348)	(37,040)	(44,671)
Total stockholders (deficiency)	$ (1,249)	$ (7,708)	$ (15,863)	$ (35,126)	$ (42,334)

(1) In April 2006, we completed the sale of an additional 21,825,131 shares of series C redeemable convertible preferred stock for proceeds of $27.5 million. After evaluating the fair value of our common stock issuable upon conversion by our preferred stockholders, we determined that the issuance of the series C redeemable convertible preferred stock sold in April 2006 will result in a beneficial conversion feature of $19.4 million which will be recorded as a deemed dividend to preferred stockholders during the second quarter of 2006.

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MANAGEMENT S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

You should read the following discussion and analysis of our financial condition and results of operations together with our financial statements and the related notes appearing at the end of this prospectus. Some of the information contained in this discussion and analysis or set forth elsewhere in this prospectus, including information with respect to our plans and strategy for our business and related financing, includes forward-looking statements that involve risks and uncertainties. You should read the Risk Factors section of this prospectus for a discussion of important factors that could cause actual results to differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis. We expressly disclaim any obligation or intention to provide updates to the forward-looking statements contained herein and the estimates and assumptions associated with them.

Overview

We are a clinical-stage biopharmaceutical company focused on the discovery, development and commercialization of novel small molecule, orally-administered drugs for the treatment of a range of human genetic diseases. Human genetic diseases result from mutations in specific genes that, in many cases, lead to the production of proteins that fold improperly or with reduced stability. Proteins with these mutations may not achieve their correct three-dimensional shape and are generally referred to as misfolded proteins. Misfolded proteins are often recognized by cells as having folding defects and, as a result, are eliminated prior to reaching their intended location in the cell. The reduced or completely absent 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 a new type of drug, which we refer to as a pharmacological chaperone, that selectively binds to the misfolded protein which increases the stability of the protein and helps it fold into the correct three-dimensional shape. This increased stability and corrected folding allow the protein to move to its proper destination in the cell, where it performs its intended biological activity. We believe this approach may restore and improve the function of the affected cells and ultimately have a beneficial therapeutic effect on the body. We are currently conducting Phase II clinical studies of our lead product candidate, Amigal, for Fabry disease. We filed an IND in April 2006 for our product candidate, AT2101, for Gaucher disease and have initiated a Phase I study. In addition, we plan to file an IND by the end of 2006 to initiate clinical testing of our product candidate, AT2220, for Pompe disease. We are also leveraging our expertise in pharmacological chaperones to build an extensive pipeline of product candidates for a range of other human genetic diseases resulting from misfolded proteins.

We have generated significant losses as we have progressed our lead product candidates into clinical development and expect to continue to generate losses as we continue the clinical development of Amigal, AT2101, and AT2220. From our inception in February 2002 through March 31, 2006, we have accumulated a deficit of $44.7 million. Since we do not generate revenue from any of our product candidates, our losses will continue as we continue to 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.

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 capital lease and equipment financings. If our development efforts result in clinical success, regulatory approval and successful commercialization of our products, we could generate revenue from sales of our products.

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Research and Development Expense

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;
- manufacturing development costs;
- personnel related expenses, including salaries, benefits, travel, and related costs for the personnel involved in drug discovery and development;
- activities relating to regulatory filings and the advancement of our product candidates through preclinical studies and clinical trials; and
- facilities and other allocated expenses, which include direct and allocated expenses for rent and maintenance of our facilities, 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 and we do not track time spent by employees on specific projects. As a result, we do not record or maintain information regarding internal costs incurred for our research and development programs on a program specific basis. In addition, we do not believe that allocating costs on the basis of estimates of time spent by our employees would accurately reflect 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 March 31, 2006, we have incurred research and development expense in the aggregate of $30.7 million, including stock-based compensation expense of $0.5 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).

	Year Ended December 31,			Three Months Ended March 31,		Period from February 4, 2002 (inception) to March 31,
Product Candidate	**2003**	**2004**	**2005**	**2005**	**2006**	**2006**
Third party direct project expenses						
Amigal (Fabry Disease Phase II)	$ 3,041	$ 4,547	$ 5,579	$ 1,452	$ 800	$ 13,967
AT2101 (Gaucher Disease Phase I)		26	2,164		2,017	4,207
AT2220 (Pompe Disease Preclinical)			374		436	810
Total third party direct project expenses	3,041	4,573	8,117	1,452	3,253	18,984

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Product Candidate	Year Ended December 31, 2003	2004	2005	Three Months Ended March 31, 2005	2006	Period from February 4, 2002 (inception) to March 31, 2006
Internal project costs[1]						
Personnel related costs	1,175	1,363	4,031	580	1,616	8,589
Other internal costs	217	365	1,504	206	677	3,146
Total internal project costs	1,392	1,728	5,535	786	2,293	11,735
Total research and development costs	$ 4,433	$ 6,301	$ 13,652	$ 2,238	$ 5,546	$ 30,719

(1) We utilize our internal resources across multiple projects and do not allocate internal costs to specific 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, AT2101, AT2220 or any of our other preclinical product candidates. This 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 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 will be required for the completion of clinical development of a product candidate, or if we experience significant delays in enrollment in any 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, advertising expenses, 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 become subject to the reporting obligations applicable to public companies. From our inception in February 2002 through March 31, 2006, we spent $12.6 million, including stock-based compensation expense of $0.7 million, on general and administrative expense.

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Beneficial Conversion Feature

In March 2006, the investors in our series C redeemable convertible preferred stock financing committed to the purchase of an additional 21,825,131 shares of series C redeemable convertible preferred stock. These shares were issued for proceeds of $27.5 million in April 2006. After evaluating the fair value of our common stock issuable upon conversion by the holders of the shares, we determined that the issuance of the additional shares of series C redeemable convertible preferred stock resulted in a beneficial conversion feature calculated in accordance with Emerging Issues Task Force (EITF) Issue No. 98-5, *Accounting for Convertible Securities with Beneficial Conversion Features or Contingently Adjustable Conversion Ratios,* as interpreted by EITF Issue No. 00-27, *Application of Issue No. 98-5 to Certain Convertible Instruments,* of $19.4 million which will be recorded as a deemed dividend to preferred stockholders for the three month period ended June 30, 2006.

The accompanying financial statements for the quarter ended March 31, 2006 were restated as we determined that our deemed dividend related to the Series C redeemable convertible preferred stock should have been recorded as a second quarter event instead of a first quarter event. The deemed dividend was initially recorded in the financial statements as of the measurement date instead of the date the shares were issued, which occurred in April 2006. The change had no impact on net loss for the three months ended March 31, 2006 and decreased net loss attributable to common shareholders for the three months ended March 31, 2006 by $19.4 million; resulting in an increase to basic and diluted earnings per share attributable to common stockholders of $4.60, from ($6.41) to ($1.81). This change was confined to the first quarter of 2006 only and had no impact on the related balance sheet accounts and statement of cash flows. The statement of changes in stockholders deficiency were revised to remove the beneficial conversion charges, however, the net impact was zero.

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 our significant accounting policies are more fully described in Note 2 to our financial statements appearing at the end of this prospectus, 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.

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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 paid 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.

Adoption of SFAS No. 123(R)

Effective January 1, 2006, we adopted Statement of Financial Accounting Standards No. 123(R), *Share-Based Payment* (SFAS 123(R)), which requires compensation costs related to share-based transactions, including employee stock options, to be recognized in the financial statements based on fair value. SFAS 123(R) revises SFAS 123, as amended, *Accounting for Stock-Based Compensation* (SFAS 123), and supersedes Accounting Principles Board (APB) Opinion No. 25, *Accounting for Stock Issued to Employees* (APB 25). We adopted SFAS 123(R) using the prospective method. Under this method, compensation cost is recognized for all share-based payments granted subsequent to December 31, 2005. Prior to January 1, 2006, we used the minimum value method, to determine values of our pro forma stock-based compensation disclosures.

Stock-Based Compensation

At March 31, 2006, we had one stock-based employee compensation plan, which is described more fully in Note 7 to our financial statements appearing at the end of this prospectus. Prior to January 1, 2006, we accounted for this plan under the recognition and measurement provisions of APB 25 and related interpretations, as permitted by SFAS 123. Stock-based employee compensation cost was recognized in the statement of operations for 2003, 2004 and 2005, to the extent options granted under the plan had an exercise price that was less than the fair market value of the underlying common stock on the date of grant. Under the prospective transition method, compensation cost recognized for all share-based payments granted subsequent to January 1, 2006 is based on the grant-date fair value estimated in accordance with the provisions of SFAS 123(R). Results for prior periods have not been restated. As a result of adopting SFAS 123(R) on January 1, 2006, our net income for the period ended March 31, 2006 was less than had we continued to account for share-based compensation under APB 25.

Prior to the adoption of SFAS 123(R), we presented our unamortized portion of deferred compensation cost for nonvested stock options in the statement of changes in shareholders deficiency with a corresponding credit to additional paid-in capital. Upon the adoption of SFAS 123(R), these amounts were offset against each other as SFAS 123(R) prohibits the gross-up of stockholders equity. Under SFAS 123(R), an equity instrument is not considered to be issued until the instrument vests. As a result, compensation cost is recognized over the requisite service period with an offsetting credit to additional paid-in capital.

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The following table illustrates the effect on our net loss and earnings per share if we had applied the provisions of SFAS 123 to options granted under our stock option plan for all periods presented prior to the adoption of SFAS 123(R). For purposes of this pro forma disclosure, the value of the options is estimated using a minimum value option-pricing formula and amortized to expense over the options vesting periods of the options.

	Years Ended December 31,			Three Months Ended March 31,
	2003	2004	2005	2005
Net loss attributable to common stockholders, as reported	$ (6,784,589)	$ (8,930,924)	$ (19,830,557)	$ (3,441,673)
Add: Non-cash employee compensation	70,340	59,842	364,551	91,138
Less: Total stock-based employee compensation expense determined under the minimum value method for all awards	(76,207)	(74,499)	(437,296)	(109,324)
Pro forma net loss attributable to common stockholders	$ (6,790,456)	$ (8,945,581)	$ (19,903,302)	$ (3,459,859)
Net loss attributable to common stockholders per common share:				
Basic and fully diluted:				
As reported	$ (2.94)	$ (3.87)	$ (6.45)	$ (1.49)
Pro forma	$ (2.94)	$ (3.88)	$ (6.47)	$ (1.49)

We recognized employee compensation expense of $70,340, $59,842, $364,551, and $91,138 and $315,671 for the years ended 2003, 2004 and 2005 and for the three months ended March 31, 2005 and 2006, respectively.

During the three months ended March 31, 2006, we recorded incremental compensation expense of approximately $152,000 ($0.04 per basic and diluted share) related to the expensing of our options under SFAS 123(R) during the quarter. The compensation expense had no impact on our cash flows from operations and financing activities. The total compensation cost related to non-vested stock option awards issued during 2006 but not yet recognized as of March 31, 2006 was approximately $7.1 million. This expense will be recorded on a straight-line basis over approximately four years.

Upon adoption of SFAS 123(R), we selected the Black-Scholes option pricing model as the most appropriate model for determining the estimated fair value for stock-based awards. The fair value of stock option awards subsequent to December 31, 2005 is amortized on a straight-line basis over the requisite service periods of the awards, which is generally the vesting period. Use of a valuation model requires management to make certain assumptions with respect to selected model inputs. Expected volatility was calculated based on a blended weighted average of historical information of our stock and the weighted average of historical information of similar public entities for which historical information was available. We will continue to use a blended weighted average approach using our own historical volatility and other similar public entity volatility information until our historical volatility is relevant to measure expected volatility for future option grants. The average expected life was determined according to the SEC shortcut approach as described in Staff Accounting Bulletin, or SAB, 107, *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. Forfeitures are estimated based on voluntary termination

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behavior, as well as a historical analysis of actual option forfeitures. The assumptions used in the Black-Scholes option pricing model are as follows:

	Three Months Ended March 31, 2006
Expected stock price volatility	72.70%
Risk free interest rate	4.59%
Expected life of options (years)	6.25
Expected annual dividend per share	$ 0.00

The weighted-average fair value (as of the date of grant) of the options granted during the three months ended March 31, 2006 is $1.52.

During the three months ended March 31, 2006 we granted stock options with exercise prices as follows:

Grant Date	Number of Options Granted	Exercise Price	Retrospective Fair Value Estimate per Common Share	Intrinsic Value per Share
January 2, 2006	17,000	$ 0.71	$ 1.44	$ 0.73
January 12, 2006	5,000	0.71	1.44	0.73
February 6, 2006	5,000	0.71	1.44	0.73
February 9, 2006	23,000	0.71	1.44	0.73
February 13, 2006	7,500	0.71	1.44	0.73
February 22, 2006	35,000	0.71	1.44	0.73
February 28, 2006	5,752,500	0.71	1.84	1.13
March 27, 2006	50,000	0.71	1.84	1.13
	5,895,000			

The exercise prices for options granted were set by our board of directors, the members of which have extensive experience in the life sciences industry and all but one of whom are non-employee directors, with input from our management, based on our determination of the fair market value of our common stock at the time of the grants. In connection with the preparation of the financial statements for this offering, we performed a retrospective determination of fair value for financial reporting purposes of our common stock underlying stock option grants in 2005 and 2006 utilizing a combination of valuation methods described in the AICPA *Technical Practice Aid, Valuation of Privately-Held-Company Equity Securities Issued as Compensation* (Practice Aid). Information on stock option grants during 2005 is as follows:

Date of 2005 Issuance	Number of Options Granted	Average Exercise Price	Retrospective Fair Value Estimate per Common Share	Intrinsic Value per Share

January - May	3,037,037	$ 0.09	$ 0.31	$ 0.22
June - July	1,768,748	0.09	0.77	0.68
August - September	315,500	0.22	0.95	0.73
October - November	2,351,000	0.71	1.14	0.43
December	104,500	0.71	1.44	0.73
	7,576,785			

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Determining the fair value of the common stock of a private enterprise requires complex and subjective judgments. Our retrospective estimates of enterprise value at each of the grant dates during 2005 and 2006 used results from both the income approach and the market approach.

Under the income approach, our enterprise value was based on the present value of our forecasted operating results. Our revenue forecasts were based on our estimates of expected annual growth rates following the anticipated commercial launch of our product candidates Amigal, AT2101 and AT2220. Estimated operating expenses were based on our internal assumptions, including continuing research and development activities for Amigal, AT2101, AT2220 and other preclinical candidates, and preparation and ongoing support for the commercialization of our lead product candidates. The assumptions underlying the estimates are consistent with our business plan. The risks associated with achieving our forecasts were assessed in selecting the appropriate discount rates, which were approximately 25% to 35%.

Under the market approach, our estimated enterprise value was developed based on a comparison of pre-money initial public offering, or IPO, values of recent biotechnology and emerging pharmaceutical companies at a similar stage of development to ours. When we achieved or exceeded a significant milestone, we reduced the discount rate applied to determine our enterprise value.

Once our enterprise value was established, an allocation method was used to allocate the enterprise value to the different classes of equity instruments. During our retrospective review, we used the probability weighted expected returns, or PWER, method to allocate our enterprise value to our common stock. Under the PWER method, the value of common stock is estimated based upon an analysis of future values for the enterprise assuming various future outcomes. In our situation, the future outcomes included two scenarios: (i) we become a public company and; (ii) we remain a private company. In general, the closer a company gets to an IPO, the higher the probability assessment weighting is for that scenario. We used a low probability assumption for our January 2005 grants and this percentage increased as significant milestones were achieved and as discussions with our investment bankers began and continued to increase as we prepared for our IPO process. An increase in the probability assessment for an IPO increases the value ascribed to our common stock.

For each of the two scenarios, estimated future and present value for the common shares were calculated using assumptions including:

- our expected pre-IPO valuation;
- a risk-adjusted discount rate associated with the IPO scenario;
- the liquidation preferences of our redeemable convertible preferred stock;
- appropriate discount for lack of marketability assuming we remained a private company;
- the expected probability of completing IPO versus remaining a private company; and
- the estimated timing of a potential IPO.

The increase in the fair value of our common stock for financial reporting purposes during 2005 and 2006 principally reflects a significant increase in our probability weighting for the IPO scenario and increases resulting from achieving significant clinical milestones.

The reassessed fair value for financial reporting purposes of common stock underlying 3,037,037 options granted to employees during the period from January 2005 through May 2005 was $0.31 per share. This valuation was attributable to the hiring of our President and Chief Executive Officer and other members of executive management and a relatively low probability estimate for the IPO scenario under the PWER method.

The reassessed fair value for financial reporting purposes of common stock underlying 1,768,748 options granted to employees during the period from June 2005 through July 2005 was determined to be $0.77 per share based on the ongoing clinical trial of Amigal, additional development of our preclinical programs, and an increased probability

estimate for the IPO scenario under the PWER method.

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The reassessed fair value for financial reporting purposes of common stock underlying 315,500 options granted to employees during the period from August 2005 through September 2005 was determined to be $0.95 per share. This increase in valuation was based on the completion of Phase I clinical trials for Amigal and completion of our series C redeemable convertible preferred stock financing of $55 million.

The reassessed fair value for financial reporting purposes of common stock underlying 2,351,000 options granted to employees during the period from October 2005 through November 2005 was determined to be $1.14 per share. This increase was primarily based on positive developments in the capital markets for early stage life science companies, the start of Phase II clinical trials for Amigal, and further preclinical development of our other programs.

The reassessed fair value for financial reporting purposes of common stock underlying 104,500 options granted to employees in December 2005 and 92,500 options granted to employees in the period from January 1, 2006 to February 22, 2006 was determined to be $1.44 per share. This increase was primarily based on preclinical development of AT2101 and AT2220, as well as an acceleration of our IPO planning.

The reassessed fair value for financial reporting purposes of common stock underlying 5,802,500 options granted to employees in the period from February 28, 2006 to March 27, 2006 was determined to be $1.84 per share. This increase was primarily based on initial data from our Phase II studies in Fabry disease and a further acceleration of our IPO timeline.

The intrinsic value of all outstanding vested and unvested options based on the estimated IPO price of $ was $ based on 14,573,975 options outstanding at March 31, 2006.

Results of Operations

Three Months Ended March 31, 2006 Compared to Three Months Ended March 31, 2005

Research and Development Expense. Research and development expense was $5.5 million for the three months ended March 31, 2006, an increase of $3.3 million, or 150%, from $2.2 million for the three months ended March 31, 2005. We attribute the increase primarily to a rise in contract research and manufacturing costs of $1.8 million due to our continued development of AT2101 and AT2220, and increases in personnel related costs of $1.1 million.

During the remainder of 2006, and thereafter, we expect research and development expenses to continue to increase substantially as our existing and future product candidates proceed through clinical trials. The timing and amount of these expenses will depend upon the outcome of our current clinical trials, particularly the costs associated with our current Phase II clinical trials of Amigal and Phase I clinical trial of AT2101 and our planned Phase I clinical trials of AT2101 and AT2220. The timing and amount of these expenses will also depend on the costs associated with potential future clinical trials of our product candidates, and the related expansion of our research and development organization, regulatory requirements, advancement of our preclinical programs and product candidate manufacturing costs.

General and Administrative Expense. General and administrative expense was $2.1 million for the three months ended March 31, 2006, an increase of $0.9 million, or 75%, from $1.2 million in for the three months ended March 31, 2005. The increase resulted primarily from an increase of personnel costs of $0.6 million attributable to increased headcount in finance, information technology, human resources, and general management and an increase of facility related expense of $0.4 million related to our new facility.

During the remainder of 2006, and thereafter, we expect our general and administrative expenses to increase substantially as we add personnel, increase investor relations activities, obtain insurance coverage appropriate for a public company, and become subject to public reporting obligations.

Interest Income and Interest Expense. Interest income was $238,000 in the three months ended March 31, 2006, compared to $57,000 in the three months ended March 31, 2005. Interest expense was $59,000 in the three months ended March 31, 2006, compared to $4,000 in the three months ended March 31, 2005. The increase in interest income resulted from higher average cash and cash equivalents

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balances and higher average interest rates in the 2006 period. The increase in interest expense resulted from a increase in our equipment financing and capital lease obligations as we continued to expand our business.

Year Ended December 31, 2005 Compared to Year Ended December 31, 2004

Research and Development Expense. Research and development expense was $13.7 million in 2005, an increase of $7.4 million, or 117%, from $6.3 million in 2004. The increase resulted primarily from an increase in contract research costs for Amigal, AT2101 and AT2220 of $3.5 million during 2005, and a rise in personnel related costs of $2.7 million.

General and Administrative Expense. General and administrative expense was $6.9 million in 2005, an increase of $4.8 million, or 228%, from $2.1 million in 2004. This increase is primarily attributable to a rise in salaries, as well as an increase in headcount in finance, human resources, information technology and general management, including the hiring of many of our current senior executives.

Interest Income and Interest Expense. Interest income was $610,000 in 2005, compared to $190,000 in 2004. Interest expense was $82,000 in 2005, compared to $550,000 in 2004. The increase in interest income resulted from higher average cash and cash equivalents balances and higher average interest rates in 2005. The reduction in interest expense resulted from the conversion of our bridge loans into series B redeemable convertible preferred stock during 2004.

Tax Benefit. In 2005 and 2004, we recognized tax benefits related to our sale of net operating losses in the New Jersey Tax Transfer Program. Our tax benefit was $612,000 in 2005 and $83,000 in 2004. We sold $6.7 million and $1.1 million of net operating losses in 2005 and 2004, respectively.

Year Ended December 31, 2004 Compared to Year Ended December 31, 2003

Research and Development Expense. Research and development expense was $6.3 million in 2004, an increase of $1.9 million, or 42%, from $4.4 million in 2003. The increase resulted principally from an increase in contract research costs attributable to preclinical development activities for Amigal and AT2101 of $1.5 million, and an increase in personnel costs of $0.2 million.

General and Administrative Expense. General and administrative expense was $2.1 million in 2004, an increase of $1.0 million, or 110%, from $1.0 million in 2003. The increase resulted principally from an increase in personnel costs of $0.1 million attributable to a rise in salaries and increased headcount, greater legal and consulting expense of $0.4 million, and an increase in miscellaneous corporate expenses of $0.2 million.

Interest Income and Interest Expense. Interest income was $190,000 in 2004, compared to $5,000 in 2003. Interest expense was $550,000 in 2004, compared to $172,000 in 2003. The increase in interest income resulted from higher average cash and cash equivalents balances and higher average interest rates in 2004. The increase in interest expense resulted from bridge loans issued in 2003 and early 2004 which were converted to series B redeemable convertible preferred stock during 2004.

Tax Benefit. The tax benefit related to our sale of $1.1 million of net operating losses in the New Jersey Tax Transfer Program was $83,000 in 2004.

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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 $61.0 million of proceeds from redeemable convertible preferred stock offerings through March 31, 2006 and additional $27.5 million of proceeds from redeemable convertible preferred stock offerings in April 2006. The following table summarizes our funding sources inclusive of our April 2006 issuance of an additional 21,825,131 shares of series C redeemable convertible preferred stock.

Issue	Year	No. Shares	Approximate Amount(1)
			(in millions)
Series A Redeemable Convertible Preferred Stock	2002	3,333,334	$ 2.5
Series B Redeemable Convertible Preferred Stock	2004, 2005	36,470,591	31.0
Series C Redeemable Convertible Preferred Stock	2005, 2006	43,650,262	55.0
Total		83,454,187	$ 88.5

(1) Represents gross proceeds.

As of March 31, 2006, we had cash and cash equivalents and marketable securities of $19.6 million. An additional $27.5 million of cash was raised in connection with our April 2006 sale of series C redeemable convertible preferred stock. 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.

Cash Flows

Net cash used in operations was $2.9 million and $6.1 million for the three months ended March 31, 2005 and 2006, respectively. The net loss for the three months ended March 31, 2006 of $7.6 million was offset primarily by non-cash charges for depreciation and amortization of $0.2 million, stock-based compensation of $0.3 million, $0.1 of non-cash compensation issued to consultants, partially offset by changes in operating assets and liabilities of $0.8 million. Net cash generated in investing activities for the three months ended March 31, 2006 was $8.1 million and consisted primarily of proceeds from the sale of marketable securities, partially offset by $0.6 million of capital expenditures. Net cash provided by financing activities for the three months ended March 31, 2006 was $1.9 million, consisting primarily of $2.0 million of proceeds from our capital asset financing arrangement, offset primarily by payments of equipment debt financing obligations of $0.2 million.

Net cash used in operations was $9.2 million and $18.1 million for the years ended December 31, 2004 and 2005, respectively. The net loss for 2005 of $19.7 million was partially offset by $0.1 million of non-cash stock issued to consultants, $0.3 million of depreciation and amortization, and $0.4 million amortization of non-cash compensation, and a net change in operating assets and liabilities of $0.8 million. Net cash used from investing activities for the year ended December 31, 2005 was $16.9 million and consisted primarily of $17.0 million of purchases of marketable securities and $3.0 million of equipment purchases, partially offset by the sale and redemption of marketable securities for $3.1 million. Net cash from financing activities for 2005 was $41.3 million, which consists primarily of net proceeds from the issuance of series B redeemable convertible preferred stock of $13.0 million and net proceeds from the issuance of series C redeemable convertible preferred stock of $27.3 million.

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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 timing and outcome of clinical trials and regulatory approvals, 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, and our success in developing markets for our product candidates.

We believe that the net proceeds from this offering, 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 . We believe that if we sell the shares of our common stock in this offering at an initial public offering price of $ per share ($1.00 lower than the mid-point of the price range set forth on the cover page), the resultant reduction in proceeds we receive from the offering would cause us to require additional capital earlier. 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.

Our future capital requirements will depend on many factors, including 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, and the costs of commercialization activities, including product marketing, sales and distribution.

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.

To the extent our capital resources are insufficient to meet our future capital requirements, 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 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.

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Contractual Obligations

The following table summarizes our significant contractual obligations and commercial commitments at March 31, 2006 and the effects such obligations are expected to have on our liquidity and cash flows in future periods.

	Total	Remainder of 2006	2007-2009	2010-2011	2012
Operating lease obligations	$ 8,596	$ 1,019	$ 4,392	$ 2,940	$ 245
Capital lease obligations	3,341	842	2,491	8	
Total fixed contractual obligations	$ 11,937	$ 1,861	$ 6,883	$ 2,948	$ 245

In May 2005, we entered into a seven-year non-cancelable operating lease agreement for office and laboratory space in Cranbury, New Jersey. The operating lease will expire by its terms on February 28, 2012.

In August 2002, we entered into capital lease agreements that provide for up to $1.0 million of equipment financing through August 2004. The facility was increased to $3.0 million in May of 2005 and to $5.0 million in November 2005. These financing arrangements include interest of approximately 9-12%, and lease terms of 36 or 48 months. Eligible assets under the lease lines include laboratory and scientific equipment, computer hardware and software, general office equipment, furniture, and tenant improvements. Upon termination of the lease agreements, we may renew the lease or purchase the leased equipment for $1.00. We also have the option to purchase the equipment at set prices before termination of the lease. In addition, at lease inception, we issued a warrant to the equipment financing lender to purchase 40,000 shares of common stock. The warrant was valued at $8,000 using a Black-Scholes option pricing model and this value was amortized to interest.

We have entered into agreements with clinical research organizations and other outside contractors who will be partially responsible for conducting and monitoring our clinical trials for Amigal and AT2101 as well as preclinical studies of AT2220. These contractual obligations are not reflected in the table above because we may terminate them without penalty.

Except for the capital lease agreements described above, we have no other lines of credit or other committed sources of capital. To the extent our capital resources are insufficient to meet future capital requirements, we will need to raise additional capital or incur indebtedness to fund our operations. We cannot assure you that additional debt or equity financing will be available on acceptable terms, if at all.

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 March 31, 2006, we had cash and cash equivalents and marketable securities of $19.6 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.

Effects of Inflation

Inflation generally affects us by increasing our cost of labor and clinical trial costs. We do not believe that inflation has had a material effect on our results of operations during 2003, 2004, 2005 or for the three months ended March 31, 2006.

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Off-Balance Sheet Arrangements

We have no off-balance sheet arrangements as of December 31, 2004, 2005 and March 31, 2006.

Recent Accounting Pronouncements

In February 2006, FASB, issued SFAS No. 155, *Accounting for Certain Hybrid Instruments*, or SFAS 155. SFAS 155 allows financial instruments that have embedded derivatives to be accounted for as a whole (eliminating the need to bifurcate the derivative from its host) if the holder elects to account for the whole instrument on a fair value basis. This statement is effective for all financial instruments acquired or issued after the beginning of an entity s first fiscal year that begins after September 15, 2006. We believe the adoption of SFAS 155 will not have a material impact on our financial statements.

In May 2005, FASB issued SFAS No. 154, *Accounting Changes and Error Corrections*, or SFAS 154, a replacement of APB No. 20, *Accounting Changes*, and SFAS No. 3, *Reporting Accounting Changes in Interim Financial Statements*. SFAS 154 applies to all voluntary changes in accounting principle and changes the requirements for accounting for and reporting of a change in accounting principle. This statement establishes that, unless impracticable, retrospective application is the required method for reporting a change in accounting principle in the absence of explicit transition requirements specific to the newly adopted accounting principle. It also requires the reporting of an error correction which involves adjustments to previously issued financial statements similar to those generally applicable to reporting an accounting change retrospectively. SFAS 154 is effective for accounting changes and corrections of errors made in fiscal years beginning after December 15, 2005.

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BUSINESS

Overview

We are a clinical-stage biopharmaceutical company focused on the discovery, development and commercialization of a new class of orally-administered, small molecule drugs, known as pharmacological chaperones, for the treatment of a range of human genetic diseases. Our lead product development programs are Amigal for Fabry disease, AT2101 for Gaucher disease and AT2220 for Pompe disease. We are currently conducting Phase II clinical trials of Amigal and have initiated a Phase I trial for AT2101. We expect to file an IND for AT2220 by the end of 2006. We recently observed results from the first four patients treated with Amigal in our Phase II trial. We hold worldwide commercialization rights to Amigal, AT2101 and AT2220 and we intend to establish a commercial infrastructure and targeted sales force to market some or all of our products.

Certain human diseases result from mutations in specific genes that, in many cases, lead to the production of proteins with reduced stability. Proteins with these mutations may not achieve 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, are eliminated prior to reaching their intended location in the cell. The reduced or completely absent 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 a new type of drug, which we refer to as a pharmacological chaperone, that selectively binds to the target protein, which increases the stability of the protein and helps it fold into the correct three-dimensional shape. This restores appropriate trafficking of the protein, thereby increasing protein activity, improving cellular function and reducing stress on cells.

Current treatment for some of these genetic diseases consists of compensating for the reduced or missing protein activity through regular infusions with large quantities of protein. Instead of adding protein from an external source by intravenous infusion, which is called enzyme replacement therapy, our approach utilizes orally-administered, small molecule pharmacological chaperones to improve the function of the patient s own protein. Our approach to the treatment of human genetic diseases is novel and has the potential to improve the treatment of these diseases. In addition, we believe our technology is broadly applicable to diseases that have been linked to misfolded proteins, including certain types of neurological disease, metabolic disease, cardiovascular disease and cancer.

Our Lead Programs

Our three most advanced product development programs target lysosomal storage disorders, which are chronic genetic diseases that frequently result in severe symptoms. Each of these disorders results from the deficiency of a single enzyme.

Amigal for Fabry disease. We are developing Amigal for the treatment of patients with Fabry disease and are currently conducting multiple Phase II clinical studies. We expect to complete enrollment in these studies by the end of 2006 and, assuming positive results from these studies, to initiate a Phase III study in 2007.

AT2101 for Gaucher disease. We are developing AT2101 for the treatment of Gaucher disease. We have filed an IND for AT2101 and have initiated a Phase I study. If these studies are successful, we plan to initiate a Phase II study in the first half of 2007.

AT2220 for Pompe disease. We are developing AT2220 for the treatment of Pompe disease. We plan to file an IND for AT2220 by the end of 2006.

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Our Pharmacological Chaperone Technology

In the human body, proteins are involved in almost every aspect of cellular function. Proteins are linear strings of amino acids that fold and twist into specific three-dimensional shapes in order to function properly. Certain human diseases result from mutations in specific genes that lead to the production of misfolded proteins. The majority of genetic mutations that cause misfolded proteins are called missense mutations. These mutations result in the substitution of a single amino acid for another in the protein. Because of this error, missense mutations often result in proteins that have a reduced level of biological activity. In addition to missense mutations, there are also other types of genetic mutations that can result in proteins with reduced biological activity.

Proteins generally fold in a specific region of the cell known as the endoplasmic reticulum, or ER. The cell has quality control mechanisms that ensure that proteins are folded into their correct three-dimensional shape before they can move from the ER to the appropriate destination in the cell, a process generally referred to as protein trafficking. Misfolded proteins are often eliminated by the quality control mechanisms after initially being retained in the ER. In certain instances, misfolded proteins can accumulate in the ER instead of being eliminated.

The retention of misfolded proteins in the ER interrupts their proper trafficking, and the resulting reduced biological activity can lead to impaired cellular function and ultimately to disease. In addition, the accumulation of misfolded proteins in the ER may lead to various types of stress on cells, which may also contribute to cellular dysfunction and disease.

At Amicus, we have developed a novel approach to address human genetic diseases resulting from misfolded proteins. We use small molecule drugs, which are called pharmacological chaperones, to selectively bind to a target protein and increase its stability. The binding of the chaperone molecule helps the protein fold into its correct three-dimensional shape. This allows the protein to be trafficked from the ER to the appropriate location in the cell, thereby increasing protein activity and cellular function and reducing stress on cells.

Pharmacological chaperones represent a new way of affecting specific proteins, improving cellular function and treating disease. Our proprietary approach to the discovery of pharmacological chaperone drug candidates entails the use of rapid molecular and cell-based screening technology combined with our understanding of the intended biological function of proteins implicated in disease. We use this knowledge to select and develop compounds with optimized properties. In many cases, we are able to start with specific molecules and classes of compounds already known to interact with the target protein but not used previously as therapies. This can greatly reduce the time and cost of the early stages of drug discovery and development.

We believe that our pharmacological chaperone technology may be applicable to many types of diseases that involve misfolded proteins. In particular, pharmacological chaperone therapies could, in our view, provide a benefit in areas such as neurological disease, metabolic disease, cardiovascular disease and cancer, the causes of which have been linked to various misfolded proteins. We are also exploring other applications in which the ability of pharmacological chaperones to increase the activity of normal proteins may provide a therapeutic benefit.

Potential Advantages of Pharmacological Chaperones for the Treatment of Lysosomal Storage Disorders

To date, we have focused on developing pharmacological chaperones for the treatment of lysosomal storage disorders. Lysosomal storage disorders are a type of metabolic disorder characterized by mutations in lysosomal enzymes, which are specialized proteins that break down cellular substrates in a part of the cell called the lysosome. Substrates are byproducts of cellular metabolism.

The current therapeutic standard of care for the most common lysosomal storage disorders is enzyme replacement therapy. Enzyme replacement therapy involves regular infusions to compensate for the deficient lysosomal enzyme. A therapeutic alternative involving the use of small molecules is substrate reduction therapy. We believe that pharmacological chaperone therapy may have advantages relative to

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these other therapeutic approaches for the treatment of lysosomal storage disorders. The following table compares enzyme replacement therapy to pharmacological chaperone therapy.

Product Characteristic	Enzyme Replacement Therapy	Pharmacological Chaperone Therapy
Biodistribution	Variable tissue distribution	Broad tissue distribution, including brain
Treatment effect	Reduce substrate accumulation	Reduce substrate accumulation Reduce accumulation of misfolded protein
Ease of Use	Weekly or every other week intravenous infusion	Oral administration
Manufacturing	Recombinant protein manufacturing	Chemical synthesis

In addition, we believe our pharmacological chaperone therapies may have advantages relative to substrate reduction therapy. Substrate reduction therapy uses orally-administered small molecules; however, the underlying mechanism of action is very different than for pharmacological chaperones. Substrate reduction therapies are designed to prevent the production of the substrate that accumulates in disease by inhibiting an enzyme required to make the substrate in cells. This is not the same enzyme that is deficient in the disease. Importantly, the enzyme that is inhibited is needed to make other molecules that are used in many biological processes. As a result, inhibiting this enzyme may have adverse effects on the cell that are difficult to predict. By contrast, our pharmacological chaperone therapies are designed to work by binding directly to the enzyme deficient in the disease, increasing its stability and helping it fold into its correct three-dimensional shape. This in turn enables proper trafficking to the lysosome where the enzyme can directly decrease substrate accumulation.

To date, one substrate reduction therapy product has received regulatory approval in the United States and the European Union for the treatment of one lysosomal storage disorder. Zavesca, a substrate reduction therapy product commercialized by Actelion, Ltd., is approved for the treatment of Gaucher disease in the United States, the European Union and other countries.

Our Lead Product Candidates

The following table summarizes key information about our product candidates. All of our current product candidates are orally-administered, small molecules based on our pharmacological chaperone technology.

Product Candidate Indication	Stage of Development	Worldwide Commercial Rights
Amigal *Fabry Disease*	Phase II	Amicus
AT2101 *Gaucher Disease*	Phase I	Amicus
AT2220 *Pompe Disease*	Preclinical	Amicus

Amigal for Fabry Disease

Overview

Our most advanced product candidate, Amigal, is an orally-administered, small molecule for the treatment of Fabry disease. We are currently conducting Phase II clinical studies. Administration of Amigal to the first four

patients in one of these studies, Study 201, resulted in an average five-fold

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increase in enzyme activity in white blood cells after six weeks of treatment. After 12 weeks of treatment, enzyme activity levels remained elevated. The levels of GL-3 measured in patient plasma, urine and skin, both before treatment and after 12 weeks of treatment, were in the range of healthy individuals. GL-3, a complex lipid called globotriaosylceramide, is the substrate broken down by the enzyme deficient in Fabry disease. Assuming the successful completion of our current Phase II clinical studies, we expect to initiate a Phase III clinical study of Amigal in 2007. In February 2004, the FDA granted orphan drug designation to Amigal for the treatment of Fabry disease and in March 2006, the European Medicines Agency, or EMEA, recommended orphan medicinal product designation for Amigal.

Causes of Fabry Disease and Rationale for Use of Amigal

Fabry disease is a lysosomal storage disorder resulting from a deficiency in a key metabolic enzyme, alpha-galactosidase A, or -GAL. The deficiency of -GAL in Fabry patients is caused by inherited genetic mutations. Certain of these mutations cause changes in the amino acid sequence of -GAL that may result in the production of -GAL with reduced stability that does not achieve its correct three-dimensional shape. Although misfolded -GAL often retains the potential for some level of biological activity, the cell s quality control mechanisms recognize and retain misfolded -GAL in the ER until it is ultimately moved to another part of the cell for degradation and elimination. Consequently, little or no -GAL moves to the lysosome, where it normally breaks down GL-3. This leads to accumulation of GL-3 in cells, which is believed to contribute to most of the complications associated with Fabry disease. In addition, accumulation of the misfolded -GAL enzyme in the ER may lead to stress on cells and inflammatory-like responses, which may contribute to cellular dysfunction and disease. Symptoms can be severe and debilitating, including dysfunction of major organs such as the heart, kidneys and brain, leading to cardiac disease, renal failure and strokes.

Amigal is designed to act as a pharmacological chaperone for -GAL by selectively binding to the enzyme, which increases its stability and helps the enzyme fold into its correct three-dimensional shape. This stabilization of -GAL allows the cell s quality control mechanisms to recognize the enzyme as properly folded so that trafficking of the enzyme to the lysosome is increased, enabling it to carry out its intended biological function, the metabolism of GL-3. As a result of restoring the proper trafficking of -GAL from the ER to the lysosome, Amigal reduces the accumulation of misfolded protein in the ER, which may alleviate stress on cells and some inflammatory-like responses that may be contributing factors in Fabry disease.

Because Amigal works by increasing the activity of a patient s naturally produced -GAL, those Fabry disease patients with a missense mutation or other genetic mutation that results in production of -GAL that is less stable but with some residual enzyme activity are the ones most likely to respond to treatment with Amigal. We estimate that the majority of patients with Fabry disease may respond to pharmacological chaperone therapy. Patients with genetic mutations leading to a partially made -GAL enzyme or -GAL enzyme with an irreversible loss of activity are less likely to respond to treatment with Amigal.

Fabry Disease Background

The clinical manifestations of Fabry disease span a broad continuum of severity and roughly correlate with a patient s level of residual -GAL activity. The majority of currently treated patients are referred to as classic Fabry disease patients, most of whom are males. These patients experience disease of various organs, including the kidneys, heart and brain, with disease symptoms first appearing in adolescence and typically progressing in severity until death in the fourth or fifth decade of life. A number of recent studies suggest that there are a large number of undiagnosed male and female patients, referred to as later-onset Fabry disease patients, with higher levels of residual -GAL activity than classic Fabry disease patients. Later-onset Fabry disease patients have a broad range of disease symptoms, such as impaired cardiac function, stroke or renal failure, that usually first appear in adulthood. Although Fabry disease should be thought of as a continuum, it is useful to classify patients as having classic or later-onset Fabry disease when discussing the disease and its market opportunity.

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Classic Fabry Disease

Patients with classic Fabry disease are in most instances males. They have little or no detectable -GAL activity and are the most severely affected. These patients first experience disease symptoms in adolescence, including pain and tingling in the extremities, skin lesions, a decreased ability to sweat and clouded eyes. If these patients are not treated, their life expectancy is reduced and death usually occurs in the fourth or fifth decade of life from renal failure, cardiac dysfunction or stroke. Studies reported in JAMA (January 1999) and The Metabolic and Molecular Bases of Inherited Disease (8th edition 2001) suggest the annual incidence of Fabry disease in newborn males is 1:40,000-1:60,000. Current estimates from the University of Iowa and the National Kidney Foundation suggest that there are a total of approximately 5,000 classic Fabry disease patients worldwide.

Later-onset Fabry Disease

Patients with later-onset Fabry disease can be male or female. These patients typically first experience disease symptoms in adulthood, and often have disease symptoms focused on a single organ. For example, many males and females with later-onset Fabry disease have enlargement of the left ventricle of the heart. As the patients advance in age, the cardiac complications of the disease progress and can lead to death. Studies reported in Circulation, Journal of the American Heart Association (March 2002 and August 2004), estimate that 6-12% of patients in the 40 to 60 year age range who have an unexplained enlargement of the left ventricle of the heart, a condition referred to as left ventricular hypertrophy, have Fabry disease.

A number of males and females also have later-onset Fabry disease with disease symptoms focused on the kidney that progress to end stage renal failure and eventually death. Studies reported in Nephrology Dial Transplant (2003), Clinical and Experimental Nephrology (2005) and Nephrology Clinical Practice (2005) estimate a general range of 0.20-0.94% of the patients on dialysis have Fabry disease.

In addition, later-onset Fabry disease may also present in the form of strokes of unknown cause. A recent published study reported in The Lancet (November 2005) found that approximately 4% of 721 male and female patients in Germany between the ages of 18 to 55 had Fabry disease with stroke of unknown cause.

It was previously believed that it was rare for female Fabry disease patients to develop overt clinical manifestations of Fabry disease. However, several studies reported in the Journal of Medical Genetics (2001), the Internal Medicine Journal (2002) and the Journal of Inherited Metabolic Disease (2001), each of which is summarized on the website of the Mount Sinai School of Medicine, Department of Human Genetics, report that the majority of female Fabry disease patients have mild symptoms and that other female Fabry disease patients have severe symptoms, including enlargement of the left ventricle of the heart and/or renal failure. Fabry disease is X-linked, which means that an X chromosome containing an -GAL gene mutation is inherited. Females inherit an X chromosome from each parent and therefore can inherit a Fabry mutation from either parent. By contrast, males inherit an X chromosome (and potentially a Fabry mutation) only from their mothers. For this reason, there are expected to be roughly twice as many female Fabry disease patients as male Fabry disease patients.

Fabry Disease Market Opportunity

Fabry disease is a relatively rare disorder. The current estimates of approximately 5,000 patients worldwide are generally based on a small number of studies in single ethnic populations in which people were screened for classic Fabry disease. The results of these studies were subsequently extrapolated to the broader world population assuming similar prevalence rates across populations. We believe these previously reported studies did not account for the prevalence of later-onset Fabry disease and, as described above, a number of recent studies suggest that the prevalence of Fabry disease could be many times higher than previously reported.

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We expect that as awareness of later-onset Fabry disease grows, the number of patients diagnosed with Fabry disease will increase. Increased awareness of all forms of Fabry disease, particularly for specialists not accustomed to treating Fabry disease patients, may lead to increased testing and diagnosis of patients with the disease. We intend to develop and launch educational and awareness campaigns targeting cardiologists, nephrologists and neurologists regarding Fabry disease and its under-diagnosis. Assuming we receive regulatory approval, we expect these educational and awareness campaigns would continue as a part of the marketing of Amigal. In order to facilitate the proper diagnosis of Fabry disease patients seen by these specialist physicians, we intend to provide support for testing for the disease, which is performed using a simple blood test for the level of -GAL activity.

Based on published data from the Human Gene Mutation Database and our experience in the field, we believe the majority of the known genetic mutations that cause Fabry disease are missense mutations. There are few widely-occurring genetic mutations reported for Fabry disease, suggesting that the frequency of a specific genetic mutation reported in the Human Gene Mutation Database reflects the frequency of that mutation in the general Fabry patient population. In addition, data presented at the 11th International Conference on Health Problems Related to the Chinese (2002) show that the vast majority of newly diagnosed patients with later-onset Fabry disease also have missense mutations. Because missense mutations often result in less stable, misfolded -GAL with some residual enzyme activity, we believe patients with these mutations are candidates for treatment with Amigal. We also believe that other types of genetic mutations result in misfolded -GAL and therefore may respond to treatment with Amigal. Based on this, we believe that a majority of the Fabry disease patient population may benefit from treatment with Amigal.

Existing Products for the Treatment of Fabry Disease and Potential Advantages of Amigal

Prior to the availability of enzyme replacement therapy, treatments for Fabry disease were directed at ameliorating symptoms without treating the underlying disease. Some of these treatments include opiates, anticonvulsants, antipsychotics and antidepressants to control pain in the extremities and beta-blockers, calcium channel blockers, ACE inhibitors, angiotensin receptor antagonists and other agents to treat blood pressure and vascular disease.

The current standard of treatment for Fabry disease is enzyme replacement therapy. There are currently two products approved for the treatment of Fabry disease. One of the products is Fabrazyme, a product approved globally and commercialized by Genzyme Corporation. Fabrazyme was approved in the United States in 2003 and has orphan drug exclusivity in the United States until 2010. It was approved in the European Union in 2001 and has orphan drug exclusivity in the European Union until 2011. The other product approved for treatment of Fabry disease is Replagal, a product approved in the European Union and other countries but not in the United States, commercialized by Shire PLC. Replagal was approved in the European Union in August 2001 and has orphan drug exclusivity in the European Union until 2011. The net product sales of Fabrazyme and Replagal for 2005 were approximately $305 million and $95 million, respectively, as publicly reported by Genzyme Corporation and Shire PLC, respectively.

For Fabry disease patients who respond to Amigal, we believe that the use of Amigal may have advantages relative to the use of Fabrazyme and Replagal. Published data for patients treated with Fabrazyme and Replagal for periods of up to five years demonstrate that these drugs can lead to the reduction of GL-3 in the cells that line the blood vessels in the kidneys of Fabry disease. Because they are large protein molecules, Fabrazyme and Replagal are believed to have difficulty penetrating many tissues and cell types. In particular, it is widely believed that Fabrazyme and Replagal are unable to cross the blood-brain barrier and thus are unlikely to address the neurological symptoms of Fabry disease. As a small molecule therapy that has demonstrated high oral bioavailability and good biodistribution properties in preclinical testing, Amigal has the potential to reach cells of all the target tissues of Fabry disease. Furthermore, treatment with Fabrazyme and Replagal requires intravenous infusions every other week, frequently on site at health care facilities, presenting an inconvenience to Fabry patients. Oral treatment with Amigal may be much more convenient for patients and may not have the safety risks associated with

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intravenous infusions. See Potential Advantages of Pharmacological Chaperones in the Treatment of Lysosomal Storage Disorders.

In February 2004, we were granted orphan drug designation by the FDA for Amigal for treatment of Fabry disease and in March 2006 the EMEA recommended orphan medicinal product designation for Amigal. We believe that orphan drug designation of Fabrazyme in the United States and of Fabrazyme and Replagal in the European Union will not prevent us from obtaining marketing approval of Amigal in either geography. See Government Regulation.

Amigal Development Activities

Preclinical Activities

We have conducted multiple in vitro and in vivo preclinical studies of Amigal. Key findings of our studies include:

Amigal increased -GAL enzyme activity in cells derived from a variety of different Fabry disease patients. Over 75 different -GAL missense mutations have been examined in cell culture assays with the majority showing an increase in -GAL enzyme activity after incubation with Amigal for several days.

Treatment of normal mice and mice that produce defective human -GAL resulted in a dose-dependent increase in -GAL enzyme activity in a variety of tissues including liver, heart, kidney and spleen. The table below summarizes the results of treatment with Amigal in mice that produce a defective human -GAL.

Note: Error bars indicate standard error of the mean.

Amigal had an acceptable toxicity profile when tested at high exposure levels in rats, dogs and monkeys. Amigal showed no signs of systemic toxicity in two-week studies in rats, dogs and monkeys, in six-month studies in rats and in nine-month studies in monkeys when tested at levels that were well above those that we are studying in our current Phase II clinical trials. In the nine-month monkey study, all doses were well tolerated and showed no signs of toxicity.

Some treatment-related impacts on reproduction and fertility have been observed in rabbit and rat studies. At high exposure levels that were well above those that we are studying in our current Phase II clinical trials, maternal toxicity studies in rabbits showed a dose-related increase in embryonic death, a reduction in fetal weight, delayed bone development and slightly increased incidences of other minor skeletal abnormalities. These effects were not seen in rats. At exposure levels within the range of those we are studying in our current Phase II clinical trials, male rats experienced infertility, which was completely reversible within four weeks after discontinuation of treatment. No treatment-related changes have been detected in the male rat reproductive organs or sperm to account for the infertility and no mechanism of

action has been established to explain this effect. The implications for humans, if any, of these treatment-related reproductive and fertility effects in rabbit and rat studies are unknown at this time.

Phase I Clinical Trials

We have completed three Phase I clinical studies of Amigal in a total of 63 healthy volunteers, of which 51 were treated with Amigal and 12 were given placebo.

Single Dose Phase I Study. Our single dose Phase I study was a single center, randomized, dose ranging study in healthy volunteers. The clinical phase began in July 2004 and was completed in November 2004. The study consisted of a total of 32 healthy volunteers divided into four groups of eight subjects. Six subjects in each group received Amigal and two subjects received placebo. All subjects received single doses of 25 mg, 75 mg, 225 mg or 675 mg of Amigal and were evaluated on Day 1 and on Day 8. The primary objective of the study was to evaluate the safety and pharmacokinetics of Amigal in healthy volunteers.

Multi-Dose Phase I Study. Our multi-dose Phase I study was a single center, randomized, dose ranging study in healthy volunteers. The clinical phase began in December 2004 and was completed in January 2005. The study consisted of a total of 16 healthy volunteers divided into two groups of eight subjects. Six subjects in each group received Amigal and two subjects received placebo. All subjects in one group received 50 mg twice a day for seven days, and all subjects in the other group received 150 mg twice a day for seven days. Subjects were evaluated at the beginning of the study, on Day 7 after seven days of treatment and on Day 14 after a seven day washout period. The primary objectives of the study were to evaluate the safety and pharmacokinetics of Amigal in healthy volunteers and to measure the level of -GAL enzyme activity in white blood cells of healthy volunteers treated with Amigal.

Absorption Phase I Study. Our absorption Phase I study was a single center, randomized, three-way crossover, three-sequence, comparative study in healthy volunteers. The clinical phase began in August 2005 and was completed in September 2005. The study consisted of a total of 15 healthy volunteers divided into three groups of five subjects. All subjects in a group received a single dose of 100 mg of Amigal as a capsule while fasting, as a capsule after a meal or as a solution while fasting. After being evaluated on Day 1 and on Day 8, patients in each group crossed over to one of the other treatments. This was repeated again after the second treatment so that each group of patients received each of the three treatments. The primary objective of the study was to evaluate the bioequivalency between solution and capsule forms and the effect of food on absorption from the capsule.

The data from our three Phase I clinical studies in healthy volunteers showed that Amigal was well tolerated, even at the highest doses, without any drug related adverse effects. The studies also demonstrate that Amigal has high oral bioavailability, good pharmacokinetics with a half-life in plasma of approximately three to four hours and that the drug should be taken without food.

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In addition, the data from the multi-dose Phase I study showed a dose-related increase in the level of -GAL activity in the white blood cells of healthy volunteers. The results are summarized below in the following graph.

Note: Error bars indicate standard error of the mean.

We believe these Phase I results are the first demonstration of an increase in enzyme activity in humans following oral administration of a pharmacological chaperone. In normal, healthy individuals treated with Amigal for seven days we observed a dose-related increase in enzyme activity, with the increase maintained for at least seven days after the last dose. We believe normal enzyme activity can be increased because some fraction of normal protein molecules can also misfold and fail to pass the cell s quality control mechanisms. Normal -GAL is stabilized by binding to the pharmacological chaperone, which results in an increase in the amount successfully trafficked to the lysosome. We believe the sustained elevation of enzyme activity levels following discontinuation of treatment occurs because the enzyme is stable for many days once it reaches the lysosome.

Phase II Clinical Trials

We are conducting open-label Phase II clinical studies in up to 48 adult male and female patients with Fabry disease. These studies may enroll patients that have classic Fabry disease, as well as patients with later-onset Fabry disease, including females and patients with cardiac symptoms.

In order to qualify for these clinical studies, patients must have a confirmed diagnosis of Fabry disease with a documented missense mutation in -GAL and a positive result in an in vitro test of -GAL enzyme activity enhancement with Amigal. This in vitro test requires a simple blood draw and consists of incubation of a patient s cells derived from white blood cells, with and without Amigal for a period of time followed by measurement of -GAL enzyme activity. For entry into the Phase II clinical studies, patients must have a baseline -GAL activity level in white blood cells of at least 3% of normal and have cells derived from white blood cells that show a relative increase of at least 20% in -GAL activity after cell culture incubation with Amigal.

We expect to complete enrollment of our current Phase II studies by the end of 2006.

Phase II Study 201. We are conducting a Phase II study in which four patients are currently enrolled. The first patient was enrolled in January 2006 and the study is expected to complete enrollment by the end of 2006. The study consists of treatment with Amigal for a period of twelve weeks with a possible extension up to 48 weeks in up to 20 male Fabry disease patients that are naïve to enzyme replacement therapy or have not had enzyme replacement therapy for at least one month. All four patients have received 25 mg of Amigal twice a day for two weeks, followed by

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100 mg of Amigal twice a day for two weeks, followed by 250 mg of Amigal twice a day for two weeks and followed by 25 mg of Amigal twice a day for six weeks. These patients are currently receiving 25 mg of Amigal twice a day for the extension phase of this study. Based on the -GAL activity data observed in the first four patients after 12 weeks of treatment with Amigal, we may amend this protocol to replace the in vitro assay screening criteria with an in vivo drug exposure in which patients would be given Amigal for a short period and then tested to determine if -GAL activity has increased. We believe this would allow us to enroll a larger segment of patients with mutations likely to respond to treatment with Amigal. We are also considering modifying the dosing regimen for this study.

Phase II Study 202. We are conducting a Phase II study in which we are seeking to enroll patients. The study consists of treatment with Amigal for a period of 24 weeks with a possible extension to 48 weeks in up to eight male Fabry disease patients that are naïve to enzyme replacement therapy. All patients will receive 150 mg of Amigal every other day during the duration of the study.

Phase II Study 203. We are conducting a Phase II study in which we are seeking to enroll patients. The study consists of treatment with Amigal for a period of 12 weeks with a possible extension to 48 weeks in up to eight male Fabry disease patients that are naïve to enzyme replacement therapy. All patients will receive 150 mg of Amigal every other day during the duration of the study.

Phase II Study 204. We are conducting a Phase II study in which we are seeking to enroll patients. The study consists of treatment with Amigal for a period of 12 weeks with a possible extension to 48 weeks in up to 12 female Fabry disease patients that are naïve to enzyme replacement therapy. Patients will receive 50 mg, 150 mg or 250 mg doses of Amigal every other day for 12 weeks. If the patient participates in the extension phase, the dose for the extension will be determined based on data from the first 12 weeks.

The primary objective of the Phase II clinical studies is to evaluate the safety and tolerability of Amigal in patients with Fabry disease. The secondary objective is to evaluate certain pharmacodynamic measures of treatment with Amigal. These pharmacodynamic measures consist of the following:

-GAL activity in white blood cells and skin biopsies;

-GAL activity in heart and kidney biopsies (not performed in Study 201);

GL-3 in plasma, urine and skin biopsies; and

GL-3 in heart and kidney biopsies (not performed in Study 201).

An additional objective is the preliminary assessment of Amigal s impact on cardiac, renal and central nervous system function in Fabry disease patients.

Initial Results of Phase II Study 201

The initial data from the first four Fabry disease patients enrolled in Study 201 showed that the -GAL enzyme activity in white blood cells after six weeks of treatment was on average more than five-fold higher than before treatment. The four patients had three different genetic mutations and we observed

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an increase in the level of -GAL enzyme activity in all of these patients. The graph below summarizes these data.

Note: Error bars indicate standard error of the mean.

After six weeks of treatment, in accordance with the protocol, the dose was decreased to the same 25 mg of Amigal used in the first two weeks of the study. Patients received this lower dose for six weeks. An analysis of the 12 week data from the same four patients has been completed and a summary is provided below:

- Enzyme activity in white blood cells remained elevated at levels approximately four-fold higher than baseline. Enzyme activity in skin was increased in two of the four patients. Results of -GAL enzyme activity levels in skin of the other two patients were inconclusive due to insufficient biopsy sample size.

- GL-3 levels in patient plasma, urine and skin, both before and after 12 weeks of treatment, were in the normal range of healthy individuals.

- Measures of cardiac, renal and central nervous system function before treatment were normal or near normal, and no clinically meaningful changes were observed after 12 weeks of treatment.

To enroll in this clinical study, patients were required to have greater than or equal to 3% of normal -GAL enzyme activity level in white blood cells. We believe that this residual level of -GAL activity is responsible for the normal levels of GL-3 in the plasma, urine and skin of these patients, before treatment with Amigal and thus why levels were unchanged after treatment. We believe the results from the 12 weeks of treatment of the first four patients enrolled in Study 201 support the continuation of our current Phase II clinical studies. It is generally believed that even small increases in lysosomal enzyme activity may have clinical benefits.

Amigal was well-tolerated with no reported serious adverse events. Adverse events were mostly mild and reported by the investigators as unlikely to be related to Amigal. A fifth patient with a history of hypertension discontinued study treatment due to increased blood pressure, which was reported by the investigator as possibly related to the study drug.

The results of our Phase II clinical studies to date do not necessarily predict final results for our Phase II clinical studies. The results from additional patients in our ongoing Phase II clinical studies or additional data from these first four patients may cause the results of Study 201 or our other Phase II studies to differ from or be less favorable than the initial data presented above. We cannot assure you that our Phase II clinical studies will ultimately be successful.

Assuming successful completion of our Phase II clinical studies, we expect to initiate a Phase III clinical study of Amigal in Fabry patients in 2007.

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AT2101 for Gaucher Disease

Overview

Our second most advanced clinical product candidate, AT2101, is an orally-administered, small molecule for the treatment of Gaucher disease. In April 2006, we filed an IND for AT2101 in Gaucher disease, and have initiated a Phase I clinical study. Assuming the successful completion of our Phase I clinical studies, we expect to initiate a Phase II clinical study of AT2101 in Gaucher patients in the first half of 2007. In February 2006, the FDA granted orphan drug designation to AT2101.

Causes of Gaucher Disease and Rationale for Use of AT2101

Gaucher disease is a lysosomal storage disorder resulting from a deficiency in a key enzyme, -glucocerebrosidase, or GCase. The deficiency of GCase in Gaucher patients is caused by inherited genetic mutations. Certain of these mutations cause changes in the amino acid sequence of GCase that may result in the production of GCase with reduced stability that does not achieve its correct three-dimensional shape. Although misfolded GCase retains the potential for some level of biological activity, the cell s quality control mechanisms recognize and retain misfolded GCase in the ER until it is ultimately moved to another part of the cell for degradation and elimination. Consequently, little or no GCase moves to the lysosome, where it normally breaks down its substrate, a complex lipid called glucocerebroside. This leads to accumulation of glucocerebroside in cells, which is believed to result in the clinical manifestations of Gaucher disease. In addition, the accumulation of the misfolded GCase enzyme in the ER may lead to cellular stress and inflammatory-like responses, which may contribute to cellular dysfunction and disease. Symptoms can be severe and debilitating, including an enlarged liver and spleen, low levels of red blood cells and platelets, bone pain and fractures. In addition, some patients experience impairment of the lungs and the central nervous system.

AT2101 is designed to act as a pharmacological chaperone for GCase by selectively binding to the enzyme, which increases the stability of the enzyme and helps it fold into its correct three-dimensional shape. This stabilization of GCase allows the cell s quality control mechanisms to recognize the enzyme as properly folded so that trafficking of the enzyme to the lysosome is increased, enabling it to carry out its intended biological function, the metabolism of glucocerebroside. As a result of restoring proper trafficking of GCase from the ER to the lysosome, AT2101 reduces the accumulation of misfolded GCase in the ER, which may alleviate cellular stress and inflammatory-like responses that may be contributing factors in Gaucher disease.

Because AT2101 works by increasing the activity of a patient s naturally produced GCase, those Gaucher disease patients with a missense mutation or other genetic mutation that results in production of GCase that is less stable but with some residual enzyme activity are the ones most likely to respond to treatment with AT2101. We estimate that the substantial majority of patients with Gaucher disease may respond to pharmacological chaperone therapy. Patients with genetic mutations leading to a partially made GCase enzyme or GCase enzyme with an irreversible loss of activity are less likely to respond to treatment with AT2101.

Gaucher Disease Background

Gaucher disease is often described in terms of the following three clinical subtypes:

Type I Chronic Nonneuronopathic Gaucher Disease. Type I Gaucher disease is the most common subtype and symptoms usually first appear in adulthood. Type I Gaucher disease is characterized by the occurrence of an enlarged spleen and liver, anemia, low platelet counts and fractures and bone pain. Patients with Type I Gaucher disease do not experience the neurological features associated with Types II and III Gaucher disease. The clinical severity of Type I Gaucher disease is extremely variable with some patients experiencing the full range of symptoms, while others are asymptomatic throughout most of their lives.

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Type II Acute Neuronopathic Gaucher Disease. Type II Gaucher disease symptoms typically appear in infancy with an average age of onset of about three months. Type II Gaucher disease involves a rapid neurodegeneration with extensive visceral involvement that usually results in death before two years of age, typically due to respiratory complications. The clinical presentation in Type II Gaucher disease is typically more uniform than Type I Gaucher disease.

Type III Subacute Neuronopathic Gaucher Disease. Type III Gaucher disease symptoms typically first appear in infancy or early childhood and involve some neurological symptoms, along with visceral and bone complications. Age of onset and disease severity can vary widely. Disease progression in Type III Gaucher disease is typically slower than in Type II Gaucher disease.

Gaucher Disease Market Opportunity

Gaucher disease is a relatively rare disorder. According to estimates reported by the American Society of Health-System Pharmacists (August 2003) and the National Institute of Neurological Disorders and Stroke (updated as of January 2006) there are approximately 10,000 patients worldwide. By far, Type I Gaucher disease is the most common.

Based on published data from the Human Gene Mutation Database and our experience in the field, we believe that the majority of the known genetic mutations that cause Gaucher disease are missense mutations. Because missense mutations often result in less stable, misfolded GCase, we believe patients with missense mutations are candidates for treatment with AT2101. We also believe that other types of genetic mutations that may result in misfolded GCase could potentially respond to treatment with AT2101. The majority of the Type I Gaucher patient population in the United States, Europe and Israel have the same missense mutation known as N370S. In preclinical tests, AT2101 has shown the ability to increase GCase activity in cells with N370S and other mutations that cause Gaucher disease. In addition, we believe that AT2101 may also benefit some patients with the neurological forms of Gaucher disease (Type II and Type III) because of the ability of the small molecule to cross the blood-brain barrier. Based on this, we believe that a substantial majority of the Gaucher patient population may benefit from treatment with AT2101.

Existing Products for the Treatment of Gaucher Disease and Potential Advantages of AT2101

The current standard of treatment for Gaucher patients is enzyme replacement therapy. There are currently two products approved for the treatment of Gaucher disease. One of the products is Cerezyme, a product approved globally and commercialized by Genzyme Corporation. Cerezyme was approved in the United States in 1994 and in the European Union in 1997 and no longer has orphan drug exclusivity in the United States. In the United States, Cerezyme is indicated for long-term enzyme replacement therapy for pediatric and adult patients with a confirmed diagnosis of Type I Gaucher disease. In the European Union, it is indicated for long-term enzyme replacement therapy for pediatric and adult patients with a confirmed diagnosis of Type I Gaucher disease and for Type III Gaucher disease patients who exhibit clinically significant non-neurological manifestations. The other product approved for treatment of Gaucher disease is Zavesca, a substrate reduction therapy product approved in the United States, the European Union and other countries and commercialized by Actelion, Ltd. Zavesca was approved in the United States in 2003 and has orphan drug exclusivity in the United States until 2010. It was approved in the European Union in 2002 and has orphan drug exclusivity in the European Union until 2012. It is indicated for adults with mild to moderate Type I Gaucher disease for whom enzyme replacement therapy is not an option. The net product sales of Cerezyme and Zavesca for the year 2005 were approximately $932 million and $11 million, respectively, as publicly reported by Genzyme Corporation and Shire PLC, respectively.

For Gaucher disease patients who respond to AT2101, we believe that the use of AT2101 may have advantages relative to the use of Cerezyme. Studies in animals show that AT2101 is distributed throughout the body. Published data demonstrate that treatment with Cerezyme can lead to the reduction of glucocerebroside in multiple tissue types, especially the liver and spleen, and to improve low levels of red blood cells and platelets. Because it is a large protein molecule, Cerezyme is believed to have difficulty

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penetrating some tissues and cell types. In particular, it is widely believed that Cerezyme is unable to cross the blood-brain barrier and thus unlikely to address the neurological symptoms of Type II and Type III Gaucher disease. Studies in animals show that AT2101 crosses the blood-brain barrier, suggesting that it may provide a clinical benefit to patients with Type II and Type III Gaucher disease. In addition, treatment with Cerezyme requires intravenous infusions every other week, presenting an inconvenience to Gaucher disease patients. Oral treatment with AT2101 may be more convenient for patients and may not have the safety risks associated with intravenous infusions. See Potential Advantages of Pharmacological Chaperones in the Treatment of Lysosomal Storage Disorders.

We also believe that AT2101 may have advantages over the use of Zavesca, a substrate reduction therapy. Zavesca is an orally-administered small molecule; however, the underlying mechanism of action is very different than for pharmacological chaperones. Substrate reduction therapies are designed to prevent the production of the substrate that accumulates in disease by inhibiting an enzyme required to make the substrate in cells. This is not the same enzyme that is deficient in Gaucher disease. Importantly, the enzyme that is inhibited is needed to make other important molecules that are used for many types of biological processes. As a result, inhibiting this enzyme may have adverse effects on the cell that are difficult to predict. By contrast, AT2101 is designed to work by binding directly to GCase, increasing its stability and helping it fold into its correct three-dimensional shape. This in turn enables proper trafficking to the lysosome where it can directly decrease substrate accumulation. Several side effects were reported by Actelion, Ltd. in clinical trials of Zavesca, including diarrhea, which was observed in more than 85% of patients who received the drug. Other side effects included hand tremors and numbness and tingling in the hands, arms, legs or feet. AT2101 is designed to work by a very different mechanism than Zavesca, and we do not expect it to have the same side-effect profile.

In February 2006, the FDA granted orphan drug designation to AT2101 for the treatment of Gaucher disease. We believe that the orphan drug designation of Zavesca in the United States and the European Union will not prevent us from obtaining marketing approval of AT2101 in either geography. See Government Regulation.

AT2101 Development Activities

Preclinical Activities

We have conducted several in vitro and in vivo preclinical studies of AT2101. Key findings of our studies include:

- AT2101 increased GCase enzyme activity in cells derived from Gaucher disease patients with different genetic mutations, including cells with a genetic mutation associated with Type II Gaucher disease.

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In normal mice, oral administration of AT2101 resulted in a dose-dependent increase in GCase activity in the liver, spleen, brain and lung. The table below summarizes the results of administration of AT2101 to normal mice for four weeks.

Note: Error bars indicate standard error of the mean.

No mortality or morbidity was observed in the 14-day repeat dose, oral administration studies in rats and monkeys at dose levels up to 1,500 mg/kg of AT2101. This dose was significantly higher than the human equivalent doses being considered for our future clinical studies. The primary treatment-related toxicities occurred in the stomach linings of rats and the skin of monkeys, primarily in the eyelids. All toxicities were found to be reversible or showed a trend toward reversibility. The clinical implications of these preclinical observations are unknown at this time. Chronic toxicity testing of AT2101 is ongoing in six-month rat studies and nine-month monkey studies. We are currently planning reproductive toxicity and carcinogenicity studies of AT2101.

Phase I Clinical Trials

In April 2006, we filed an IND for AT2101 in Gaucher disease, and in June 2006 initiated a Phase I clinical study. We intend to initiate another Phase I clinical study in the second half of 2006. The Phase Ia study is evaluating the effects of AT2101 in subjects who have received a single dose of AT2101. The Phase Ib study will evaluate the effects of AT2101 in subjects who have received AT2101 for up to seven days. Both studies will be conducted in the U.S. and will include healthy male and female adult volunteers.

Phase Ia Clinical Study. The Phase Ia clinical study is a single-center, randomized, double-blind, placebo-controlled, dose-escalation, oral-dose study in up to 40 healthy volunteers. The main objectives of the study are to assess the safety and tolerability of a single oral dose of AT2101 and to evaluate the pharmacokinetics of AT2101 after oral administration. This study also allows selection of a dose level that will be used in subsequent clinical studies.

Phase Ib Clinical Study. The Phase Ib clinical study is planned to begin approximately 6 weeks after the beginning of the Phase Ia clinical study. This study will be a single-center, randomized, double-blind, placebo-controlled, dose-escalation, multiple dose study to evaluate the safety, tolerability and pharmacokinetics of multiple doses of AT2101 in up to 16 healthy volunteers.

Assuming successful completion of our Phase I clinical studies, we expect to initiate a Phase II clinical study of AT2101 in Gaucher patients in the first half of 2007.

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AT2220 for Pompe disease

Overview

Our third most advanced product candidate, AT2220, is an orally-administered small molecule for the treatment of Pompe disease. AT2220 is currently in preclinical development and we expect to file an IND for AT2220 in Pompe disease by the end of 2006.

Causes of Pompe Disease and Rationale for Use of AT2220

Pompe disease is a neuromuscular and lysosomal storage disorder resulting from a deficiency in a key enzyme, -glucosidase, or Gaa. The deficiency of Gaa in Pompe patients is caused by inherited genetic mutations. Certain of these mutations cause changes in the amino acid sequence of Gaa that may result in the production of Gaa with reduced stability and that does not achieve its correct three-dimensional shape. Although misfolded Gaa retains the potential for some level of biological activity, the cell s quality control mechanisms recognize and retain misfolded Gaa in the ER until it is ultimately moved to another part of the cell for degradation and elimination. Consequently, little or no Gaa moves to the lysosome, where it normally breaks down its substrate, glycogen. This leads to accumulation of glycogen in cells, which is believed to result in the clinical manifestations of Pompe disease. In addition, the accumulation and mistrafficking of Gaa may lead to stress on cells and inflammatory-like responses, which may contribute to cellular dysfunction and disease. Symptoms can be severe and debilitating, including progressive muscle weakness throughout the body, particularly the heart, skeletal muscles, liver and nervous system.

AT2220 is designed to act as a pharmacological chaperone for Gaa by selectively binding to the enzyme, which increases its stability, and helps the enzyme fold into its correct three-dimensional shape. This stabilization of Gaa allows the cell s quality control mechanisms to recognize the protein as properly folded so that trafficking of the enzyme to the lysosome is increased, enabling it to carry out its intended biological function, the metabolism of glycogen. As a result of restoring proper trafficking from the ER to the lysosome, AT2220 may reduce the accumulation of misfolded Gaa in the ER, which may alleviate cellular stress and inflammatory-like responses that may be contributing factors in Pompe disease.

Because AT2220 works by increasing the activity of a patient s naturally produced Gaa, those Pompe disease patients with a missense mutation or other genetic mutation that results in production of Gaa that is less stable but with some residual enzyme activity are the ones most likely to respond to treatment with AT2220. We estimate that the majority of patients with Pompe disease may respond to pharmacological chaperone therapy. Patients with genetic mutations leading to a partially made Gaa enzyme or Gaa enzyme with an irreversible loss of activity are less likely to respond to treatment with AT2220.

Pompe Disease Background

Pompe disease, also known as glycogen storage disease type II or acid maltase deficiency, is a relatively rare disorder caused by mutations in Gaa. The mutations in Gaa result in the accumulation of lysosomal glycogen, especially in skeletal, cardiac and smooth muscle tissues. According to reported estimates of the Acid Maltase Deficiency Association, the United Pompe Foundation and the Lysosomal Disease Program at Massachusetts General Hospital, there are 5,000-10,000 patients worldwide with Pompe disease.

The onset of Pompe disease ranges from a rapidly fatal infantile form with severe cardiac involvement to a more slowly progressive, later-onset form primarily affecting skeletal muscle. All forms are characterized by severe muscle weakness that worsens over time. In the rapid onset form, patients are usually diagnosed shortly after birth and often experience enlargement of the heart and severe muscle weakness. In later-onset Pompe disease, symptoms may not appear until late childhood or adulthood and often experience progressive muscle weakness.

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Pompe Disease Market Opportunity

Pompe disease is a relatively rare disorder. Most reported estimates project that there are 5,000 10,000 patients worldwide, the majority of whom have later-onset Pompe disease.

Based on published data from the Human Gene Mutation Database and our experience in the field, we believe that the majority of the known genetic mutations that cause Pompe disease are missense mutations or mutations that result in measurable residual enzyme activity. There are a few mutations reported in Pompe disease that are more common in specific ethnic populations, including a specific one common in Caucasians with adult-onset disease. The majority of Pompe patients have either juvenile or adult-onset disease, and both types of patients generally have measurable levels of residual enzyme activity. Because pharmacological chaperone therapy is most likely to have a benefit in patients with some residual enzyme activity, we believe that a majority of the Pompe patient population may benefit from treatment with AT2220.

Existing Products for the Treatment of Pompe Disease and Potential Advantages of AT2220

The current standard of treatment for Pompe patients is enzyme replacement therapy. There is currently one product approved for the treatment of Pompe disease, Myozyme, approved in the United States and the European Union and commercialized by Genzyme Corporation. Myozyme was approved in the United States in April 2006 and has orphan drug exclusivity in the United States until 2013. It was approved in the European Union in March 2006 and has orphan drug exclusivity in the European Union until 2016. Although Myozyme is approved for use in all Pompe patients, studies have only been performed in infantile-onset disease. Myozyme has not been tested for safety and efficacy in later-onset disease.

For Pompe disease patients who respond to AT2220, we believe that the use of AT2220 may have advantages relative to the use of Myozyme. Available data demonstrate that treatment with Myozyme can improve survival in patients with the infantile form of the disease. Because it is a large protein molecule, Myozyme is believed to have difficulty penetrating many tissues and cell types. As a small molecule that has demonstrated high oral bioavailability and good biodistribution properties in preclinical testing, AT2220 has the potential to reach all cells of the target tissues of Pompe disease patients. Furthermore, treatment with Myozyme requires intravenous infusions every other week, frequently on site at health care facilities, presenting an inconvenience to Pompe disease patients. Oral treatment with AT2220 may be more convenient for patients and may not have the safety risks associated with intravenous infusions. See Potential Advantages of Pharmacological Chaperones in the Treatment of Lysosomal Storage Disorders.

We believe that the orphan drug designation of Myozyme in the United States and in the European Union will not prevent us from obtaining marketing approval of AT2220 in either geography. See Government Regulation.

AT2220 Preclinical Development Activities

We have conducted multiple in vitro and in vivo preclinical studies of AT2220. Key findings of our studies include:

- AT2220 increased Gaa enzyme activity more than five-fold after incubation with AT2220 in cells derived from Pompe disease patients with different genetic mutations.

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Treatment of normal mice with AT2220 resulted in an increase in Gaa activity in the heart, brain, diaphragm and calf. The table below summarizes the results of administration of AT2220 to normal mice for four weeks.

Note: Error bars indicate standard error of the mean.

AT2220 demonstrated a favorable pharmacokinetic profile when tested in rats, including a half life of approximately five hours and good oral bioavailability. In short-term toxicity studies in rats (seven days, dosing up to 2,000 mg/kg) and monkeys (five days, dosing up to 1,000 mg/kg), there were no observed toxicities related to AT2220 except for diarrhea on the first day at the high dose in male monkeys. The clinical implications, if any, of this preclinical observation are unknown at this time.

We plan to file an IND for AT2220 by the end of 2006 and intend to develop AT2220 for all forms of Pompe disease.

Other Programs

We believe that our pharmacological chaperone technology is applicable to the development of drugs for the treatment of a wide range of human genetic and other diseases. We are conducting research on the use of pharmacological chaperones for the treatment of diseases beyond lysosomal storage disorders. For example, we have identified a lead compound that acts as a pharmacological chaperone for a protein implicated in one disease of neurodegeneration. We are also actively engaged in research on a protein target with an established link to obesity. In addition, we are investigating targets for which the ability of pharmacological chaperones to increase normal protein activity may provide therapeutic benefit.

Our Strategy

Our goal is to become a leading biopharmaceutical company focused on the discovery, development and commercialization of pharmacological chaperone therapies for the treatment of a wide-range of human genetic diseases. To achieve this objective, we intend to:

Focus our initial clinical efforts on developing pharmacological chaperones for certain severe genetic diseases called lysosomal storage disorders. Our most advanced programs are for the treatment of Fabry, Gaucher and Pompe disease. We identify the compounds for these diseases using our proprietary approach. We believe our pharmacological chaperone therapy may have advantages over current therapies. We have focused initially on lysosomal storage disorders for a number of reasons:

the therapeutic targets involved in these diseases are amenable to rapid drug discovery and development using our pharmacological chaperone technology;

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the novel mechanisms of action of our product candidates may allow us to better address unmet medical needs in these very debilitating diseases;

the severity of these diseases may permit smaller and more expedited clinical studies; and

the specialized nature of these markets allows for small, targeted sales and marketing efforts that we can pursue independently.

Rapidly advance our lead programs. We are devoting a significant portion of our resources and business efforts to completing the development of our most advanced product candidates. We plan to complete enrollment in our Phase II clinical studies of Amigal for treatment of Fabry disease and advance this product candidate into a Phase III clinical study in 2007. In addition, we have filed an IND to commence clinical studies of AT2101 for the treatment of Gaucher disease and plan to advance this compound into a Phase II study in the first half of 2007. Also, by the end of 2006 we expect to file an IND for AT2220 for the treatment of Pompe disease. To accomplish these goals, we are building an appropriate medical, clinical and regulatory operations infrastructure. In addition, 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.

Leverage our proprietary approach to the discovery and development of additional small molecules. We are focused on the discovery and development of small molecules designed to exert therapeutic effects by acting as pharmacological chaperones. We have steadily advanced these proprietary technologies and built an intellectual property position protecting our discoveries over a number of years. Our technologies span the disciplines of biology, chemistry and pharmacology. We believe many diseases outside of lysosomal storage disorders, such as neurologic diseases and other metabolic diseases, involve misfolded proteins. We also believe that our approach may be broadly applicable. We plan to continue to apply our technologies to discovering and developing treatments for genetic diseases as well as other therapeutic areas.

Build a targeted sales and marketing infrastructure. We plan to establish our own sales and marketing capabilities in the U.S. and potentially in other major markets. We believe that because our current clinical pipeline is focused on relatively rare genetic disorders, we will be able to access the market through a focused, targeted sales force. For example, for Amigal, we believe that the clinical geneticists who are the key specialists in treating Fabry disease are sufficiently concentrated that we will be able to effectively promote the product with our own targeted sales force.

Intellectual Property

Patents and Trade Secrets

Our success depends in part on our ability to maintain proprietary protection surrounding our product candidates, technology and know-how, to operate without infringing the proprietary rights of others, and to prevent others from infringing our proprietary rights. Our policy is to seek to protect our proprietary position by filing U.S. and foreign patent applications related to our proprietary technology, including both new inventions and improvements of existing technology, that are important to the development of our business. Our patent strategy includes obtaining patent protection, where possible, on compositions of matter, methods of manufacture, methods of use, combination therapies, dosing and administration regimens, formulations, therapeutic monitoring, screening methods and assays. We also rely on trade secrets, know-how, continuing technological innovation, in-licensing and partnership opportunities to develop and maintain our proprietary position. Lastly, we monitor third parties for activities that may infringe our proprietary rights, as well as the progression of third party patent applications that may have the potential to create blocks to our products or otherwise interfere with the development of our business. We are aware, for example, of U.S. patents, and corresponding international counterparts, owned by third parties that contain claims related to treating protein misfolding. If any of these 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. There is no assurance that a court would find in our favor or that, if we choose or are required to seek a license, a license to any of these patents would be available to us on acceptable terms or at all.

As of the date of this prospectus, we own or license rights to a total of 10 patents issued in the United States, 5 issued in current member states of the European Patent Convention and 24 pending foreign applications, which are foreign counterparts of many of our U.S. patents. We also own or license rights to 26 pending U.S. applications, 18 of which are provisional. Our patent portfolio includes patents and patent applications with claims relating to methods of increasing deficient enzyme activity to treat genetic diseases. The patent positions for our three leading product candidates are described below and include both patents and patent applications we own or exclusively license:

We have an exclusive license to five U.S. patents and a pending U.S. application that cover use of Amigal, as well as corresponding foreign applications. U.S. patents relating to Amigal expire in 2018, while the foreign counterpart patents, if granted, would expire in 2019. The patents and the pending applications include claims covering methods of increasing the activity of and preventing the degradation of -GAL, and methods for the treatment of Fabry disease using Amigal and other specific competitive inhibitors of -GAL. In addition, we own a pending application directed to specific treatment regimens with Amigal, which may result in a patent that expires in 2027; pending applications directed to synthetic steps related to the commercial process for preparing Amigal, which may result in patents that expire in 2026; and an application for diagnosis of Fabry patients that will respond to treatment with Amigal, which may result in a patent that expires in 2027.

We have an exclusive license to seven U.S. patents and a pending U.S. application, and five foreign patents and one pending foreign application, that cover AT2101 or its use. Two of the U.S. patents relating to AT2101 compositions of matter expire in 2015 and 2016; the five composition of matter foreign patents and one pending foreign application expire in 2015. The other five U.S. patents and one pending application, which claim methods of increasing the activity of and preventing the degradation of GCase, and methods for the treatment of Gaucher disease using AT2101 and other specific competitive inhibitors of GCase, expire in 2018.

We have an exclusive license to three U.S. patents that cover use of AT2220, as well as corresponding foreign applications. The U.S. patents relating to AT2220 expire in 2018, while the foreign counterpart patents, if granted, would expire in 2019. The patents and the pending applications include claims covering methods of increasing the activity of and preventing the degradation of Gaa, and methods for the treatment of Pompe disease using AT2220 and other specific competitive inhibitors of Gaa.

Our patent estate includes patent applications relating to several other potential product candidates. Some of these applications are pending in the United States and foreign patent offices, including two families of patents licensed from Mt. Sinai School of Medicine. Others have to date only been filed as provisional applications in the United States. We expect to file these as non-provisional applications in United States and in other countries at the appropriate time. One application licensed from Mt. Sinai is only pending in the United States. These patent applications, assuming they issue as patents, would expire in the United States between 2023 and 2027.

Individual patents extend for varying periods depending on the effective date of filing of the patent application or the date of patent issuance, and the legal term of the patents in the countries in which they are obtained. Generally, patents issued in the United States are effective for:

the longer of 17 years from the issue date or 20 years from the earliest effective filing date, if the patent application was filed prior to June 8, 1995; and

20 years from the earliest effective filing date, if the patent application was filed on or after June 8, 1995.

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The term of foreign patents varies in accordance with provisions of applicable local law, but typically is 20 years from the earliest effective filing date.

The United States Drug Price Competition and Patent Term Restoration Act of 1984, more commonly known as the Hatch-Waxman Act, provides for an extension of patent protection for drug compounds for a period of up to five years to compensate for time spent in regulatory review. Similar provisions are available in European countries, Japan and other countries. However, we will not know what, if any, extensions are available until a drug is approved. In addition, in the U.S. we may be entitled to an additional six month period of patent exclusivity for pediatric clinical studies.

The patent positions of companies like ours are generally uncertain and involve complex legal, technical, scientific and factual questions. Our ability to maintain and solidify our proprietary position for our technology will depend on our success in promptly filing patent applications on new discoveries, and in obtaining effective claims and enforcing those claims once granted. We seek to protect our proprietary technology and processes, in part, by contracting with our employees, collaborators, scientific advisors and our commercial consultants to ensure that any inventions resulting from the relationship are disclosed promptly, maintained in confidence until a patent application is filed and preferably until publication of the patent application, and assigned to us or subject to a right to obtain a license. We do not know whether any of our own patent applications or those patent applications that are licensed to us will result in the issuance of any patents. Our issued patents and those that may issue in the future, or those licensed to us, may be challenged, narrowed invalidated or circumvented or be found to be invalid or unenforceable, which could limit our ability to stop competitors from marketing related products and reduce the term of patent protection that we may have for our products. Neither we nor our licensors can be certain that we were the first to invent the inventions claimed in our owned or licensed patents or patent applications. In addition, our competitors may independently develop similar technologies or duplicate any technology developed by us and the rights granted under any issued patents may not provide us with any meaningful competitive advantages against these competitors. Furthermore, because of the extensive time required for development, testing and regulatory review of a potential product, it is possible that any related patent may expire prior to or shortly after commencing commercialization, thereby reducing the advantage of the patent to our business and products.

We may rely, in some circumstances, on trade secrets to protect our technology. However, trade secrets are difficult to protect. We seek to protect our trade secret technology and processes, in part, by entering into confidentiality agreements with commercial partners, collaborators, employees, consultants, scientific advisors and other contractors, and by contracting with our employees and some of our commercial consultants to ensure that any trade secrets resulting from such employment or consulting are owned by us. We also seek to preserve the integrity and confidentiality of our data and trade secrets by maintaining physical security of our premises and physical and electronic security of our information technology systems. While we have confidence in these individuals, organizations and systems, agreements or security measures may be breached, and we may not have adequate remedies for any breach. In addition, our trade secrets may otherwise become known or be discovered independently by others. To the extent that our consultants, contractors or collaborators use intellectual property owned by others in their work for us, disputes may arise as to the rights in related or resulting know-how and inventions.

License Agreements

We have acquired rights to develop and commercialize our product candidates through licenses granted by various parties. The following summarizes our material rights and obligations under those licenses:

Mt. Sinai School of Medicine We have acquired exclusive worldwide patent rights to develop and commercialize Amigal, AT2101 and AT2220 and other pharmacological chaperones for the treatment of human diseases caused by misfolded proteins pursuant to a license agreement with Mt. Sinai School of Medicine of New York University. Under this agreement, to date we have paid no upfront or annual license fees and we have no milestone or future payments other than

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royalties on net sales. In connection with this agreement, we issued 1,742,000 shares of our common stock to Mt. Sinai School of Medicine in April 2002. This agreement expires upon expiration of the last of the licensed patent rights, which will be in 2019 if a foreign patent is granted and 2018 otherwise.

University of Maryland, Baltimore County We have acquired exclusive U.S. patent rights to develop and commercialize AT2101 for the treatment of Gaucher disease from the University of Maryland, Baltimore County. Under this agreement, to date we have paid aggregate upfront and annual license fees of $24,500. Upon the satisfaction of certain milestones and assuming successful development of AT2101, we could be required to make up to $175,000 in aggregate payments. We are also required to pay royalties on net sales. This agreement expires upon expiration of the last of the licensed patent rights in 2015.

Novo Nordisk A/S We have acquired exclusive patent rights to develop and commercialize AT2101 for all human indications. Under this agreement, to date we have paid an aggregate of $400,000 in license fees. Upon the satisfaction of certain milestones and assuming successful development of AT2101, we could be required to make up to $7,750,000 in aggregate payments. We are also required to pay royalties on net sales. This license will terminate in 2016.

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 and will owe no milestone payments. We expect to pay royalties to all three licensors with respect to AT2101.

Our rights with respect to these agreements to develop and commercialize Amigal, AT2101 and AT2220 may terminate, in whole or in part, if we fail to meet certain development or commercialization requirements or if we do not meet our obligations to make royalty payments.

Trademarks

In addition to our patents and trade secrets, we have filed applications to register certain trademarks in the U.S. and abroad, including AMICUS, AMICUS THERAPEUTICS (and design) and AMIGAL. At present we do not have any issued registrations for these marks. Our ability to obtain and maintain trademark registrations will in certain instances depend on making use of the mark in commerce on or in connection with our products.

Manufacturing

We rely on contract manufacturers to supply the active pharmaceutical ingredients for Amigal, AT2101 and AT2220. The active pharmaceutical ingredients for all three products are manufactured under current good manufacturing practices, or cGMP, at kilogram scale initiated with commercially available starting materials. We also rely on a separate contract manufacturer to formulate the active pharmaceutical ingredients into hard gelatin capsules that are also made under cGMP. The components in the final formulation for each product are commonly used in other encapsulated products and are well characterized ingredients. We have implemented appropriate controls for assuring the quality of both active pharmaceutical ingredients and the formulated capsules. Product specifications will be established in concurrence with regulatory bodies at the time of product registration.

Competition

Overview

The biotechnology and pharmaceutical industries are characterized by rapidly advancing technologies, intense competition and a strong emphasis on proprietary products. While we believe that our technologies, knowledge, experience and scientific resources provide us with competitive advantages, we face potential

competition from many different sources, including commercial pharmaceutical and biotechnology enterprises, academic institutions, government agencies and private and public research institutions. Any product candidates that we successfully develop and commercialize will compete with existing therapies and new therapies that may become available in the future.

Many of our competitors may have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical studies, obtaining regulatory approvals and marketing approved products than we do. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel, as well as in acquiring technologies complementary to, or necessary for, our programs. Smaller or early stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies.

Our commercial opportunities could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer side effects, are more convenient or are less expensive than products that we may develop. In addition, our ability to compete may be affected because in some cases insurers or other third party payors seek to encourage the use of generic products. This may have the effect of making branded products less attractive to buyers.

Major Competitors

Our major competitors include pharmaceutical and biotechnology companies in the United States and abroad that have approved therapies or therapies in development for lysosomal storage disorders within our core programs. Other competitors are pharmaceutical and biotechnology companies that have approved therapies or therapies in development for genetic diseases for which pharmacological chaperone technology may be applicable. Additionally, we are aware of several early-stage, niche pharmaceutical and biotechnology companies whose core business revolves around protein misfolding; however, we are not aware that any of these companies is currently working to develop products that would directly compete with ours. The key competitive factors affecting the success of our product candidates are likely to be their efficacy, safety, convenience and price.

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Any product candidates that we successfully develop and commercialize will compete with existing therapies and new therapies that may become available in the future. The following table lists our principal competitors and publicly available information on the status of their product offerings:

Competitor	Indication	Product	Class of Product	Status	2005 Sales (in millions)
Genzyme Corporation	Fabry disease	Fabrazyme	Enzyme Replacement Therapy	Marketed	$ 305
	Gaucher disease	Cerezyme	Enzyme Replacement Therapy	Marketed	$ 932
	Pompe disease	Myozyme	Enzyme Replacement Therapy	Marketed	N/A
	Gaucher disease	Genz-112638	Substrate Reduction Therapy	Phase I/II	N/A
Shire PLC	Fabry disease	Replagal	Enzyme Replacement Therapy	Marketed	$95
	Gaucher disease	GA-GCB	Enzyme Replacement Therapy	Phase I/II	N/A
Actelion, Ltd.	Gaucher disease	Zavesca	Substrate Reduction Therapy	Marketed	$11

We are aware of other companies that are conducting preclinical development activities for enzyme replacement therapies to treat Gaucher disease and Pompe disease.

Government Regulation

Government authorities in the United States, at the federal, state and local level, and in other countries, extensively regulate, among other things, the research, development, testing, approval, manufacturing, labeling, post-approval monitoring and reporting, packaging promotion, storage advertising, distribution, sampling, marketing, import and export of pharmaceutical products such as those we are developing. The process of obtaining regulatory approvals and the subsequent substantial compliance with appropriate federal, state, local and foreign statues and regulations require the expenditure of substantial time and human and financial resources.

United States Government Regulation

In the United States, the information that must be submitted to the FDA in order to obtain approval to market a new drug varies depending upon whether the drug is a new product whose safety and efficacy have not previously been demonstrated in humans or a drug whose active ingredients and certain other properties are the same as those of a previously approved drug. A product whose safety and efficacy have not previously been demonstrated in humans

has to comply with the New Drug Application, or NDA, approval process.

The NDA Approval Process

In the United States, the FDA regulates drugs under the Federal Food, Drug, and Cosmetic Act and implementing regulations. Failure to comply with the applicable FDA requirements at any time during the product development process, approval process or after approval may result in administrative or judicial

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sanctions. These sanctions could include the FDA s imposition of a clinical hold on studies, refusal to approve pending applications, withdrawal of an approval, warning letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, civil penalties or criminal prosecution. Any agency or judicial enforcement action could have a material adverse effect on us.

The steps required before a drug may be marketed in the United States include:

- completion of preclinical laboratory tests, animal studies and formulation studies under the FDA s good laboratory practices regulations;
- development of adequate manufacturing and quality control procedures to ensure that clinical supplies meet FDA requirements for identity, strength, quality and purity;
- submission to the FDA of an IND for human clinical testing, which must become effective before human clinical studies may begin and which must include independent Institutional Review Board, or IRB, approval at each clinical site before the studies may be initiated;
- performance of adequate and well-controlled clinical studies in accordance with Good Clinical Practices, known as GCP, to establish the safety and efficacy of the product for each indication;
- submission to the FDA of an NDA;
- satisfactory completion of an FDA Advisory Committee review, if applicable;
- satisfactory completion of an FDA inspection of the manufacturing facility or facilities at which the product is produced to assess compliance with current good manufacturing practices, or cGMP, to assure that the facilities, methods and controls are adequate to preserve the product s identity, strength, quality and purity; and
- FDA review and approval of the NDA.

Preclinical tests include laboratory evaluations of product chemistry, toxicity and formulation, as well as animal studies. An IND sponsor must submit the results of the preclinical tests, together with manufacturing information and analytical data, to the FDA as part of the IND. Some preclinical testing may continue after the IND is submitted. The IND must become effective before human clinical studies may begin. An IND will automatically become effective 30 days after receipt by the FDA, unless before that time the FDA raises concerns or questions about issues such as the conduct of the studies as outlined in the IND. In that case, the IND sponsor and the FDA must resolve any outstanding FDA concerns or questions before clinical studies can proceed. In other words, submission of an IND may not result in the FDA allowing clinical studies to commence.

Clinical studies involve the administration of the investigational product to human subjects under the supervision of qualified investigators. Clinical studies are conducted under protocols detailing, among other things, the objectives of the study, the parameters to be used in monitoring safety and the effectiveness criteria to be evaluated. A protocol for each clinical study and any subsequent protocol amendments must be submitted to the FDA as part of the IND. In addition, an IRB at each site at which the study is conducted must approve the protocol and any amendments. All research subjects must provide their informed consent in writing.

Clinical studies typically are conducted in three sequential phases, but the phases may overlap or be combined. Phase I studies usually involve the initial introduction of the investigational drug into healthy volunteers to evaluate the product s safety, dosage tolerance and pharmacokinetics and, if possible, to gain an early indication of its effectiveness.

Phase II studies usually involve controlled studies in a limited patient population to:

- evaluate dosage tolerance and appropriate dosage;

identify possible adverse effects and safety risks; and

provide a preliminary evaluation of the efficacy of the drug for specific indications.

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Phase II studies are sometimes denoted by companies as Phase IIa or Phase IIb studies. Phase IIa studies typically represent the first human clinical study of a drug candidate in a smaller patient population and are designed to provide earlier information on drug safety and efficacy. Phase IIb studies typically involve larger numbers of patients or longer durations of therapy and may involve comparison with placebo, standard treatments or other active comparators.

Phase III studies usually further evaluate clinical efficacy and test further for safety in an expanded patient population. Phase III studies usually involve comparison with placebo, standard treatments or other active comparators. These studies are intended to establish the overall risk-benefit profile of the product and provide an adequate basis for physician labeling. Phase III studies are usually larger, more time consuming, more complex and more costly than Phase I and Phase II studies. As noted above, Amigal is currently in Phase II studies for the treatment of Fabry disease and we have filed an IND to conduct two Phase I studies for the treatment of Gaucher disease with AT2101.

Phase I, Phase II and Phase III testing may not be completed successfully within any specified period, if at all. Furthermore, the FDA or we may suspend or terminate clinical studies at any time on various grounds, including a finding that the subjects or patients are being exposed to an unacceptable health risk. Similarly, an IRB can suspend or terminate approval of research if the research is not being conducted in accordance with the IRB s requirements or if the research has been associated with unexpected serious harm to patients.

Assuming successful completion of the required clinical testing, the results of the preclinical studies and of the clinical studies, together with other detailed information, including information on the chemistry, manufacture and composition of the product, are submitted to the FDA in the form of an NDA requesting approval to market the product for one or more indications. In most cases, the NDA must be accompanied by a substantial user fee. The FDA will initially review the NDA for completeness before it accepts the NDA for filing. After the NDA submission is accepted for filing, the FDA reviews the NDA to determine, among other things, whether a product is safe and effective for its intended use and whether the product is being manufactured in accordance with cGMP to assure and preserve the product s identity, strength, quality and purity.

Under the Pediatric Research Equity Act of 2003, or PREA, NDAs or supplements to NDAs must contain data to assess the safety and effectiveness of the drug for the claimed indications in all relevant pediatric subpopulations and to support dosing and administration for each pediatric subpopulation for which the drug is safe and effective. The FDA may grant deferrals for submission of data or full or partial waivers. Unless otherwise required by regulation, PREA does not apply to any drug for an indication for which orphan designation has been granted.

Before approving an NDA, the FDA will typically inspect one or more clinical sites to assure compliance with GCP. Additionally, the FDA will inspect the facility or the facilities at which the product is manufactured. The FDA will not approve the product unless cGMP compliance is satisfactory. If the FDA determines the application, manufacturing process or manufacturing facilities are not acceptable, it will outline the deficiencies in the submission and often will request additional testing or information. Notwithstanding the submission of any requested additional information, the FDA ultimately may decide that the application does not satisfy the regulatory criteria for approval. The testing and approval process requires substantial time, effort and financial resources, and may take several years to complete. Data obtained from clinical activities are not always conclusive and may be susceptible to varying interpretations, which could delay, limit or prevent regulatory approval. The FDA may not grant approval on a timely basis, or at all. We may encounter difficulties or unanticipated costs in our efforts to secure necessary governmental approvals, which could delay or preclude us from marketing our products. The FDA may limit the indications for use or place other conditions on any approvals that could restrict the commercial application of the products. After approval, some types of changes to the approved product, such as adding new indications, manufacturing changes and additional labeling claims, are subject to further testing requirements and FDA review and approval.

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Post-Approval Requirements

After regulatory approval of a product is obtained, we are required to comply with a number of post-approval requirements. For example, as a condition of approval of an NDA, the FDA may require post-marketing testing and surveillance to monitor the product s safety or efficacy. In addition, holders of an approved NDA are required to report certain adverse reactions and production problems to the FDA, to provide updated safety and efficacy information and to comply with requirements concerning advertising and promotional labeling for their products. Also, quality control and manufacturing procedures must continue to conform to cGMP after approval. The FDA periodically inspects manufacturing facilities to assess compliance with cGMP, which imposes certain procedural, substantive and recordkeeping requirements. Accordingly, manufacturers must continue to expend time, money and effort in the area of production and quality control to maintain compliance with cGMP and other aspects of regulatory compliance.

New products that are being developed for the treatment of serious or life-threatening diseases where the product would provide therapeutic advantage over the existing treatment may be considered for accelerated approval by the FDA. In these cases, approval can be based on surrogate markers that may predict, but do not establish, clinical benefit. Sponsors of products that receive accelerated approval must carry out clinical trials post-approval to verify the desired clinical benefit. If the post-approval studies fail to demonstrate clinical benefit, the FDA can withdraw the approval of the drug through expedited procedures. Promotional materials for products that receive accelerated approval must be submitted to the FDA for review prior to use.

We rely, and expect to continue to rely, on third parties for the production of clinical and commercial quantities of our product candidates. Future FDA inspections may identify compliance issues at our facilities or at the facilities of our contract manufacturers that may disrupt production or distribution, or require substantial resources to correct. In addition, discovery of problems with a product or the failure to comply with applicable requirements may result in restrictions on a product or a manufacturer or holder of an approved NDA, including withdrawal or recall of the product from the market or other voluntary, FDA-initiated or judicial action that could delay or prohibit further marketing. Newly discovered or developed safety or effectiveness data may require changes to a product s approved labeling, including the addition of new warnings and contraindications. Improper promotional activities can result in significant financial penalties. Also, new government requirements, including those resulting from new legislation, may be established that could delay or prevent regulatory approval of our products under development.

Orphan Drug Designation

We have received orphan drug designation in the United States from the FDA for Amigal for the treatment of Fabry disease and for AT2101 for the treatment of Gaucher disease, and we anticipate filing for orphan drug designation from the FDA for AT2220 for the treatment of Pompe disease. The FDA may grant orphan drug designation to drugs intended to treat a rare disease or condition that affects fewer than 200,000 individuals in the United States, or more than 200,000 individuals in the United States but for which there is no reasonable expectation that the cost of developing and making available in the United States a drug for this type of disease or condition will be recovered from sales in the United States for that drug. Orphan drug designation must be requested before submitting an application for marketing approval. Orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process.

Orphan drug designation can provide opportunities for grant funding towards clinical study costs, tax advantages, and eliminates the need to submit an FDA user-fee with the NDA. In addition, if a product which has an orphan drug designation subsequently receives FDA marketing approval for the indication for which it has such designation, the product is entitled to orphan drug exclusivity, which means the FDA may not approve any other application to market the same drug for the same indication for a period of seven years, except in limited circumstances, such as a showing of clinical superiority to the product with orphan exclusivity. Competitors may receive marketing approval of different drugs or biologics for the indications for which the orphan product has exclusivity.

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As described in the section of this prospectus entitled Our Lead Product Candidates Amigal for Fabry Disease Existing Products for the Treatment of Fabry Disease and Potential Advantages of Amigal and Our Lead Product Candidates AT2101 for Gaucher Disease Existing Products for the Treatment of Gaucher Disease and Potential Advantages of AT2101, we believe that despite the existence of orphan drug exclusivity for Fabrazyme for the treatment of Fabry disease and Zavesca for the treatment of Gaucher disease these exclusivities will not prevent us from obtaining marketing approval of Amigal and AT2101 in the United States for the treatment of Fabry disease and Gaucher disease, respectively, because, among other things, Amigal and AT2101 are different molecules than Fabrazyme and Zavesca, respectively.

Regulation Outside the United States

In addition to regulations in the United States, we will be subject to a variety of regulations in other jurisdictions governing clinical studies and commercial sales and distribution of our products. Whether or not we obtain FDA approval for a product, we must obtain approval of a product by the comparable regulatory authorities of countries outside the United States before we can commence clinical studies or marketing of the product in those countries. The approval process varies from country to country, and the time may be longer or shorter than that required for FDA approval.

To obtain regulatory approval of a drug under European Union regulatory systems, we may submit marketing authorizations either under a centralized or decentralized procedure. The centralized procedure, which is compulsory for medicines produced by certain biotechnological processes and optional for those which are highly innovative, provides for the grant of a single marketing authorization that is valid for all European Union member states. The decentralized procedure provides for approval by one or more other, or concerned, member states of an assessment of an application performed by one member state, known as the reference member state. Under this procedure, an applicant submits an application, or dossier, and related materials including a draft summary of product characteristics, and draft labeling and package leaflet, to the reference member state and concerned member states. The reference member state prepares a draft assessment and drafts of the related materials within 120 days after receipt of a valid application. Within 90 days of receiving the reference member state s assessment report, each concerned member state must decide whether to approve the assessment report and related materials. If a member state cannot approve the assessment report and related materials on the grounds of potential serious risk to the public health, the disputed points may eventually be referred to the European Commission, whose decision is binding on all member states.

We have obtained an orphan medicinal product designation in the European Union from the EMEA for Amigal for the treatment of Fabry disease and we anticipate filing for orphan medicinal product designation from the EMEA for AT2101 for the treatment of Gaucher disease and for AT2220 for the treatment of Pompe disease. The EMEA grants orphan drug designation to promote the development of products that may offer therapeutic benefits for life-threatening or chronically debilitating conditions affecting not more than five in 10,000 people in the European Union. In addition, orphan drug designation can be granted if the drug is intended for a life threatening, seriously debilitating or serious and chronic condition in the European Union and that without incentives it is unlikely that sales of the drug in the European Union would be sufficient to justify developing the drug. Orphan drug designation is only available if there is no other satisfactory method approved in the European Union of diagnosing, preventing or treating the condition, or if such a method exists, the proposed orphan drug will be of significant benefit to patients.

Orphan drug designation provides opportunities for free protocol assistance and fee reductions for access to the centralized regulatory procedures before and during the first year after marketing approval, which reductions are not limited to the first year after marketing approval for small and medium enterprises. In addition, if a product which has an orphan drug designation subsequently receives EMEA marketing approval for the indication for which it has such designation, the product is entitled to orphan drug exclusivity, which means the EMEA may not approve any other application to market the same drug for the same indication for a period of ten years. The exclusivity period may be reduced to six years if the

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designation criteria are no longer met, including where it is shown that the product is sufficiently profitable not to justify maintenance of market exclusivity. Competitors may receive marketing approval of different drugs or biologics for the indications for which the orphan product has exclusivity. In order to do so, however, they must demonstrate that the new drugs or biologics provide a significant benefit over the existing orphan product. This demonstration of significant benefit may be done at the time of initial approval or in post-approval studies, depending on the type of marketing authorization granted.

As described in the section of this prospectus entitled Our Lead Product Candidates Amigal for Fabry Disease Existing Products for the Treatment of Fabry Disease and Potential Advantages of Amigal, we believe that the orphan designation of Fabrazyme and Replagal in the European Union will not prevent us from obtaining marketing approval of Amigal in the European Union for the treatment of Fabry disease because Amigal will provide significant benefits over Fabrazyme and Replagal. Similarly, we believe the orphan drug designation of Zavesca in the European Union will not prevent us from obtaining marketing approval of AT2101 in the European Union for the treatment of Gaucher disease because AT2101 will provide significant benefits over Zavesca.

Pharmaceutical Pricing and Reimbursement

In the United States and markets in other countries, sales of any products for which we receive regulatory approval for commercial sale will depend in part on the availability of reimbursement from third party payors. Third party payors include government health administrative authorities, managed care providers, private health insurers and other organizations. These third party payors are increasingly challenging the price and examining the cost-effectiveness of medical products and services. In addition, significant uncertainty exists as to the reimbursement status of newly approved healthcare product candidates. We may need to conduct expensive pharmacoeconomic studies in order to demonstrate the cost-effectiveness of our products. Our product candidates may not be considered cost-effective. Adequate third party reimbursement may not be available to enable us to maintain price levels sufficient to realize an appropriate return on our investment in product development.

In 2003, the United States government enacted legislation providing a partial prescription drug benefit for Medicare recipients, that began in 2006. Government payment for some of the costs of prescription drugs may increase demand for any products for which we receive marketing approval. However, to obtain payments under this program, we would be required to sell products to Medicare recipients through managed care organizations and other health care delivery systems operating pursuant to this legislation. These organizations would negotiate prices for our products, which are likely to be lower than we might otherwise obtain. Federal, state and local governments in the United States continue to consider legislation to limit the growth of healthcare costs, including the cost of prescription drugs. Future legislation could limit payments for pharmaceuticals such as the drug candidates that we are developing.

The marketability of any products for which we receive regulatory approval for commercial sale may suffer if the government and third party payors fail to provide adequate coverage and reimbursement. In addition, an increasing emphasis on managed care in the United States has increased and will continue to increase the pressure on pharmaceutical pricing.

Scientific Advisory Board

Our scientific advisory board consists of scientific and clinical advisors who are leading experts in the fields of lysosomal enzymes, protein folding, protein trafficking and structure, sugar and carbohydrate chemistry, genetics, post-transcriptional regulation, preclinical studies, drug manufacturing and clinical studies. Our scientific advisory board consults with us regularly on matters relating to:

- our research and development programs;
- the design and implementation of our clinical studies;
- market opportunities from a clinical perspective;

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new technologies relevant to our research and development programs; and

scientific and technical issues relevant to our business.

Our current scientific advisory board members are:

Name	Professional Affiliation
Michel Bouvier, M.D., Ph.D.	Professor and Director, Academic Research Group on Therapeutics; Canada Research Chair in Signal Transduction and Molecular Pharmacology; Department of Biochemistry, Faculty of Medicine, University of Montreal
Gregory Fricchione, M.D.	Associate Professor of Psychiatry at the Harvard Medical School; Associate Chief of Psychiatry and Director of the Division of Psychiatry and Medicine and the Division of International Psychiatry at Massachusetts General Hospital in Boston
Bruce Ganem, Ph.D.	Franz and Elisabeth Roessler Professor of Chemistry; J. Thomas Clark Professor of Entrepreneurship and Personal Enterprise, The Johnson School, Cornell University
Arthur L. Horwich, M.D.	Professor of Genetics and Pediatrics, Yale University; Investigator, Howard Hughes Medical Institute
Stuart A. Kornfeld, M.D.	Professor, Department of Medicine, Hematology Division; Professor, Department of Biochemistry & Molecular Biophysics, Washington University Medical School
Gregory A. Petsko, D.Phil., Ph.D.	Gyula and Katica Tauber Professor, Department of Biochemistry and Department of Chemistry and Director, Rosenstiel Basic Medical Sciences Research Center, Brandeis University; Adjunct Professor, Department of Neurology and Center for Neurologic Diseases, Harvard Medical School

Employees

As of April 30, 2006, we had 52 full-time employees, 35 of whom were primarily engaged in research and development activities and 17 of whom provide administrative services. A total of 24 employees have an M.D. or Ph.D. degree. None of our employees are represented by a labor union. We have not experienced any work stoppages and consider our employee relations to be good.

Property

Our headquarters are located in Cranbury, New Jersey, consisting of approximately 32,000 square feet of subleased office and laboratory space. In May 2005, we entered into a seven-year non-cancelable operating lease agreement for this office and laboratory space. This operating lease will expire by its terms on February 28, 2012.

Legal Proceedings

We are not currently a party to any material legal proceedings.

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MANAGEMENT

Our executive officers and directors and their respective ages and positions as of June 19, 2006 are as follows:

Name	Age	Position
John F. Crowley	39	President and Chief Executive Officer and Director
Matthew R. Patterson	35	Chief Business Officer
Pedro Huertas, M.D., Ph.D.	52	Chief Strategic Officer
David J. Lockhart, Ph.D.	44	Chief Scientific Officer
David Palling, Ph.D.	52	Senior Vice President, Drug Development
Karin Ludwig, M.D.	44	Senior Vice President, Clinical Research
Mark Simon	44	Senior Vice President, Business Development
Gregory P. Licholai, M.D.	41	Vice President, Medical Affairs
S. Nicole Schaeffer	38	Vice President, Human Resources and Leadership Development
Douglas A. Branch	49	Vice President, General Counsel and Secretary
Donald J. Hayden(3)	50	Executive Chairman
Alexander E. Barkas, Ph.D.(3)	58	Director
Michael G. Raab(2)(3)	41	Director
James N. Topper, M.D., Ph.D.(1)	44	Director
Stephen Bloch, M.D.(2)	44	Director
Gregory M. Weinhoff, M.D.(1)	35	Director
P. Sherrill Neff(1)	54	Director

(1) Member of Compensation Committee.
(2) Member of Audit Committee.
(3) Member of Nominating/Corporate Governance Committee.

John F. Crowley has served as President and Chief Executive Officer of Amicus since January 2005, and has also served as a Director of Amicus since August 2004. He was President and Chief Executive Officer of Orexigen Therapeutics, Inc. from 2003 to December 2004. Mr. Crowley was President and Chief Executive Officer of Novazyme Pharmaceuticals, Inc., from March 2000 until that company was acquired by Genzyme Corporation in September 2001; thereafter he served as Senior Vice President of Genzyme Therapeutics until December 2002. Mr. Crowley received a B.S. degree in Foreign Service from Georgetown University s School of Foreign Service, a J.D. from the University of Notre Dame Law School, and an M.B.A. from Harvard Business School.

Matthew R. Patterson has served as Chief Business Officer of Amicus since December 2004. Prior to joining Amicus, Mr. Patterson was with BioMarin Pharmaceutical Inc. from 1998 to December 2004, most recently serving as Vice President, Commercial Planning and Government Affairs. Prior to BioMarin, Mr. Patterson worked at Genzyme Corporation from 1993 to 1998 in Regulatory Affairs and Manufacturing. Mr. Patterson received a B.A. in Biochemistry from Bowdoin College.

Pedro Huertas, M.D., Ph.D., is our Chief Strategic Officer and had previously served as our Chief Development Officer since July 2005. Prior to joining Amicus, Dr. Huertas was Chief Medical Officer (acting) at StemCells, Inc. from October 2003 to March 2005. Dr. Huertas was Chief Medical Officer of Novazyme Pharmaceuticals, Inc. from 2000 until that company was acquired by Genzyme Corporation in September 2001; prior to joining Novazyme he served as Director of Strategic Development and Medical Director of Genzyme until December 2000. Dr. Huertas received his undergraduate degree from Stanford University, his Ph.D. from Harvard University and his M.D. from the Division of Health Sciences and Technology, Harvard Medical School Massachusetts Institute of Technology, and his M.B.A. from the Sloan School at the Massachusetts Institute of Technology.

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David J. Lockhart, Ph.D., has served as our Chief Scientific Officer since January 2006. Prior to joining Amicus, Dr. Lockhart served as President, Chief Scientific Officer and co-founder of Ambit Biosciences, a biotechnology company specializing in small molecule kinase inhibitors, from March 2001 to July 2005. Dr. Lockhart served as a consultant to Ambit Biosciences from August 2000 to March 2001, and as a visiting scholar at the Salk Institute for Biological Studies from October 2000 to March 2001. Prior to that, Dr. Lockhart served in various positions, including Vice President of Genomics Research at Affymetrix, and was the Director of Genomics at the Genomics Institute of the Novartis Research Foundation from February 1999 to July 2000. He received his Ph.D. from Stanford University and was a post-doctoral fellow at the Whitehead Institute for Biomedical Research at the Massachusetts Institute of Technology.

David Palling, Ph.D., has served as Senior Vice President, Drug Development, since August, 2002. From September 1998 until August, 2002, Dr. Palling was with Johnson & Johnson, most recently serving as Vice President of Worldwide Assay Research and Development at Ortho Clinical Diagnostics, a subsidiary of Johnson & Johnson. Dr. Palling received B.Sc. and Ph.D. degrees in Chemistry from the University of London, King s College, and conducted post-doctoral research in Biochemistry at Brandeis University.

Karin Ludwig, M.D., has served as our Senior Vice President, Clinical Research, since February 2006. From 1993 until February 2006, Dr. Ludwig served in a variety of clinical research positions at Pharmacia Corporation and subsequently Pfizer, Inc., after its acquisition of Pharmacia in 2003, most recently Group Leader/ Senior Director, U.S. Medical, Endocrinology and Ophthalmology. She received her M.D. from the University Freiburg Medical School.

Mark Simon has served as Senior Vice President, Business Development since June 2006. Since October 2005 he has served as an industry consultant to multiple biopharmaceutical companies. From 2002 to 2005 he was Managing Director and Head of Life Sciences Investment Banking for Citigroup Global Markets. From 1989 to 2002 he served as a Senior Research Analyst and later as Managing Director, Investment Banking for Robertson Stephens. He received his B.A. from Columbia College and his M.B.A. from Harvard Business School.

Gregory P. Licholai, M.D., has served as Vice President, Medical Affairs since December 2004. From November 2002 to December 2004, Dr. Licholai was with Domain Associates, a venture capital firm. From September 2000 to November 2002, he was director of Ventures and Business Associates for Medtronic Neurological, a division of Medtronic, Inc. Dr. Licholai received his B.A. from Boston College and completed Pre-Medical studies at Columbia University, his M.D. from Yale Medical School and his M.B.A. from Harvard Business School.

S. Nicole Schaeffer has served as Vice President, Human Resources and Leadership Development since March 2005. From 2001 to 2004, she served as Senior Director, Human Resources, for three portfolio companies of Flagship Ventures, a venture capital firm, and in that capacity she managed human resources for three life sciences companies. Ms. Schaeffer received her B.A. from the University of Rochester and her M.B.A. from Boston University.

Douglas A. Branch has served as General Counsel and Secretary since December 2005, and as Vice President since May 2006. He is also President of Biotech Law Associates, P.C., a law firm, where he has practiced since April 2004. From 1996 to April 2004, he was a Director and Shareholder of Phillips McFall McCaffrey McVay & Murrah, P.C., an Oklahoma City law firm. He holds B.B.A. (Finance) and J.D. degrees from the University of Oklahoma.

Donald J. Hayden, Jr. has served as our Chairman since March 2006. From 1991 to 2005 he held several executive positions with Bristol-Myers Squibb Company, most recently serving as Executive Vice President and President, Americas, Worldwide Medicines Group. Mr. Hayden holds a B.A. from Harvard University and an M.B.A. from Indiana University.

Alexander E. Barkas, Ph.D., has served as a member of our board of directors since 2004. Since June 1997, Dr. Barkas has served as a Managing Member of Prospect Management Co. II, LLC, a venture

capital management company. Dr. Barkas also serves as the chairman of the board of directors of Tercica, Inc. and Geron Corporation. He holds a B.A. from Brandeis University and a Ph.D. from New York University.

Michael G. Raab has served as a member of our board of directors since 2004. Mr. Raab has served as a partner of New Enterprise Associates since June 2002. From 1999 to 2002, Mr. Raab was a Senior Vice President, Therapeutics and General Manager, Renagel® at Genzyme Corporation. Mr. Raab holds a B.A. from DePauw University.

James N. Topper, M.D., Ph.D., has served as a member of our board of directors since 2004. Dr. Topper has been a partner with Frazier Healthcare Ventures since August 2003, holding the position of General Partner since 2004. Prior to joining Frazier Healthcare, he served as Head of the Cardiovascular Research and Development Division of Millennium Pharmaceuticals and ran Millennium San Francisco (formerly COR Therapeutics) from 2002 until 2003. Prior to the merger of COR and Millennium in 2002, Dr. Topper served as the Vice President of Biology at COR from August 1999 to February 2002. He holds an appointment as a Clinical Assistant Professor of Medicine at Stanford University and as a Cardiology Consultant to the Palo Alto Veterans Administration Hospital. Dr. Topper currently serves on the board of La Jolla Pharmaceuticals Company. Dr. Topper holds an M.D. and a Ph.D. in Biophysics from Stanford University School of Medicine.

Stephen Bloch, M.D., has served as a member of our board of directors since 2004. He has served as a Principal at Canaan Partners since June 2002. Prior to joining Canaan, Dr. Bloch founded and served as the Chief Executive Officer of Radiology Management Sciences, a risk manager of diagnostic imaging services for health plans and provider networks, from 1995 to 2002. Dr. Bloch received his M.D. from the University of Rochester. He also received a M.A. in history of science from Harvard University and an A.B. degree in history from Dartmouth College.

Gregory M. Weinhoff, M.D. has served as a member of our board of directors since our inception. Since 2001, Dr. Weinhoff has served as a Member of Collinson Howe & Lennox II, L.L.C., the general partner of CHL Medical Partners II, L.P. Dr. Weinhoff served as our founding Chief Executive Officer from inception until October 2002. From 2000 to 2001, Dr. Weinhoff was a Senior Associate at Whitney & Co. Dr. Weinhoff holds an A.B. degree from Harvard College, an M.D. degree from Harvard Medical School and an M.B.A. degree from Harvard Business School.

P. Sherrill Neff has served as a member of our board of directors since 2005. Mr. Neff is a founding partner and has served as the managing partner of Quaker BioVentures, L.P. since 2002. Prior to forming Quaker BioVentures, L.P., he was President, Chief Operating Officer, and a director of Neose Technologies, Inc. from 1994 to 2002. Mr. Neff currently sits on the board of Resource Capital Corporation. Mr. Neff is a graduate of Wesleyan University and the University of Michigan Law School.

Board Compensation and Election of Directors

Our board of directors is currently authorized to have, and we currently have, eight members. In accordance with the terms of our certificate of incorporation and bylaws that will become effective upon the closing of this offering, our board of directors will be divided into three classes, class I, class II and class III, with each class serving staggered three-year terms. Upon the closing of this offering, the members of the classes will be divided as follows:

- the class I directors will be , and , and their term will expire at the annual meeting of stockholders to be held in 2007;
- the class II directors will be , , and , and their term will expire at the annual meeting of stockholders to be held in 2008; and
- the class III directors will be , and and their term will expire at the annual meeting of stockholders to be held in 2009.

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Our certificate of incorporation to be effective upon the closing of this offering provides that our directors may be removed only for cause and by the affirmative vote of the holders of a majority of our voting stock. Upon the expiration of the term of a class of directors, directors in that class will be eligible to be elected for a new three-year term at the annual meeting of stockholders in the year in which their term expires.

All of our current directors, except John F. Crowley and Donald J. Hayden, are independent directors, as defined by the applicable rules of The Nasdaq National Market. We refer to these directors as our independent directors. Upon the closing of this offering each of these independent directors will serve on one or more of our audit committee, compensation committee and nominating and corporate governance committees. There are no family relationships among any of our directors or executive officers.

Board Committees

Our board currently has established an audit committee, a compensation committee and a nominating/corporate governance committee. The composition of each committee is effective currently but we expect will be modified prior to the closing of this offering.

Audit Committee

The members of our audit committee are Dr. Bloch and Mr. Raab. Mr. Raab chairs the audit committee. Our audit committee assists our board of directors in its oversight of the integrity of our financial statements, our independent registered public accounting firm s qualifications and independence and the performance of our independent registered public accounting firm.

Upon closing of this offering, our audit committees responsibilities will include:

- appointing, approving the compensation of, and assessing the independence of our independent registered public accounting firm;
- overseeing the work of our independent registered public accounting firm, including through the receipt and consideration of certain reports from our independent registered public accounting firm;
- reviewing and discussing with management and the independent registered public accounting firm our annual and quarterly financial statements and related disclosures;
- monitoring our internal control over financial reporting, disclosure controls and procedures and code of business conduct and ethics;
- establishing policies regarding hiring employees from our independent registered public accounting firm and procedures for the receipt and retention of accounting related complaints and concerns;
- meeting independently with our independent registered public accounting firm and management; and
- preparing the audit committee report required by Securities and Exchange Commission rules.

All audit and non-audit services to be provided to us by our independent registered public accounting firm must be approved in advance by our audit committee.

We are seeking to retain a person who will qualify as our audit committee financial expert. We believe that the composition of our audit committee will meet the requirements for independence under the current Nasdaq National Market and Securities and Exchange Commission rules and regulations.

Compensation Committee

Mr. Neff and Drs. Topper and Weinhoff are the members of our compensation committee. Mr. Neff is the chair of the committee. Our compensation committee assists our board of directors in the discharge of its responsibilities relating to the compensation of our executive officers.

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Our compensation committee s responsibilities include:

- reviewing and approving, or making recommendations to our board of directors with respect to, the compensation of our chief executive officer and our other executive officers;
- overseeing the evaluation of performance of our senior executives;
- overseeing and administering, and making recommendations to our board of directors with respect to, our cash and equity incentive plans;
- calculating potential executive and succession plans; and
- reviewing and approving non-routine employment agreements, severance agreements and change in control agreements.

Nominating and Corporate Governance Committee

Messrs. Barkas, Raab and Hayden are the members of our nominating and corporate governance committee. Mr. Barkas chairs the committee.

Our nominating and corporate governance committee s responsibilities include:

- recommending to our board of directors the persons to be nominated for election as directors and to each of the board of director s committees;
- conducting searches for appropriate directors;
- reviewing the size, composition and structure of our board of directors;
- developing and recommending to our board of directors corporate governance principles;
- overseeing a periodic self-evaluation of our board of directors and any board committees; and
- overseeing compensation and benefits for directors and board committee members.

Compensation Committee Interlocks and Insider Participation

None of our executive officers serves as a member of the board of directors or compensation committee, or other committee serving an equivalent function, of any entity that has one or more of its executive officers serving as a member of our board of directors or our compensation committee. None of the members of our compensation committee has ever been our employee.

Director Compensation

Our board of directors will consider and intends to implement a compensation program pursuant to which we will pay each of our non-employee directors appropriate fees, whether in the form of cash compensation or equity or both, in support of our efforts to attract and retain qualified board members. We reimburse each non-employee member of our board of directors for out-of-pocket expenses incurred in connection with attending our board and committee meetings.

Executive Compensation

The following persons currently constitute our five highest paid executive officers:

Name	Position	Base Salary
John F. Crowley	President and Chief Executive Officer	$ 400,000
Matthew R. Patterson	Chief Business Officer	$ 280,000
David J. Lockhart, Ph.D.	Chief Scientific Officer	$ 280,000

Pedro Huertas, M.D., Ph.D.	Chief Strategic Officer	$	281,875
David Palling, Ph.D.	Senior Vice President, Drug Development	$	236,250

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The following summary compensation table sets forth the compensation paid or accrued for the year ended December 31, 2005 to our Chief Executive Officer and the four other highest paid executive officers serving as of December 31, 2005 and whose total annual compensation exceeded $100,000 for the year ended December 31, 2005. We use the term named executive officers to refer to these people later in this prospectus.

Summary Compensation Table

Name and Principal Position	Year	Salary	Bonus	Long-Term Compensation Awards: Shares Underlying Options	All Other Compensation
John F. Crowley President and Chief Executive Officer	2005	$ 384,872	$ 133,334	2,998,773	$ 261,000(1)
Matthew R. Patterson Chief Business Officer	2005	250,962	62,500	275,000	
David Palling, Ph.D. Senior Vice President, Drug Development	2005	225,866	56,250	225,000	
Pedro Huertas, M.D., Ph.D. Chief Strategic Officer	2005	121,629	59,375	894,101	16,929(2)
Gregory P. Licholai, M.D. Vice President, Medical Affairs	2005	215,827	68,000	803,417	
Norman Hardman, Ph.D.(3)	2005	322,355			

(1) Paid in connection with executive medical expense reimbursement.

(2) Paid in connection with relocation expense reimbursement, including a related tax gross-up payment.

(3) Dr. Hardman ceased to be our chief executive officer in January 2005, and ceased being one of our employees in May 2005.

Stock Option Grants

The following table contains information regarding grants of stock options to purchase shares of our common stock made to our named executive officers during the year ended December 31, 2005.

Amounts in the following table represent potential realizable gains that could be obtained for the options if exercised at the end of the option term. The 5% and 10% assumed annual rates of compounded stock price appreciation are calculated based on the requirements of the Securities and Exchange Commission, and do not represent an estimate or projection of our future common stock prices. These amounts represent certain assumed rates of appreciation in the value of our common stock from the fair market value on the date of grant. Actual gains, if any, on stock option exercises depend on the future performance of the common stock and overall stock market conditions. The amounts reflected in the following table may not necessarily be obtained.

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Option Grants in Last Fiscal Year

	Individual Grants				Potential Realizable Value of Assumed Annual Rates of Stock Price Appreciation for Option Term[3]	
Name	Number of Securities Underlying Options Granted (#)	Percent of Total Options Granted to Employees in Fiscal Year[1]	Exercise Price Per Share[2]	Expiration Date	5% ($)	10% ($)
John F. Crowley	2,248,773	32.6%	$ .085	1/6/2015		
	750,000	10.9%	.71	10/20/2015		
Matthew R. Patterson	275,000	4.0%	.71	10/20/2015		
David Palling, Ph.D.	225,000	3.3%	.71	10/20/2015		
Pedro Huertas, M.D., Ph.D.	724,101	10.5%	.085	6/9/2015		
	20,000	0.3%	.085	6/9/2015		
	150,000	2.2%	.71	10/20/2015		
Gregory P. Licholai, M.D.	603,417	8.7%	.085	1/3/2015		
	200,000	2.9%	.71	10/20/2015		
Norman Hardman, Ph.D.[4]	40,000		.085	6/9/2015		

(1) The figures representing percentages of total options granted to employees in the last fiscal year are based on a total of 6,904,785 option shares granted to our employees during fiscal year 2005.

(2) The exercise price of each option granted was equal to the fair market value of our common stock as valued by our board of directors on the date of grant. The exercise price may be paid in cash, cash equivalents, or in shares of our common stock.

(3) The dollar amounts under these columns are the result of calculations at rates set by the Securities and Exchange Commission and, therefore, are not intended to forecast possible future appreciation, if any, in the price of the underlying common stock. The potential realizable values are calculated using the assumed initial public offering price of $ per share and assuming that the market price appreciates from this price at the indicated rate for the entire term of each option and that each option is exercised and sold on the last day of its term at the assumed appreciated price.

(4) Dr. Hardman ceased to be our chief executive officer in January 2005, ceased being one of our employees in May 2005, and in 2005 received an option to purchase 40,000 shares of common stock in connection with his execution of a scientific advisory board agreement with us after he had left our employ.

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Option Exercises and Year-End Option Values

The following table provides information about the number of shares issued upon option exercises by our named executive officers during the year ended December 31, 2005, and the value realized by our named executive officers. The table also provides information about the number and value of shares underlying options held by our named executive officers at December 31, 2005. There was no public trading market for our common stock as of December 31, 2005. Accordingly, as permitted by the rules of the Securities and Exchange Commission, we have calculated the value of unexercised in-the-money options at fiscal year-end assuming that the fair market value of our common stock as of December 31, 2005 was equal to the assumed initial public offering price of $ per share, less the aggregate exercise price.

Aggregated Option Exercises in Last Fiscal Year and Fiscal Year-End Option Values

Name	Shares Acquired on Exercise (#)	Value Realized	Number of Securities Underlying Unexercised Options at December 31, 2005		Value of Unexercised In-the-Money Options at December 31, 2005	
			Exercisable	Unexercisable	Exercisable	Unexercisable
John F. Crowley	41,224		13,736	3,108,709		
Matthew R. Patterson			181,025	818,076		
David Palling, Ph.D.			323,409	525,008		
Pedro Huertas, M.D., Ph.D.	20,000			874,101		
Gregory P. Licholai, M.D.				803,417		
Norman Hardman, Ph.D.[1]	616,367		9,996	30,004		

(1) Dr. Hardman ceased to be our chief executive officer in January 2005, and ceased being one of our employees in May 2005.

Employment Agreements

John F. Crowley. Pursuant to an amended and restated employment agreement dated as of April 28, 2006, we employ Mr. Crowley as our president and chief executive officer. Under this agreement, Mr. Crowley is entitled to an annual base salary of $400,000. Adjustments to his base salary are in the discretion of our board of directors and we have agreed not to reduce his base salary below $400,000. The agreement provides that Mr. Crowley is eligible to receive a cash bonus of up to 50% of his base salary if performance criteria are met for the year in which the bonus is to be paid. The agreement provides that Mr. Crowley is eligible to participate in any executive bonus plans established by the board from time to time. We have agreed to secure and maintain an executive medical reimbursement contract with a named insurance company covering Mr. Crowley, his spouse and his dependents. We have also agreed that we shall reimburse Mr. Crowley up to $220,000 for any medical expenses incurred by Mr. Crowley, his spouse or his dependent children, if the amount of those expenses are not covered by the executive medical reimbursement contract or our medical or health insurance policies (and such amount shall be grossed up for any federal and state income tax incurred as a consequence of our reimbursement of such expenses and the grossing up thereof).

The agreement will continue for successive one-year terms until either Mr. Crowley or we provide written notice of termination to the other in accordance with the terms of the agreement. Upon the termination of his employment by us other than for cause, or if we decide not to extend Mr. Crowley s agreement at the end of any term, or termination

by him for good reason, Mr. Crowley has the right to receive (i) a severance payment in an amount equal to 18 times his monthly base salary then in effect, payable in accordance with our regular payroll practices, (ii) an additional payment equal to 150% of the target bonus for the year in which the termination occurs, and (iii) continuation of benefits for a comparable period as a result of any such termination. Further, the vesting of all options then held by

Mr. Crowley shall accelerate by one year. Mr. Crowley is not entitled to severance payments if we terminate him for cause or if he resigns without good reason. Mr. Crowley is bound by non-disclosure, inventions and non-competition covenants that prohibit him from competing with us during the term of his employment and for one year after termination of employment.

If Mr. Crowley resigns for good reason, we or our successor terminate him without cause, or we decide not to extend his employment agreement at the end of any term, in each case within 3 months prior to, or 12 months following a change of control, then Mr. Crowley has the right to receive a severance payment in an amount equal to twice his monthly base salary then in effect, payable over 24 months in accordance with our regular payroll schedule, as well as an additional payment equal to 200% of the target bonus for the year in which the termination occurs. In addition, Mr. Crowley is entitled to the continuation of benefits for a comparable period as a result of any such termination. Further, the vesting of all options then held by him shall accelerate in full, and all repurchase rights that we may have as to any of his stock will automatically lapse.

Other Executive Officers. We have entered into employment agreements with the following executive officers, which set forth the officer s position, duties, base salary and benefits, among other things: Matthew R. Patterson, David Lockhart, Ph.D., Karin Ludwig, M.D., Mark Simon, David Palling, Ph.D., Pedro Huertas, M.D., Ph.D., Gregory P. Licholai, M.D., S. Nicole Schaeffer and Douglas Branch.

Our executive employment agreements with Drs. Lockhart, Ludwig and Huertas and Messrs. Branch and Simon each provide that the executive shall be eligible to receive a bonus of up to 25% of base salary annually, if in the judgment of the compensation committee the qualifying criteria established by the committee for payment of a bonus have been met. Our executive employment agreement with Dr. Palling and Ms. Schaeffer provides that the executive shall be eligible to receive a bonus of up to 20% of base salary annually, if in the judgment of the compensation committee the qualifying criteria established by the committee for payment of a bonus have been met. Mr. Patterson s executive employment agreement provides that he shall be eligible to receive a bonus of up to $50,000 annually, if in the judgment of the compensation committee the qualifying criteria established by the committee for payment of a bonus have been met. Dr. Licholai s executive employment agreement provides that he shall be eligible to receive a bonus of up to $43,000 annually, if in the judgment of the compensation committee the qualifying criteria established by the committee for payment of a bonus have been met. Our executive employment agreements with Drs. Lockhart, Ludwig and Huertas and Mr. Patterson 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. Our executive employment agreements with Dr. Palling, Dr. Licholai, Ms. Schaeffer and Messrs. Branch and Simon have no term and are at will .

If any of Drs. Lockhart, Ludwig and Huertas or Messrs. Patterson or Simon is terminated without cause, then we will be obligated to pay that executive six months of base salary following that termination plus an amount equal to any bonus paid to such executive in the previous year. In addition, the vesting on options then held by them will automatically accelerate by six months. If any of Dr. Palling, Dr. Licholai, Ms. Schaeffer or Mr. Branch is terminated without cause, we will be obligated to pay that executive six months of base salary following termination.

The employment agreements with Mr. Patterson, Mr. Branch, Mr. Simon and Drs. Lockhart, Ludwig and Huertas, as well as severance agreements with Dr. Palling, Dr. Licholai, and Ms. Schaeffer, provide for severance benefits for those executives in the event we or our successor terminate such executive s employment other than for cause within six months following certain corporate changes or if, following those changes, the executive resigns for good reason. In either case, the executive has the right to receive:

- a lump-sum severance payment in an amount equal to 12 times his or her monthly base salary in effect as of the date of the corporate change;

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payment of a bonus equal to the bonus earned in the preceding year; and

any outstanding unvested stock options held by the executive will fully vest.

Each executive is bound by non-disclosure, inventions transfer, non-solicitation and non-competition covenants that prohibit the executive from competing with us during the term of his or her employment and for 12 months after termination of employment.

Stock Option and Other Compensation Plans

2002 Equity Incentive Plan

Our 2002 equity incentive plan, as amended, was adopted by our board of directors and approved by our stockholders. The plan provides for the grant of incentive and nonstatutory stock options to purchase shares of our common stock, and restricted and other stock awards, in each case to our employees, directors and consultants.

In accordance with the terms of the 2002 equity incentive plan, our board of directors or one or more committees appointed by the board of directors administers the plan.

Under our 2002 equity incentive plan, if a merger or other reorganization event occurs, the board of directors may either (i) make appropriate provision for the protection of any outstanding options by substitution on an equitable basis of appropriate stock of ours or securities of the merged, consolidated or otherwise reorganized corporation which are issuable in connection therewith, subject to certain conditions, or (ii) provide that all unexercised options must be exercised or they will be terminated.

As of May 3, 2006, there were options to purchase 13,552,120 shares of common stock outstanding under the 2002 equity incentive plan. After the effective date of this offering, we will grant no further stock options or other equity incentive awards under the 2002 equity incentive plan.

2006 Equity Incentive Plan

In May 2006, our board of directors and stockholders approved our 2006 equity incentive plan, to become effective on the closing of this offering. The 2006 equity incentive plan provides for the grant of incentive stock options, within the meaning of Section 422 of the Internal Revenue Code, to employees, and non-qualified stock options and restricted and other stock awards to our employees, directors, and consultants.

The aggregate number of shares of our common stock that may be issued under the 2006 equity incentive plan is . The aggregate number of shares of common stock that may be granted in any calendar year to any one person pursuant to the 2006 equity incentive plan may not exceed 50% of the aggregate number shares of our common stock that may be issued pursuant to the 2006 equity incentive plan.

The 2006 equity incentive plan will be administered by the compensation committee of our board of directors. Subject to the provisions of the 2006 equity incentive plan, the compensation committee has been granted the discretion to determine when awards are made, which directors, employees or consultants receive awards, whether an award will be in the form of an incentive stock option, a nonqualified stock option or stock (with or without restrictions), the number of shares subject to each award, and all other relevant terms of the award, including vesting and acceleration of vesting, if any. The compensation committee also has been granted broad discretion to construe and interpret the 2006 equity incentive plan and adopt rules and regulations thereunder. Generally, options granted under the 2006 equity incentive plan are expected to vest over a four-year period from the date of grant in the case of employees, and over a two-year period from the date of grant for consultants.

Our board of directors may amend, modify, or terminate our 2006 equity incentive plan at any time, subject to applicable rules and law and the rights of holders of outstanding awards. Our 2006 equity

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incentive plan will automatically terminate in May 2016 unless our board of directors terminates it prior to that time.

2006 Employee Stock Purchase Plan

In May 2006, our board of directors and stockholders approved our 2006 employee stock purchase plan, to become effective upon the closing of this offering. The 2006 employee stock purchase plan authorizes the issuance of up to a total of shares of our common stock to eligible employees. The 2006 employee stock purchase plan shall terminate in May 2011.

The 2006 employee stock purchase plan, which is intended to qualify under Section 423 of the Internal Revenue Code, will be implemented by a series of six-month offering periods. New offering periods are expected to commence on January 1 or July 1 of each year and end on the next following June 30 or December 31, respectively. Each offering period will generally consist of a consecutive six-month purchase period, and at the end of each six-month period an automatic purchase will be made for participants. The 2006 employee stock purchase plan will be administered by the board of directors or by a committee appointed by the board. Employees are eligible to participate if we employ them for at least 20 hours per week and more than five months per year. Eligible employees may purchase common stock through payroll deductions only after the effectiveness of an appropriate registration statement, which in any event may not exceed 15% of an employee s compensation, at a price equal to the lower of 85% of the fair market value of the common stock at the beginning of each offering period or at the end of each purchase period.

Under the 2006 employee stock purchase plan, no employee shall be granted an option under the plan if immediately after the grant the employee would own stock, including any outstanding options to purchase stock, equaling 5% or more of the total voting power or value of all classes of our stock. In addition, the 2006 employee stock purchase plan provides that no employee shall be granted an option under the 2006 employee stock purchase plan if the option would permit the employee to purchase stock under all of our employee stock purchase plans in an amount that exceeds $25,000 of the fair market value of such stock for each calendar year in which the option is outstanding at any time, and that no employee may purchase more than shares of common stock under the plan in any one purchase period. The board of directors may, at its discretion, prior to the beginning of an offering period, subject the shares acquired (or to be acquired) by employees for such offering period to certain transfer restrictions.

In the event of a merger, consolidation, or other acquisition event resulting in any change of control of us, each right to purchase stock under the 2006 employee stock purchase plan will be assumed, or an equivalent right will be substituted by, the successor corporation. Any ongoing offering period, however, will be shortened so that employees rights to purchase stock under the 2006 employee stock purchase plan are exercised prior to the transaction, unless the employee has withdrawn, in the event that the successor corporation refuses to assume each purchase right or to substitute an equivalent right. The board of directors has the power to amend or terminate the 2006 employee stock purchase plan and to change or terminate offering periods as long as any action does not adversely affect any outstanding rights to purchase stock. Our board of directors may amend or terminate the 2006 employee stock purchase plan or an offering period even if it would adversely affect outstanding options in order to avoid our incurring adverse accounting charges.

401(k) plan

We have established a 401(k) plan to allow our employees to save on a tax-favorable basis for their retirements. We have not matched contributions made by employees pursuant to the plan.

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Limitation of Liability and Indemnification of Officers and Directors

Our certificate of incorporation that will be in effect upon the closing of this offering limits the personal liability of directors for breach of fiduciary duty to the maximum extent permitted by the Delaware General Corporation Law. Our certificate of incorporation provides that no director will have personal liability to us or to our stockholders for monetary damages for breach of fiduciary duty or other duty as a director. However, these provisions do not eliminate or limit the liability of any of our directors:

for any breach of their duty of loyalty to us or our stockholders;

for acts or omissions not in good faith or which involve intentional misconduct or a knowing violation of law;

for voting or assenting to unlawful payments of dividends or other distributions; or

for any transaction from which the director derived an improper personal benefit.

Any amendment to or repeal of these provisions will not eliminate or reduce the effect of these provisions in respect of any act or failure to act, or any cause of action, suit or claim that would accrue or arise prior to any amendment or repeal or adoption of an inconsistent provision. If the Delaware General Corporation Law is amended to provide for further limitations on the personal liability of directors of corporations, then the personal liability of our directors will be further limited in accordance with the Delaware General Corporation Law.

In addition, our certificate of incorporation provides that we must indemnify our directors and officers and we must advance expenses, including attorneys fees, to our directors and officers in connection with legal proceedings, subject to very limited exceptions.

We have entered into, and intend to continue to enter into, separate indemnification agreements with each of our officers and directors. These agreements, among other things, require us to indemnify our officers and directors for certain expenses, including attorney s fees, judgments, fines and settlement amounts incurred by an officer or director in any action or proceeding arising out of their services as one of our officers and directors, or any of our subsidiaries or any other company or enterprise to which the person provides services at our request, to the fullest extent permitted by Delaware law. We will not indemnify an officer director, however, unless he or she acted in good faith, reasonably believed his or her conduct was in, and not opposed, to our best interests, and, with respect to any criminal action or proceeding, had no reason to believe his or her conduct was unlawful.

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CERTAIN RELATIONSHIPS AND RELATED PARTY TRANSACTIONS

Since January 1, 2003, we have engaged in the following transactions with our directors, executive officers and holders of more than 5% of our voting securities on an as converted to common stock basis, and affiliates of our directors, executive officers and holders of more than 5% of our voting securities. The following related party transactions are in addition to the compensation agreements and other arrangements we have made which are described as required in Management. We believe that all of these transactions were on terms as favorable as could have been obtained from unrelated third parties.

Private Placement of Securities

In May 2004 and April 2005, we issued an aggregate of 36,470,591 shares of our series B redeemable convertible preferred stock at a price of $0.85 per share, along with warrants entitling the holders to purchase an aggregate of 555,003 shares of our series B redeemable convertible preferred stock at a price of $0.85 per share at any time before May 4, 2014, for total cash proceeds to us of approximately $31.0 million before transaction expenses.

In August 2005 and April 2006, we issued an aggregate of 43,650,262 shares of our series C redeemable convertible preferred stock at a price of approximately $1.26 per share for total cash proceeds to us of approximately $55.0 million before transaction expenses.

The following table sets forth the number of shares of series B redeemable convertible preferred stock and series C redeemable convertible preferred stock sold to our 5% stockholders and directors and their affiliates in these financings. The shares of series B redeemable convertible preferred stock and series C redeemable convertible preferred stock referred to in the table will convert automatically on a one-for-one basis into shares of our common stock upon the closing of this offering.

Name	Number of Shares of Series B Redeemable Convertible Preferred Stock	Number of Shares of Series C Redeemable Convertible Preferred Stock
Entities affiliated with Prospect Venture Partners (1)	7,564,370	7,621,664
Entities affiliated with New Enterprise Associates (2)	7,564,369	7,621,664
Entities affiliated with Frazier Healthcare Ventures (3)	7,564,368	7,621,664
Entities affiliated with Canaan Partners(4)	7,094,582	6,806,250
Entities affiliated with CHL Medical Partners(5)	5,971,870	3,968,254
Entities affiliated with Quaker Bioventures(6)		7,936,506
Total	35,759,559	41,576,002

(1) Includes 113,467 shares of series B redeemable convertible preferred stock (including the automatic exercise of outstanding warrants to purchase 1,701 shares of series B redeemable convertible preferred stock) and 114,326 shares of series C redeemable convertible preferred stock, in each case issued to Prospect Associates II, L.P., and 7,450,903 shares of series B redeemable convertible preferred stock (including the automatic exercise of outstanding warrants to purchase 111,687 shares of series B redeemable convertible preferred stock) and 7,507,338 shares of series C redeemable convertible preferred stock issued to Prospect Venture Partners II, L.P. Dr. Barkas, one of our directors, is a Managing Member of the General Partner of both Prospect Venture Partners II, L.P., and Prospect Associates II, L.P.

(2) Includes 20,304 shares of series B redeemable convertible preferred stock issued to NEA Ventures 2004, Limited Partnership (including the automatic exercise of outstanding warrants to purchase 304 shares of series B redeemable convertible preferred stock), and 7,544,065 shares of series B redeemable convertible preferred stock (including the automatic exercise of outstanding warrants to purchase 113,083 shares of series B redeemable convertible preferred stock) and 7,621,664 shares of series C redeemable convertible preferred stock issued to

New Enterprise Associates 11, L.P. Mr. Raab, one of our directors, is a partner of New Enterprise Associates.

(3) Includes 38,205 shares of series B redeemable convertible preferred stock (including the automatic exercise of outstanding warrants to purchase 573 shares of series B redeemable convertible preferred stock) and 38,494 shares of series C redeemable convertible preferred stock issued to Frazier Affiliates IV, L.P., and 7,526,163 shares of series B redeemable convertible preferred stock (including the automatic exercise of outstanding warrants to purchase 112,815 shares of series B redeemable convertible preferred stock) and 7,583,170 shares of series C redeemable convertible preferred stock issued to Frazier Healthcare IV, L.P. Dr. Topper, one of our directors, holds the title of General Partner with Frazier Healthcare Ventures.

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(4) Includes 6,839,178 shares of series B redeemable convertible preferred stock (including the automatic exercise of outstanding warrants to purchase 102,518 shares of series B redeemable convertible preferred stock) and 6,561,226 shares of series C redeemable convertible preferred stock issued to Canaan Equity III, L.P., and 255,404 shares of series B redeemable convertible preferred stock (including the automatic exercise of outstanding warrants to purchase 3,828 shares of series B redeemable convertible preferred stock) and 245,024 shares of series C redeemable convertible preferred stock issued to Canaan Equity III Entrepreneurs, LLC. Dr. Bloch, one of our directors, is a Member of Canaan Equity Partners III, LLC, the sole general partner of Canaan Equity III, L.P. and the sole manager of Canaan Equity III Entrepreneurs, LLC.

(5) Includes 5,520,337 shares of series B redeemable convertible preferred stock and 3,717,758 shares of series C redeemable convertible preferred stock issued to CHL Medical Partners II, L.P. and 451,533 shares of series B redeemable convertible preferred stock and 250,496 shares of series C redeemable convertible preferred stock issued to CHL Medical Partners II Side Fund, L.P. Dr. Weinhoff, one of our directors, is a Member of the General Partner of both CHL Medical Partners II, L.P. and CHL Medical Partners II Side Fund, L.P.

(6) Includes 5,952,380 shares of series C redeemable convertible preferred stock issued to Quaker Bioventures, L.P. and 1,984,126 shares of series C redeemable convertible preferred stock issued to Garden State Life Sciences Venture Fund, L.P. Mr. Neff, one of our directors, is a member of the general partner of the general partner of both Quaker Bioventures, L.P. and Garden State Life Sciences Venture Fund, L.P.

Bridge Financings

In April 2003, June 2003, August 2003, November 2003, February 2004 and April 2004, we issued (inclusive of certain warrants to purchase common stock which have been exercised) convertible promissory notes in an aggregate principal amount of $5.5 million to certain investors.

The notes accrued interest at the prime rate plus 2%. In the event that we completed an equity financing resulting in gross proceeds to us of at least $12.0 million, the notes were automatically convertible into shares of the same class of equity issued in the financing. $5,000,000 of principal outstanding under the notes converted into shares of our series B redeemable convertible preferred stock in connection with our series B redeemable convertible preferred stock financing in May 2004. The other $500,000 of principal outstanding under the notes was repaid by the Company in May 2004.

The following table sets forth the names of holders of more than 5% of our capital stock who participated in these bridge financings, the principal amount of the notes held in the aggregate by these holders, and the number of shares of our series B redeemable convertible preferred stock issued upon conversion of the notes.

Holders of more than 5%	Aggregate Principal Amount of Notes Held	Shares of Series B Redeemable Convertible Preferred Stock Issued upon Conversion
CHL Medical Partners II, L.P.	$ 5,089,438	5,436,471
CHL Medical Partners Side Fund II, L.P.	$ 410,562	445,882

In connection with these bridge financings, we also issued warrants to the investors that were exercisable in the aggregate for 999,999 shares of our common stock at an exercise price of seven and one-half cents ($0.075) per share. The investors exercised all of these common stock warrants in August 2005.

Certain Relationships

Registration Rights

Pursuant to a second amended and restated investor rights agreement among holders of our redeemable convertible preferred stock and us, we granted registration rights to all such holders and to the holder of a warrant to

purchase 40,000 shares of our common stock. Entities affiliated with Prospect Venture Partners II, L.P., New Enterprise Associates, Frazier Healthcare Ventures, Canaan Equity, Quaker BioVentures and CHL Medical Partners, each holders of 5% or more of our voting securities, and

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their affiliates are parties to this investor rights agreement. See Description of Capital Stock Registration Rights.

Director Compensation

Please see Management Director Compensation for a discussion of options granted and other compensation to our non-employee directors.

Executive Compensation and Employment Agreements

Please see Management Executive Compensation and Management Stock Options for additional information on compensation of our executive officers. Information regarding employment agreements with our executive officers is set forth under Management Employment Agreements.

Indemnification Agreements

We have entered into indemnification agreements with each of our officers and directors. These agreements, among other things, require us to indemnify each officer and director to the fullest extent permitted by Delaware law, including indemnification of expenses such as attorneys fees, judgments, fines and settlement amounts incurred by the officer or director in any action or proceeding, including any action or proceeding by or in right of us, arising out of the person s services as an officer or director. We will not indemnify an officer or director, however, unless he or she acted in good faith, reasonably believed his or her conduct was in, and not opposed, to our best interests and, with respect to any criminal action or proceeding, had no reason to believe his or her conduct was unlawful.

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PRINCIPAL STOCKHOLDERS

The following table sets forth information with respect to the beneficial ownership of our common stock, as of May 3, 2006, by:

- each of our directors;
- each of our executive officers;
- each person, or group of affiliated persons, who is known by us to beneficially own more than 5% of our common stock; and
- all of our directors and executive officers as a group.

The column entitled Percentage of Shares Beneficially Owned Before Offering is based on a total of 89,516,214 shares of our common stock outstanding on May 3, 2006, assuming the automatic exercise of all outstanding warrants to purchase 465,486 shares of series B redeemable convertible preferred stock and the conversion of all outstanding shares of our redeemable convertible preferred stock into 84,009,910 shares of our common stock upon the closing of this offering. The column entitled Percentage of Shares Beneficially Owned After Offering is based on shares of common stock to be outstanding after this offering, including the shares that we are selling in this offering, but not including any shares issuable upon exercise of warrants or options outstanding after this offering.

For purposes of the table below, we deem shares of common stock subject to options or warrants that are currently exercisable or exercisable within 60 days of May 3, 2006 to be outstanding and to be beneficially owned by the person holding the options or warrants for the purpose of computing the percentage ownership of that person but we do not treat them as outstanding for the purpose of computing the percentage ownership of any other person. Except as otherwise noted, the persons or entities in this table have sole voting and investing power with respect to all of the shares of common stock beneficially owned by them, subject to community property laws, where applicable. Except as otherwise set forth below, the street address of the beneficial owner is c/o Amicus Therapeutics, Inc., 6 Cedar Brook Drive, Cranbury, NJ 08512.

Name and Address of Beneficial Owner	Number of Shares Beneficially Owned	Percentage of Shares Beneficially Owned	
		Before Offering	After Offering
5% Stockholders			
Entities affiliated with Prospect Venture Partners II, L.P.[1] 435 Tasso Street, Suite 200 Palo Alto, CA 94301	15,186,034	17.0%	
Entities affiliated with New Enterprise Associates[2] 1119 St. Paul Street Baltimore, MD 21202	15,186,033	17.0%	
Entities affiliated with Frazier Healthcare Ventures [3] 601 Union, Two Union Square, Suite 3200 Seattle, WA 98101	15,186,032	17.0%	
Entities affiliated with CHL Medical Partners[4] 1055 Washington Boulevard, 6th Floor Stamford, CT 06901	14,273,457	15.9%	

Entities affiliated with Canaan Partners[5] 105 Rowayton Avenue Rowayton, CT 06853	13,900,832	15.5%
Entities affiliated with Quaker Bioventures[6] Cira Center 2929 Arch Street Philadelphia, PA 19104-2868	7,936,506	8.9%

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Name and Address of Beneficial Owner	Number of Shares Beneficially Owned	Percentage of Shares Beneficially Owned Before Offering	Percentage of Shares Beneficially Owned After Offering
Executive Officers and Directors			
John F. Crowley[7]	922,302	1.0%	
David Palling, Ph.D.[8]	404,206	*	
Matthew R. Patterson[9]	286,627	*	
Gregory P. Licholai, M.D.[10]	238,858	*	
Pedro Huertas, M.D., Ph.D.[11]	201,025	*	
S. Nicole Schaeffer[12]	50,783	*	
David Lockhart, Ph.D.			
Karin Ludwig, M.D.			
Mark Simon			
Douglas Branch[13]	24,996	*	
Donald J. Hayden[14]	52,085	*	
Alexander E. Barkas, Ph.D.[15]			
Michael G. Raab[16]			
James N. Topper, M.D., Ph.D.[17]			
Stephen Bloch, M.D.[18]			
Gregory M Weinhoff, M.D.[19]			
P. Sherrill Neff[20]			
All directors and executive officers as a group (16 persons)/[21]	2,180,882	2.4%	

* Represents beneficial ownership of less than one percent of our outstanding common stock.

(1) Consists of 14,958,241 shares held of record by Prospect Venture Partners II, L.P. including 111,687 shares assuming the exercise of outstanding warrants held by Prospect Venture Partners II, L.P., and 227,793 shares held of record by Prospect Associates II, L.P. including 1,701 shares assuming the exercise of outstanding warrants held by Prospect Associates II, L.P. Dr. Barkas, a member of our board of directors and a Managing Member of the General Partner of both Prospect Venture Partners II, L.P. and Prospect Associates II, L.P., disclaims beneficial ownership of the shares held by entities affiliated with Prospect Venture Partners II, L.P. except, to the extent of any pecuniary interest therein.

(2) Consists of 15,165,729 shares held of record by New Enterprise Associates 11, Limited Partnership including 113,083 shares assuming the exercise of outstanding warrants held by New Enterprise Associates 11, Limited Partnership, and 20,304 shares held of record by NEA Ventures 2004, Limited Partnership including 304 shares assuming the exercise of outstanding warrants held by NEA Ventures 2004, Limited Partnership. Mr. Raab is a partner of New Enterprise Associates but does not have voting or dispositive power with respect to the shares held by New Enterprise Associates 11, Limited Partnership or NEA Ventures 2004, Limited Partnership and disclaims beneficial ownership of shares held by New Enterprise Associates 11, Limited Partnership, except to the to the extent of his pecuniary interest therein. Mr. Raab has no pecuniary interest in the shares held by NEA Ventures 2004, Limited Partnership.

(3) Consists of 15,109,333 shares held of record by Frazier Healthcare IV, L.P. including 112,815 shares assuming the exercise of outstanding warrants held by Frazier Healthcare IV, L.P. and 76,699 shares held of record by

Frazier Affiliates IV, L.P. including 573 shares assuming the exercise of outstanding warrants held by Frazier Affiliates IV, L.P. Dr. Topper, a member of our board of directors, holds the title of General Partner with Frazier Healthcare Ventures. In that capacity he shares voting and investment power for the shares held by both Frazier Healthcare IV, L.P. and Frazier Affiliates IV, L.P. Dr. Topper disclaims beneficial ownership of the shares held by entities affiliated with Frazier Healthcare Ventures, except to the extent of any pecuniary interest therein.

(4) Consists of 13,297,885 shares held of record by CHL Medical Partners II, L.P. and 975,572 shares held of record by CHL Medical Partners II Side Fund, L.P. Dr. Weinhoff, a member of our board of directors and a Member of the General Partner of both CHL Medical Partners II, L.P. and CHL Medical Partners II Side Fund, L.P., disclaims beneficial ownership of the shares held by entities affiliated with CHL Medical Partners, except to the extent of any pecuniary interest therein.

(5) Consists of 13,400,404 shares held of record by Canaan Equity III, L.P. including 102,518 shares assuming the exercise of outstanding warrants held by Canaan Equity III, L.P. and 500,428 shares held of record by Canaan Equity III Entrepreneurs, LLC including 3,828 shares assuming the exercise of outstanding warrants held by Canaan Equity III Entrepreneurs, LLC. Canaan Equity Partners III, LLC, the sole general partner of Canaan Equity III, L.P. and sole manager of Canaan Equity III Entrepreneurs, LLC, has sole voting and disposition power over these shares. The Managers of Canaan Equity Partners, III,

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LLC are John V. Balen, James C. Furnival, Stephen L. Green, Deepak Kamra, Gregory Kopchinsly, Seth A. Rudnick, Guy M. Russo and Eric A. Young. Dr. Bloch, a member of our board of directors, is a member of Canaan Equity Partners III, LLC. Dr. Bloch does not have sole or shared voting or disposition power over these shares.

(6) Consists of 5,952,380 shares held of record by Quaker Bioventures, L.P. and 1,984,126 shares held of record by Garden State Life Sciences Venture Fund, L.P. Mr. Neff, a member of our board of directors and a Member of the General Partner of both Quaker Bioventures, L.P., and Garden State Life Sciences Ventured Fund, L.P. disclaims beneficial ownership of the shares held by entities affiliated with Quaker Bioventures, except to the extent of any pecuniary interest therein.

(7) Consists of 281,078 shares issuable upon the exercise of stock options exercisable within 60 days of May 3, 2006, and 641,224 shares held of record. Includes 100,000 shares held of record by MPAJ, LLC, for which Mr. Crowley has sole voting and dispositive power.

(8) Consists of 37,711 shares issuable upon the exercise of stock options exercisable within 60 days of May 3, 2006, and 366,495 shares held of record.

(9) Consists of 46,627 shares issuable upon the exercise of stock options exercisable within 60 days of May 3, 2006, and 240,000 shares held of record.

(10) Consists of 37,716 shares issuable upon the exercise of stock options exercisable within 60 days of May 3, 2006, and 201,142 shares held of record.

(11) Consists of 181,025 shares issuable upon the exercise of stock options exercisable within 60 days of May 3, 2006, and 20,000 shares held of record.

(12) Consists of 9,522 shares issuable upon the exercise of stock options exercisable within 60 days of May 3, 2006, and 41,261 shares held of record.

(13) Consists of 24,996 shares issuable upon the exercise of stock options exercisable within 60 days of May 3, 2006.

(14) Consists of 52,085 shares issuable upon the exercise of stock options exercisable within 60 days of May 3, 2006.

(15) Dr. Barkas disclaims beneficial ownership of the shares held by affiliates of Prospect Venture Partners II, L.P. except to the extent of his pecuniary interest therein. See footnote 1.

(16) Mr. Raab disclaims beneficial ownership of the shares held by New Enterprise Associates, except to the extent of his pecuniary interest therein. See footnote 2.

(17) Dr. Topper disclaims beneficial ownership of the shares held by Frazier Healthcare, except to the extent of his pecuniary interest therein. See footnote 3.

(18) Dr. Bloch does not have sole or shared voting or dispositive power over shares owned by entities affiliated with Canaan Partners. Dr. Bloch disclaims beneficial ownership of such shares, except to the extent of his pecuniary interest therein. See footnote 5.

(19) Dr. Weinhoff disclaims beneficial ownership of the shares held by CHL Medical Partners, except to the extent of his pecuniary interest therein. See footnote 4.

(20) Mr. Neff disclaims beneficial ownership of the shares held by Quaker Bioventures, except to the extent of his pecuniary interest therein. See footnote 6.

(21) Consists of 670,760 total shares issuable upon the exercise of stock options exercisable within 60 days of May 3, 2006, and 1,510,122 total shares held of record.

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DESCRIPTION OF CAPITAL STOCK

The following description of our capital stock and provisions of our certificate of incorporation and bylaws are summaries and are qualified by reference to the certificate of incorporation and the bylaws that will be in effect upon the closing of this offering. We have filed copies of forms of these documents with the Securities and Exchange Commission as exhibits to our Registration Statement of which this prospectus forms a part. The description of the capital stock reflects changes to our capital structure that will occur upon the closing of this offering.

Upon the closing of this offering, our authorized capital stock will consist of shares of common stock, par value $0.01 per share, and 5,000,000 shares of preferred stock, par value $0.01 per share, all of which preferred stock will be undesignated.

As of May 3, 2006, we had issued and outstanding:

- 5,507,024 shares of common stock outstanding held by 17 stockholders of record;
- 3,333,334 shares of series A redeemable convertible preferred stock that are convertible into 3,333,334 shares of common stock;
- 36,560,108 shares of series B redeemable convertible preferred stock that are convertible into 36,560,108 shares of common stock; and
- 43,650,262 shares of series C redeemable convertible preferred stock that are convertible into 43,650,262 shares of common stock.

As of May 3, 2006, we also had outstanding:

- options to purchase 13,552,120 shares of common stock at a weighted average exercise price of $0.48 per share;
- warrants to purchase an aggregate of 465,486 shares of series B redeemable convertible preferred stock at an exercise price of $0.85 per share, which warrants are automatically exercised upon the closing of this offering; and
- a warrant to purchase 40,000 shares of common stock at an exercise price of $0.75 per share.

Upon the closing of this offering, all of the outstanding shares of our redeemable convertible preferred stock will automatically convert into a total of 84,009,190 shares of our common stock, assuming the automatic exercise of all outstanding warrants to purchase 465,486 shares of series B redeemable convertible preferred stock.

Common Stock

Holders of our common stock are entitled to one vote for each share held on all matters submitted to a vote of stockholders and do not have cumulative voting rights. An election of directors by our stockholders shall be determined by a plurality of the votes cast by the stockholders entitled to vote on the election. Holders of common stock are entitled to receive proportionately any dividends as may be declared by our board of directors, subject to any preferential dividend rights of any outstanding preferred stock.

In the event of our liquidation or dissolution, the holders of common stock are entitled to receive proportionately all assets available for distribution to stockholders after the payment of all debts and other liabilities and subject to the prior rights of any outstanding preferred stock. Holders of common stock have no preemptive, subscription, redemption or conversion rights. The rights, preferences and privileges of holders of common stock are subject to and may be adversely affected by the rights of the holders of shares of any series of preferred stock that we may designate and issue in the future.

Preferred Stock

Under the terms of our certificate of incorporation to be effective at closing, our board of directors is authorized to issue shares of preferred stock in one or more series without stockholder approval. Our board of directors has the discretion to determine the rights, preferences, privileges and restrictions, including

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voting rights, dividend rights, conversion rights, redemption privileges and liquidation preferences, of each series of preferred stock.

The purpose of authorizing our board of directors to issue preferred stock and determine its rights and preferences is to eliminate delays associated with a stockholder vote on specific issuances. The issuance of preferred stock, while providing flexibility in connection with possible acquisitions, future financings and other corporate purposes, could have the effect of making it more difficult for a third party to acquire, or could discourage a third party from seeking to acquire, a majority of our outstanding voting stock. Upon the closing of this offering, there will be no shares of preferred stock outstanding, and we have no present plans to issue any shares of preferred stock.

Warrants

As of the closing of this offering, we have an outstanding warrant to purchase an aggregate of 40,000 shares of common stock at an exercise price of $0.75.

Options

As of May 3, 2006, options to purchase 13,552,120 shares of common stock at a weighted average exercise price of $0.48 per share were outstanding.

Anti-Takeover Effects of Delaware Law and our Corporate Charter Documents

Delaware Law

We are subject to Section 203 of the Delaware General Corporation Law. Subject to certain exceptions, Section 203 prevents a publicly held Delaware corporation from engaging in a business combination with any interested stockholder for three years following the date that the person became an interested stockholder, unless the interested stockholder attained such status with the approval of our board of directors or unless the business combination is approved in a prescribed manner. A business combination includes, among other things, a merger or consolidation involving us, sales of our assets, or other transactions resulting in a financial benefit to the interested stockholder. In general, an interested stockholder is any entity or person beneficially owning, or in the past three years owning, 15% or more of our outstanding voting stock and any entity or person affiliated with or controlling or controlled by such entity or person. The restrictions contained in Section 203 are not applicable to any of our existing stockholders that will own 15% or more of our outstanding voting stock upon the closing of this offering. This statute could prohibit or delay the accomplishment of mergers or other takeover or change in control attempts with respect to us and accordingly, may discourage attempts to acquire us.

Staggered Board

Our certificate of incorporation and our bylaws to be effective at closing of this offering divide our board of directors into three classes with staggered three-year terms. In addition, our certificate of incorporation and our bylaws to be effective upon the closing of this offering provide that directors may be removed only for cause and only by the affirmative vote of a majority of the holders of our shares of capital stock present in person or by proxy and entitled to vote. Under our certificate of incorporation and bylaws, any vacancy on our board of directors, including a vacancy resulting from an enlargement of our board of directors, may be filled only by vote of a majority of our directors then in office. Furthermore, our bylaws provide that the authorized number of directors may be changed only by the resolution of our board of directors. The classification of our board of directors and the limitations on the ability of our stockholders to remove directors, change the authorized number of directors, and fill vacancies could make it more difficult for a third party to acquire, or discourage a third party from seeking to acquire, control of our company.

Stockholder Action; Special Meeting of Stockholders; Advance Notice Requirements for Stockholder Proposals and Director Nominations

Our certificate of incorporation and our bylaws to be effective at closing of this offering provide that any action required or permitted to be taken by our stockholders at an annual meeting or special meeting

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of stockholders may only be taken if it is properly brought before such meeting and may not be taken by written action in lieu of a meeting. Our certificate of incorporation and our bylaws also provide that, except as otherwise required by law, special meetings of the stockholders can only be called by our chairman of the board, our president, or a majority of our board of directors. In addition, our bylaws establish an advance notice procedure for stockholder proposals to be brought before an annual meeting of stockholders, including proposed nominations of candidates for election to the board of directors. Stockholders at an annual meeting may only consider proposals or nominations specified in the notice of meeting or brought before the meeting by, or by a stockholder of record on the record date for the meeting, who is entitled to vote at the meeting and who has delivered timely written notice in proper form to our secretary of the stockholder s intention to bring such business before the meeting. These provisions could have the effect of delaying until the next stockholder meeting stockholder actions that are favored by the holders of a majority of our outstanding voting securities.

Authorized But Unissued Shares

The authorized but unissued shares of common stock and preferred stock are available for future issuance without stockholder approval, subject to any limitations imposed by the Nasdaq National Market. These additional shares may be utilized for a variety of corporate acquisitions and employee benefit plans. The existence of authorized but unissued and unreserved common stock could make more difficult or discourage an attempt to obtain control of us by means of a proxy contest, tender offer, merger, or otherwise.

Super-Majority Voting

The Delaware General Corporation Law provides generally that the affirmative vote of a majority of the shares entitled to vote on any matter is required to amend a corporation s certificate of incorporation or bylaws, unless a corporation s certificate of incorporation or bylaws, as the case may be, requires a greater percentage. Our bylaws to be effective at closing of this offering may be amended or repealed by a majority vote of our board of directors or the affirmative vote of the holders of at least 67% of our outstanding voting stock to amend, repeal or adopt any provisions inconsistent with provisions described above and contained in our by-laws. In addition, the affirmative vote of the holders of at least 67% of our outstanding voting stock is required to amend or repeal or to adopt any provisions inconsistent with any of the provisions of our certificate of incorporation described above, or to amend certain provisions relating to indemnification.

Board Discretion in Considering Certain Offers

Our certificate of incorporation to be effective at closing of this offering empowers our board of directors, when considering a tender offer or merger or acquisition proposal, to take into account factors in addition to potential economic benefits to stockholders. Such factors may include (i) comparison of the proposed consideration to be received by stockholders in relation to the then-current market price of our capital stock, our estimated current value in a freely negotiated transaction, and our estimated future value as an independent entity, and (ii) the impact of such a transaction on our employees, suppliers, and customers and its effect on the communities in which we operate.

Limitation of Liability

Our certificate of incorporation to be effective at closing of this offering contains certain provisions permitted under the Delaware General Corporation Law relating to the liability of directors. These provisions eliminate a director s personal liability for monetary damages resulting from a breach of fiduciary duty, except in certain circumstances involving certain wrongful acts, such as the breach of a director s duty of loyalty or acts or omissions that involve intentional misconduct or a knowing violation of law. These provisions do not limit or eliminate our rights or the rights of any stockholder to seek non-monetary relief, such as an injunction or rescission, in the event of a breach of a director s fiduciary duty. These provisions will not alter a director s liability under federal securities laws. Our certificate of incorporation and by-laws to be effective on closing also contain provisions indemnifying our directors and

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officers to the fullest extent permitted by the Delaware General Corporation Law. We believe that these provisions will assist us in attracting and retaining qualified individuals to serve as directors.

Registration Rights

Upon the closing of this offering, holders of an aggregate of 84,049,190 shares of our common will have the right to require us to register these shares under the Securities Act under specified circumstances.

Demand Registration Rights

After the closing of this offering and subject to certain limitations, these stockholders may require on up to two occasions, and as long as the aggregate price to the public for the securities to be sold in each instance is $5,000,000 or more, that we use our best efforts register all or part of their securities for sale under the Securities Act.

Form S-3 Registration Rights

If we register any of our common stock on Form S-3, either for our own account or for the account of other securityholders, these stockholders holding registration rights are entitled to notice of the registration and further entitled to include their shares of common stock in the registration. This right is subject to specified limitations, including but not limited to (i) if the Company has already effected a registration within 90 days or has effected two or more registration statements on Form S-3 within the preceding 12 month period and (ii) if the aggregate price to the public for the securities to be sold is less than $2,500,000. Additionally, if we certify that such registration would have a materially detrimental effect on any material corporate event, we may delay the request for up to three months, but not more than once in any twelve month period.

Incidental Registration Rights

At any time after this offering, if we register any of our common stock, either for our own account or for the account of other securityholders, then all holders of registrable securities are entitled to notice of the registration and to include their shares of common stock in the registration. In the case of an underwritten registration, we must use our reasonable efforts to obtain the permission of the underwriters to the inclusion of the holder s shares in the offering on the same terms.

Limitations and Expenses

With specified exceptions, a holder s right to include shares in a registration is subject to the right of the underwriters to limit the number of shares included in the offering. All fees, costs and expenses of any registrations will generally be paid by us.

Transfer Agent and Registrar

The transfer agent and registrar for our common stock will be following the closing of this offering.

Nasdaq National Market

We have applied to have our common stock approved for quotation on The Nasdaq National Market under the symbol AMTX.

SHARES ELIGIBLE FOR FUTURE SALE

Prior to this offering, there has been no public market for our common stock and a liquid trading market for our common stock may not develop or be sustained after this offering. Future sales of substantial amounts of common stock, including shares issued upon exercise of outstanding options and warrants or in the public market after this offering, or the anticipation of those sales, could adversely affect market prices prevailing from time to time and could impair our ability to raise capital through sales of our equity securities.

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Upon the closing of this offering, we will have outstanding shares of common stock, after giving effect to the issuance of shares of common stock in this offering and the automatic conversion of all outstanding shares of our convertible preferred stock, into an aggregate of 84,009,190 shares of our common stock, assuming the automatic exercise of all outstanding warrants to purchase 465,486 shares of series B redeemable convertible preferred stock, and assuming no exercise of the underwriters over-allotment option and no exercise of options or other warrants outstanding as of May 3, 2006.

Of the shares to be outstanding immediately after the closing of this offering, the shares to be sold in this offering will be freely tradable without restriction under the Securities Act unless purchased by our affiliates, as that term is defined in Rule 144 under the Securities Act. The remaining 89,516,214 shares of common stock are restricted securities under Rule 144. Substantially all of these restricted securities will be subject to the 180-day lock-up period described below.

After the 180-day lock-up period, these restricted securities may be sold in the public market only if registered or if they qualify for an exemption from registration under Rules 144 or 701 under the Securities Act, which exemptions are summarized below.

Rule 144

In general, under Rule 144, beginning 90 days after the date of this prospectus, a person who has beneficially owned shares of our common stock for at least one year, including the holding period of any prior owner other than one of our affiliates, would be entitled to sell within any three-month period a number of shares that does not exceed the greater of:

- 1% of the number of shares of our common stock then outstanding, which will equal approximately shares immediately after this offering, and
- the average weekly trading volume in our common stock on The Nasdaq National Market during the four calendar weeks preceding the date of filing of a Notice of Proposed Sale of Securities Pursuant to Rule 144 with respect to the sale.

Sales under Rule 144 are also subject to manner of sale provisions and notice requirements, and to the availability of current public information about us.

Upon expiration of the 180-day lock-up period described below, of shares of our common stock will be eligible for sale under Rule 144, excluding shares eligible for resale under Rule 144(k) as described below. We cannot estimate the number of shares of common stock that our existing stockholders will elect to sell under Rule 144.

Rule 144(k)

Subject to the lock-up agreements described below, shares of our common stock eligible for sale under Rule 144(k) may be sold immediately upon the closing of this offering. In general, under Rule 144(k), a person may sell shares of common stock acquired from us immediately upon the closing of this offering, without regard to manner of sale, the availability of public information about us or volume limitations, if:

- the person is not our affiliate and has not been our affiliate at any time during the three months preceding the sale; and
- the person has beneficially owned the shares proposed to be sold for at least two years, including the holding period of any prior owner other than our affiliates.

Upon the expiration of the 180-day lock-up period described below, approximately shares of common stock will be eligible for sale under Rule 144(k).

Rule 701

In general, under Rule 701 of the Securities Act, any of our employees, consultants or advisors who purchased shares from us in connection with a qualified compensatory stock plan or other written agreement is eligible to resell those shares 90 days after the effective date of the offering in reliance on

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Rule 144, but without compliance with the various restrictions, including the holding period, contained in Rule 144. Subject to the 180-day lock-up period described below, approximately shares of our common stock will be eligible for sale in accordance with Rule 701.

Lock-up Agreements

We expect that the holders of substantially all of our currently outstanding capital stock will agree that, without the prior written consent of Morgan Stanley, they will not, during the period ending 180 days after the date of this prospectus, subject to exceptions specified in the lock-up agreements, offer, sell, contract to sell or otherwise dispose of, directly or indirectly, or hedge our common stock or securities convertible into or exchangeable for or exercisable for our common stock, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase, lend or otherwise dispose of, directly or indirectly, any shares of our common stock or any securities convertible into or exercisable for our common stock. Further, these holders have agreed that, during this period, they will not make any demand for, or exercise any right with respect to, the registration of our common stock.

Registration Rights

Upon the closing of this offering, the holders of an aggregate of 84,049,190 shares of our common stock will have the right to require us to use our best efforts register these shares under the Securities Act under specified circumstances. After registration pursuant to these rights, these shares will become freely tradable without restriction under the Securities Act. Please see Description of Capital Stock Registration Rights for additional information regarding these registration rights.

Stock Options

As of May 3, 2006, we had outstanding options to purchase 13,552,120 shares of common stock, of which options to purchase 795,381 shares were vested. In connection with this offering, we intend to file a registration statement on Form S-8 under the Securities Act to register all of the shares of common stock subject to outstanding options and other awards issuable pursuant to our 2002 equity incentive plan, our 2006 equity incentive plan and our 2006 employee stock purchase plan. Please see Management-Stock Option and Other Compensation Plans for additional information regarding these plans. Accordingly, shares of our common stock registered under the registration statements will be available for sale in the open market, subject to Rule 144 volume limitations applicable to affiliates, and subject to any vesting restrictions and lock-up agreements applicable to these shares.

Warrants

Upon the closing of this offering, we will have an outstanding warrant to purchase an aggregate of 40,000 shares of our common stock at an exercise price of $0.75 per share. Any shares purchased pursuant to the cashless exercise features of this warrant will be freely tradeable under Rule 144(k), subject to the 180-day lock-up period described above.

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UNDERWRITERS

Under the terms and subject to the conditions contained in an underwriting agreement dated the date of this prospectus, the underwriters named below, for whom Morgan Stanley & Co. Incorporated, Goldman, Sachs & Co. and Pacific Growth Equities, LLC are acting as representatives, have severally agreed to purchase, and we have agreed to sell to them, the number of shares of common stock indicated in the table below:

Name	Number of Shares
Morgan Stanley & Co. Incorporated	
Goldman, Sachs & Co.	
Pacific Growth Equities, LLC	
Total	

The underwriters are offering the shares of common stock subject to their acceptance of the shares from us and subject to prior sale. The underwriting agreement provides that the obligations of the several underwriters to pay for and accept delivery of the shares of common stock offered by this prospectus are subject to the approval of certain legal matters by their counsel and to other conditions. The underwriters are obligated to take and pay for all of the shares of common stock offered by this prospectus if any such shares are taken. However, the underwriters are not required to take or pay for the shares covered by the underwriters over-allotment option described below.

The underwriters initially propose to offer part of the shares of common stock directly to the public at the public offering price listed on the cover page of this prospectus, and part to certain dealers at a price that represents a concession not in excess of $ a share under the public offering price. No underwriter may allow, and no dealer may re-allow, any concession to other underwriters or to certain dealers. After the initial offering of the shares of common stock, the offering price and other selling terms may from time to time be varied by the representatives.

We have granted to the underwriters an option, exercisable for 30 days from the date of this prospectus, to purchase up to an aggregate of additional shares of common stock at the public offering price, less underwriting discounts and commissions. The underwriters may exercise this option solely for the purpose of covering over-allotments, if any, made in connection with the offering of the shares of common stock offered by this prospectus. To the extent the option is exercised, each underwriter will become obligated, subject to certain conditions, to purchase approximately the same percentage of the additional shares of common stock as the number listed next to the underwriter s name in the preceding table bears to the total number of shares of common stock listed next to the names of all underwriters in the preceding table. If the underwriters over-allotment option is exercised in full, the total price to the public would be $, the total underwriters discounts and commissions would be $ and the total proceeds to us would be $.

The following table shows the per share and total underwriting discounts and commissions that we are to pay to the underwriters in connection with this offering. These amounts are shown assuming both no exercise and full exercise of the underwriters option.

	No Exercise	Full Exercise
Per share	$	$
Total	$	$

In addition, we estimate that the expenses of this offering payable by us, other than underwriting discounts and commissions, will be approximately $ million.

The underwriters have informed us that they do not intend sales to discretionary accounts to exceed 5% of the total number of shares of common stock offered by them.

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We, all of our directors and officers and holders of substantially all our outstanding stock have agreed that, without the prior written consent of Morgan Stanley & Co. Incorporated on behalf of the underwriters, we and they will not, during the period ending 180 days after the date of this prospectus:

- offer, pledge, sell, contract to sell, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase, lend, or otherwise transfer or dispose of, directly or indirectly, any shares of common stock or any securities convertible into or exercisable or exchangeable for common stock; or

- enter into any swap or other arrangement that transfers to another, in whole or in part, any of the economic consequences of ownership of the common stock.

whether any such transaction described above is to be settled by delivery of common stock or such other securities, in cash or otherwise.

The 180-day restricted period described in the preceding paragraph will be extended if:

- during the last 17 days of the 180-day restricted period we issue an earnings release or material news or a material event relating to our company occurs; or

- prior to the expiration of the 180-day restricted period, we announce that we will release earnings results during the 16-day period beginning on the last day of the 180-day period,

in which case the restrictions described in the preceding paragraph will continue to apply until the expiration of the 18-day period beginning on the issuance of the earnings release or the occurrence of the material news or material event.

These restrictions do not apply to:

- the sale of shares to the underwriters;

- the issuance by us of shares of common stock upon the exercise of an option or a warrant or the conversion of a security outstanding on the date of this prospectus of which the underwriters have been advised in writing;

- the grant of options or the issuance of shares of common stock by us pursuant to equity incentive plans described in this prospectus, provided that the recipient of the option or shares agree to be subject to the restrictions described in this paragraph;

- the issuance by us of shares of common stock in connection with any strategic transactions, such as collaboration or license agreements, provided that the recipient of the shares agrees to be subject to the restrictions described in this paragraph;

- transactions by any person other than us relating to shares of common stock or other securities acquired in open market transactions after the completion of the offering of the shares;

- transfers by any person other than us of shares of common stock or other securities as a bona fide gift or in connection with bona fide estate planning or by intestacy; or

- distributions by any person other than by us of shares of common stock or other securities to limited partners, members, stockholders or affiliates of such person;

provided that in the case of each of the last three transactions, no filing under Section 16(a) of the Exchange Act is required or is voluntarily made in connection with the transaction, and in the case of each of the last two transactions, each done or distribute agrees to be subject to the restrictions on transfer described above.

In order to facilitate this offering of common stock, the underwriters may engage in transactions that stabilize, maintain or otherwise affect the price of the common stock. Specifically, the underwriters may sell more shares than

they are obligated to purchase under the underwriting agreement, creating a short position. A short sale is covered if the short position is no greater than the number of shares available for

purchase by the underwriters under the over-allotment option. The underwriters can close out a covered short sale by exercising the over-allotment option or by purchasing shares in the open market. In determining the source of shares to close out a covered short sale, the underwriters will consider, among other things, the open market price of shares compared to the price available under the over-allotment option. The underwriters may also sell shares in excess of the over-allotment option, creating a naked short position. The underwriters must close out any naked short position by purchasing shares in the open market. A naked short position is more likely to be created if the underwriters are concerned that there may be downward pressure on the price of the common stock in the open market after pricing that could adversely affect investors who purchase in this offering. In addition, to stabilize the price of the common stock, the underwriters may bid for and purchase shares of common stock in the open market. Finally, the underwriters may reclaim selling concessions allowed to an underwriter or a dealer for distributing the common stock in the offering, if the syndicate repurchases previously distributed common stock to cover syndicate short positions or to stabilize the price of the common stock. Any of these activities may raise or maintain the market price of the common stock above independent market levels or prevent or retard a decline in the market price of the common stock. The underwriters are not required to engage in these activities and may end any of these activities at any time.

We have applied for quotation of our common stock approved for quotation on the Nasdaq National Market under the symbol AMTX.

We and the underwriters have agreed to indemnify each other against certain liabilities, including liabilities under the Securities Act.

Directed Share Program

At our request, the underwriters have reserved for sale, at the initial public offering price, up to shares offered by this prospectus to directors, officers, employees and other individuals associated with us through a directed share program. The number of shares of our common stock available for sale to the general public in the offering will be reduced to the extent these persons purchase these reserved shares. Any reserved shares not purchased by these persons will be offered by the underwriters to the general public on the same basis as the other shares offered by this prospectus. Recipients of reserved shares will be required to agree with the underwriters not to sell, transfer, assign, pledge or hypothecate these shares for a period of 180 days after purchasing the shares.

Pricing of the Offering

Prior to this offering, there has been no public market for the shares of common stock. The initial public offering price will be determined by negotiations between us and the representatives of the underwriters. Among the factors to be considered in determining the initial public offering price will be our future prospects and those of our industry in general; sales, earnings and other financial operating information in recent periods; and the price-earnings ratios, price-sales ratios and market prices of securities and certain financial and operating information of companies engaged in activities similar to ours. The estimated initial public offering price range set forth on the cover page of this preliminary prospectus is subject to change as a result of market conditions and other factors.

A prospectus in electronic format may be made available on the web sites maintained by one or more of the underwriters, and one or more of the underwriters may distribute prospectuses electronically. The underwriters may agree to allocate a number of shares to underwriters for sale to their online brokerage account holders. Internet distributions will be allocated by the underwriters that make Internet distributions on the same basis as other allocations.

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LEGAL MATTERS

The validity of the common stock we are offering will be passed upon by Bingham McCutchen LLP. Ropes & Gray LLP has acted as counsel for the underwriters in connection with certain legal matters related to this offering.

EXPERTS

Ernst & Young LLP, independent registered public accounting firm, has audited our financial statements at December 31, 2005 and 2004, and for each of the three years in the period ended December 31, 2005, as set forth in their report. We have included our financial statements in the prospectus and elsewhere in the registration statement in reliance on Ernst & Young LLP s report, given on their authority as experts in accounting and auditing.

WHERE YOU CAN FIND MORE INFORMATION

We have filed with the Securities and Exchange Commission a Registration Statement on Form S-1 under the Securities Act with respect to the shares of common stock we are offering to sell. This prospectus, which constitutes part of the Registration Statement, does not include all of the information contained in the Registration Statement and the exhibits, schedules and amendments to the Registration Statement. For further information with respect to us and our common stock, we refer you to the Registration Statement and to the exhibits and schedules to the Registration Statement. Statements contained in this prospectus about the contents of any contract or any other document are not necessarily complete, and, in each instance, we refer you to the copy of the contract or other documents filed as an exhibit to the Registration Statement. Each of these statements is qualified in all respects by this reference.

You may read and copy the Registration Statement of which this prospectus is a part at the Securities and Exchange Commission s public reference room, which is located at 100 F Street, N.E., Room 1580, Washington, D.C. 20549. You can request copies of the Registration Statement by writing to the Securities and Exchange Commission and paying a fee for the copying cost. Please call the Securities and Exchange Commission at 1-800-SEC-0330 for more information about the operation of the Securities and Exchange Commission s public reference room. In addition, the Securities and Exchange Commission maintains an Internet website, which is located at http://www.sec.gov, that contains reports, proxy and information statements and other information regarding issuers that file electronically with the Securities and Exchange Commission. You may access the Registration Statement of which this prospectus is a part at the Securities and Exchange Commission s Internet website. Upon closing of this offering, we will be subject to the information reporting requirements of the Securities Exchange Act of 1934, as amended, and we will file reports, proxy statements and other information with the Securities and Exchange Commission.

This prospectus includes statistical data that were obtained from industry publications. These industry publications generally indicate that the authors of these publications have obtained information from sources believed to be reliable but do not guarantee the accuracy and completeness of their information. While we believe these industry publications to be reliable, we have not independently verified their data.

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Amicus Therapeutics, Inc.
(a development stage company)
Financial Statements
March 31, 2006 (unaudited)
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Report of Independent Registered Public Accounting Firm

Board of Directors and Stockholders
Amicus Therapeutics, Inc.

We have audited the balance sheets of Amicus Therapeutics, Inc. (a development stage company) as of December 31, 2004 and 2005 and the related statements of operations, changes in stockholders deficiency and cash flows for each of the three years in the period ended December 31, 2005. These financial statements are the responsibility of the Company s management. Our responsibility is to express an opinion on these financial statements based on our audits.

We conducted our audits in accordance with the standards of the Public Company Accounting Oversight Board (United States). Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement. We were not engaged to perform an audit of the Company s internal control over financial reporting. Our audits included consideration of internal control over financial reporting as a basis for designing audit procedures that are appropriate in the circumstances, but not for the purpose of expressing an opinion on the effectiveness of the Company s internal control over financial reporting. Accordingly, we express no such opinion. An audit also includes examining, on a test basis, evidence supporting the amounts and disclosures in the financial statements, assessing the accounting principles used and significant estimates made by management, and evaluating the overall financial statement presentation. We believe that our audits provide a reasonable basis for our opinion.

In our opinion, the financial statements referred to above present fairly, in all material respects, the financial position of Amicus Therapeutics, Inc. as of December 31, 2004 and 2005 and the results of its operations and its cash flows for each of the three years in the period ended December 31, 2005, in conformity with U.S. generally accepted accounting principles.

/s/ Ernst & Young LLP

MetroPark, New Jersey
May 12, 2006

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Amicus Therapeutics, Inc.
(a development stage company)
Balance Sheets

	December 31,		March 31,	
	2004	2005	2006	Pro Forma (Note 1)
			(unaudited)	(unaudited)
Assets				
Current assets:				
Cash and cash equivalents	$ 257,036	$ 6,449,151	$ 10,298,878	$ 38,270,631
Investment in marketable securities	4,078,925	17,969,096	9,253,492	9,253,492
Prepaid expenses and other current assets	155,383	441,081	278,538	278,538
Total current assets	4,491,344	24,859,328	19,830,908	47,802,661
Property and equipment, net	541,277	3,278,887	3,696,596	3,696,596
Other non-current assets	40,537	531,739	467,338	467,338
	$ 5,073,158	$ 28,669,954	$ 23,994,842	$ 51,966,595
Liabilities, redeemable convertible preferred stock and stockholders equity (deficiency)				
Current liabilities:				
Accounts payable	589,919	906,226	532,866	532,866
Accrued expenses and other current liabilities	157,820	1,407,025	2,439,732	2,439,732
Current portion of capital lease obligations	174,545	279,265	852,527	852,527
Total current liabilities	922,284	2,592,516	3,825,125	3,825,125
Capital lease obligations, less current portion		734,370	1,993,960	1,993,960
Commitments and contingencies				
Series A redeemable convertible preferred stock, $.01 par value; 3,333,334 shares authorized, issued and outstanding at December 31, 2004, 2005 and March 31, 2006 (unaudited) (aggregate liquidation preference $2,500,000 at December 31, 2004, 2005, and March 31, 2006 (unaudited)), zero pro-forma shares outstanding (unaudited)	2,449,321	2,466,214	2,470,437	
Series B redeemable convertible preferred stock, $.01 par value; 37,025,594 shares authorized and 21,176,472, 36,470,591, and 36,470,591 shares issued and outstanding at December 31, 2004, 2005, and March 31, 2006 (unaudited), respectively (aggregate liquidation preference $18,000,000, $31,000,000, $31,000,000 at December 31, 2004, 2005, and	17,564,636	30,668,842	30,696,342	

March 31, 2006 (unaudited) respectively), zero pro-forma shares outstanding (unaudited)				
Series C redeemable convertible preferred stock, $.01 par value; 43,650,262 shares authorized and 21,825,131 shares issued and outstanding at December 31, 2005 and March 31, 2006 (unaudited) (aggregate liquidation preference $27,499,665 at December 31, 2005 and March 31, 2006 (unaudited)), zero pro-forma shares outstanding (unaudited)		27,333,758	27,342,646	
Stockholders equity (deficiency):				
Common stock, $.01 par value; 115,000,000 shares authorized and 2,306,541, 4,035,231, 4,635,231, and 66,264,287 shares issued and outstanding at December 31, 2004, 2005, March 31, 2006 (unaudited), and March 31, 2006 Pro Forma (unaudited), respectively	23,065	40,352	46,352	886,444
Additional paid-in capital	1,604,349	4,436,942	2,296,784	89,937,870
Accumulated other comprehensive loss	(9,083)	(16,139)	(5,320)	(5,320)
Deferred compensation	(133,174)	(2,546,846)		
Deficit accumulated during the development stage	(17,348,240)	(37,040,055)	(44,671,484)	(44,671,484)
Total stockholders equity (deficiency)	(15,863,083)	(35,125,746)	(42,333,668)	46,147,510
	$ 5,073,158	$ 28,669,954	$ 23,994,842	$ 51,966,595

See accompanying notes.

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

	Years Ended December 31,			Three Months Ended March 31,		Period from February 4, 2002 (inception) to March 31, 2006
	2003	2004	2005	2005	2006	
				(unaudited)	(unaudited) (Restated)	(unaudited) (Restated)
Revenue	$	$	$	$	$	$
Operating Expenses:						
Research and development	4,433,059	6,300,885	13,651,640	2,238,366	5,545,735	30,719,421
General and administrative	1,005,416	2,081,203	6,876,883	1,177,779	2,065,467	12,580,823
Impairment of leasehold improvements	1,029,696					1,029,696
Depreciation and amortization	131,931	145,961	302,832	46,712	199,224	804,088
In-process research and development						418,080
Total operating expenses	6,600,102	8,528,049	20,831,355	3,462,857	7,810,426	45,552,108
Loss from operations	(6,600,102)	(8,528,049)	(20,831,355)	(3,462,857)	(7,810,426)	(45,552,108)
Other income (expenses):						
Interest income	4,878	189,847	609,519	56,976	237,909	1,054,767
Interest expense	(172,472)	(550,004)	(81,776)	(4,069)	(58,912)	(868,955)
Loss before tax benefit	(6,767,696)	(8,888,206)	(20,303,612)	(3,409,950)	(7,631,429)	(45,366,296)
Income tax benefit		83,015	611,797			694,812
Net loss	(6,767,696)	(8,805,191)	(19,691,815)	(3,409,950)	(7,631,429)	(44,671,484)
Preferred stock accretion	(16,893)	(125,733)	(138,742)	(31,723)	(40,611)	(332,699)
Net loss attributable to common stockholders	$ (6,784,589)	$ (8,930,924)	$ (19,830,557)	$ (3,441,673)	$ (7,672,040)	$ (45,004,183)

Net loss attributable to common stockholders per common share basic and diluted	$ (2.94)	$ (3.87)	$ (6.45)	$ (1.49)	$ (1.81)
Weighted-average common shares outstanding basic and diluted	2,306,541	2,306,541	3,076,649	2,314,804	4,228,564
Unaudited pro forma net loss			$ (19,691,815)		$ (7,631,429)
Unaudited basic and diluted pro forma net loss per share			$ (0.23)		$ (0.09)
Unaudited basic and diluted pro forma weighted-average shares outstanding			87,085,839		88,237,754

See accompanying notes.

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Amicus Therapeutics, Inc.
(a development stage company)
Statements of Changes in Stockholders Deficiency
Period from February 4, 2002 (inception) to December 31, 2002,
the three year period ended December 31, 2005,
and the three months ended March 31, 2006 (unaudited)

	Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Loss	Deferred Compensation	Deficit Accumulated During the Development Stage	Total Stockholders Deficiency
	Shares	Amount					
Balance at February 4, 2002 (inception)		$	$	$	$	$	$
Issuance of common stock to a consultant	562,041	5,620	78,243				83,863
Stock issued for in-process research and development	1,742,000	17,420	400,660				418,080
Deferred compensation			208,866		(208,866)		
Amortization of deferred compensation					27,348		27,348
Issuance of warrants with financing arrangement			8,000				8,000
Accretion of series A redeemable convertible preferred stock			(10,720)				(10,720)
Net loss						(1,775,353)	(1,775,353)
Balance at December 31, 2002	2,304,041	23,040	685,049		(181,518)	(1,775,353)	(1,248,782)
Stock issued from exercise of options	2,500	25					25
Deferred compensation			14,138		(14,138)		
Amortization of deferred compensation					70,340		70,340
			210,000				210,000

Issuance of stock warrants with convertible notes						
Issuance of stock options to consultants			4,434			4,434
Accretion of series A redeemable convertible preferred stock			(16,893)			(16,893)
Beneficial conversion feature related to bridge financing			40,500			40,500
Net loss					(6,767,696)	(6,767,696)
Balance at December 31, 2003	2,306,541	23,065	937,228	(125,316)	(8,543,049)	(7,708,072)
Deferred compensation			67,700	(67,700)		
Amortization of deferred compensation				59,842		59,842
Issuance of stock options to consultants			16,118			16,118
Accretion of series A redeemable convertible preferred stock			(16,893)			(16,893)
Accretion of series B redeemable convertible preferred stock			(108,840)			(108,840)
Issuance of warrants with series B redeemable convertible preferred stock			421,802			421,802
Interest waived on converted convertible notes			192,734			192,734
Beneficial conversion feature related to bridge financing			94,500			94,500

Comprehensive loss:							
Unrealized holding loss on available-for-sale securities				(9,083)			(9,083)
Net loss						(8,805,191)	(8,805,191)
Net total comprehensive loss							(8,814,274)
Balance at December 31, 2004	2,306,541	$ 23,065	$ 1,604,349	$ (9,083)	$ (133,174)	$ (17,348,240)	$ (15,863,083)

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Amicus Therapeutics, Inc.
(a development stage company)
Statements of Changes in Stockholders Deficiency (Continued)
Period from February 4, 2002 (inception) to December 31, 2002,
the Three Year Period Ended December 31, 2005,
and the Three Months Ended March 31, 2006 (unaudited)

	Common Stock		Additional Paid-In	Accumulated Other Comprehensive		Deficit Accumulated During the Development	Total Stockholders
	Shares	Amount	Capital	Loss	Deferred Compensation	Stage	Deficiency
Balance at December 31, 2004 (carried forward)	2,306,541	$ 23,065	$ 1,604,349	$ (9,083)	$ (133,174)	$ (17,348,240)	$ (15,863,083)
Stocks issued from exercise of stock options	728,691	7,287	16,641				23,928
Stocks issued from exercise of warrants	999,999	10,000	65,000				75,000
Deferred compensation			2,778,223		(2,778,223)		
Amortization of deferred compensation					364,551		364,551
Non-cash charge for stock options to consultants			111,471				111,471
Accretion of series A redeemable convertible preferred stock			(16,893)				(16,893)
Accretion of series B redeemable convertible preferred stock			(109,999)				(109,999)
Accretion of series C redeemable convertible preferred stock			(11,850)				(11,850)
Comprehensive loss:							
				(7,056)			(7,056)

Unrealized holding loss on available-for-sale securities							
Net loss						(19,691,815)	(19,691,815)
Net total comprehensive loss							(19,698,871)
Balance at December 31, 2005	4,035,231	40,352	4,436,942	(16,139)	(2,546,846)	(37,040,055)	(35,125,746)
Stocks issued from exercise of options	600,000	6,000	45,000				51,000
Reversal of deferred compensation upon adoption of FAS 123(R)			(2,546,846)		2,546,846		
Stock-based compensation			315,671				331,791
Issuance of stock options to consultants			86,628				70,508
Accretion of series A redeemable convertible preferred stock			(4,223)				(4,223)
Accretion of series B redeemable convertible preferred stock			(27,500)				(27,500)
Accretion of series C redeemable convertible preferred stock			(8,888)				(8,888)
Comprehensive loss:							
Unrealized gain on available-for-sale securities				10,819			10,819
Net loss						(7,631,429)	(7,631,429)
Net total comprehensive loss							(7,620,610)

Balance at March 31, 2006 (unaudited)	4,635,231	$ 46,352	$ 2,296,784	$ (5,320)	$	$ (44,671,484)	$ (42,333,668)

See accompanying notes.

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Amicus Therapeutics, Inc.
(a development stage company)
Statements of Cash Flows

	Years Ended December 31,			Three Months Ended March 31,		Period from February 4, 2002 (inception) to March 31, 2006
	2003	2004	2005	2005	2006	
				(unaudited)	(unaudited)	(unaudited)
Operating activities						
Net loss	$ (6,767,696)	$ (8,805,191)	$ (19,691,815)	$ (3,409,950)	$ (7,631,429)	$ (44,671,484)
Adjustments to reconcile net loss to net cash used in operating activities:						
Non-cash interest expense	86,666	435,934				525,267
Depreciation and amortization	131,931	143,293	302,832	46,712	199,224	801,420
Amortization of non-cash compensation	70,340	59,842	364,551	91,138		522,081
Stock-based compensation					315,671	315,671
Non-cash charge for stock issued to consultants	4,434	16,118	111,471	27,868	86,628	302,514
Impairment of leasehold improvements	1,029,696					1,029,696
Non-cash charge for in process research and development						418,080
Beneficial conversion feature related to bridge financing	40,500	94,500				135,000
Changes in operating assets and liabilities:						
Prepaid expenses and other current assets	1,217	(147,664)	(285,698)	(17,637)	162,543	(278,538)
Other non-current assets		(19,936)	(491,202)	(28,813)	64,401	(488,505)
	1,526,439	(1,008,299)	1,565,512	422,700	659,347	2,972,598

Accounts payable and accrued expenses						
Net cash used in operating activities	(3,876,473)	(9,231,403)	(18,124,349)	(2,867,982)	(6,143,615)	(38,416,200)
Investing activities						
Sale and redemption of marketable securities		2,162,275	3,092,620		8,726,423	13,981,318
Purchases of marketable securities	(4,956)	(6,362,527)	(16,989,847)	(10,065,364)		(23,357,330)
Purchases of property and equipment	(1,088,009)	(227,317)	(3,040,442)	(71,230)	(616,933)	(5,527,712)
Net cash (used in) provided by investing activities	(1,092,965)	(4,427,569)	(16,937,669)	(10,136,594)	8,109,490	(14,903,724)
Financing activities						
Proceeds from issuance of preferred stock, net of issuance costs		12,877,598	40,316,115	13,000,000		55,598,528
Proceeds from issuance of convertible notes	3,800,000	1,200,000				5,000,000
Payments of capital lease obligations	(152,303)	(171,914)	(272,697)		(175,114)	(772,028)
Proceeds from exercise of stock options	25		23,928	832	51,000	74,953
Proceeds from exercise of warrants			75,000			75,000
Proceeds from capital asset financing arrangement			1,111,787	(50,748)	2,007,966	3,642,349
Net cash provided by financing activities	3,647,722	13,905,684	41,254,133	12,950,084	1,883,852	63,618,802
Net (decrease) increase in cash and cash equivalents	(1,321,716)	246,712	6,192,115	(54,492)	3,849,727	10,298,878
Cash and cash equivalents at beginning of year/ period	1,332,040	10,324	257,036	257,036	6,449,151	
Cash and cash equivalents at end of year/ period	$ 10,324	$ 257,036	$ 6,449,151	$ 202,544	$ 10,298,878	$ 10,298,878

Supplemental disclosures of cash flow information						
Cash paid during the period for interest	$ 45,306	$ 19,570	$ 481,577	$ 3,935	$ 34,453	$ 549,577
Non-cash activities						
Warrant issued with convertible notes						8,000
Warrant issued with Series B redeemable convertible preferred stock		1,802				49,950
Conversion of notes payable to Series B redeemable convertible preferred stock		5,000,000				5,000,000
Accretion of redeemable convertible preferred stock	$ 16,893	$ 125,733	$ 138,742	$ 31,723	$ 40,611	$ 332,699

See accompanying notes.

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Amicus Therapeutics, Inc.
(a development stage company)
Notes To Financial Statements

1. Description of Business

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. Accordingly, the Company is considered to be in the development stage.

The Company has an accumulated deficit of $44.7 million at March 31, 2006 and anticipates incurring losses through the year 2006. The Company has not yet generated revenues and has been able to fund its operating losses to date through the sale of its redeemable convertible preferred stock, issuance of convertible notes, and other financing arrangements. The Company s management intends to raise additional funds through the issuance of equity securities. If adequate funds are not available, the Company may have to substantially reduce or eliminate expenditures for the development of its products or cease operations.

In April 2006, the Company received cash amounting to approximately $27.5 million from the issuance of its second tranche series C redeemable convertible preferred stock. Management believes that the Company s current cash position and the additional funds received in April 2006 are sufficient to cover its cash flow requirements for 2006.

2. Summary of Significant Accounting Policies

Unaudited Interim Financial Statements

The financial statements as of March 31, 2006 and for the three months ended March 31, 2005 and 2006 have been prepared by the Company without an audit. All disclosures as of March 31, 2006 and for the three months ended March 31, 2005 and 2006, presented in the notes to the financial statements are unaudited. In the opinion of management, all adjustments (which include only normal recurring adjustments) considered necessary to present fairly the financial condition as of March 31, 2006 and results of operations and cash flows for the three months ended March 31, 2005 and 2006, have been made. The results of operations for the three months ended March 31, 2006 are not necessarily indicative of the results that may be expected for the full year ended December 31, 2006.

Unaudited Pro Forma Information

The unaudited pro forma balance sheet data as of March 31, 2006 gives effect to the Company s issuance on April 17, 2006 of 21,825,131 shares of series C redeemable convertible preferred stock, the automatic or voluntary exercise of warrants outstanding as of March 31, 2006 to purchase 555,003 shares of series B redeemable convertible preferred stock, and the automatic conversion of all outstanding shares of the Company s series A, B, and C redeemable convertible preferred stock into an aggregate of 84,009,190 shares of common stock upon completion of the Company s initial public offering.

Pro forma net loss per share is computed using the weighted-average number of common shares outstanding, including the pro forma effects of the items in the foregoing paragraph effective upon the assumed closing of the Company s proposed initial public offering, as if they had occurred at the beginning of the period.

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Amicus Therapeutics, Inc.
(a development stage company)
Notes To Financial Statements (Continued)

Use of Estimates

The preparation of financial statements in conformity with U.S. generally accepted accounting principles requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities, the disclosure of contingent assets and liabilities at the date of the financial statements, and the reported amounts of revenues and expenses during the reporting periods. Actual results could differ from those estimates.

Cash Equivalents

The Company considers all highly liquid investments purchased with a maturity of three months or less at the date of acquisition, to be cash equivalents.

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 Financial Accounting Standards Board (FASB) Statement of Financial Accounting Standards (SFAS) No. 115, *Accounting for Certain Investments in Debt and Equity Securities*, 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 as a separate component of stockholders 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 in any of the years presented herein.

Property and Equipment

Property and equipment are stated at cost, less accumulated depreciation and amortization. Depreciation is calculated over the estimated useful lives of the respective assets, which range from three to six years, or the lesser of the related initial term of the lease or useful life for leasehold improvements. Assets under capital leases are amortized over the terms of the related leases or their estimated useful lives, whichever is shorter.

The initial cost of property and equipment consists of its purchase price and any directly attributable costs of bringing the asset to its working condition and location for its intended use. Expenditures incurred after the fixed assets have been put into operation, such as repairs and maintenance, are charged to income in the period in which the costs are incurred. Major replacements, improvements and additions are capitalized in accordance with Company policy.

Deferred Offering Costs

Costs directly attributable to the Company s offering of its equity securities have been deferred and capitalized as part of other non-current assets. These costs will be charged against the proceeds of the offering once completed. The amount deferred as of March 31, 2006 was not significant.

Impairment of Long-Lived Assets

The Company performs a review of long-lived assets for impairment when events or changes in circumstances indicate the carrying value of such assets may not be recoverable. If an indication of impairment is present, the Company compares the estimated undiscounted future cash flows to be

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generated by the asset to its carrying amount. If the undiscounted future cash flows are less than the carrying amount of the asset, the Company records an impairment loss equal to the excess of the asset s carrying amount over its fair value. The fair value is determined based on valuation techniques such as a comparison to fair values of similar assets or using a discounted cash flow analysis. The Company reported an impairment charge of $1,029,696 during 2003 related to impaired capitalized leasehold improvements. There were no other impairment charges recognized during the years ended December 31, 2004 and 2005, or the three months ended March 31, 2005 and 2006.

Redeemable Convertible Preferred Stock

The carrying value of redeemable convertible preferred stock is increased by periodic accretions so that the carrying amount will equal the redemption amount at the earliest redemption date. These increases are reflected through charges to additional paid-in capital since the Company does not have retained earnings.

Research and Development Costs

Research and development costs are expensed as incurred. Research and development expense consists primarily of costs related to personnel, including salaries and other personnel-related expenses, consulting fees and the cost of facilities and support services used in drug development. Assets acquired that are used for research and development and have no future alternative use are expensed as in-process research and development.

Concentration of Credit Risk

The Company s financial instruments that are exposed to concentration of credit risk consist primarily of cash and cash equivalents and marketable securities. The Company maintains its cash and cash equivalents in bank accounts, which, at times, exceed federally insured limits. The Company invests its marketable securities in high-quality commercial financial instruments. The Company has not recognized any losses from credit risks on such accounts during any of the periods presented. The Company believes it is not exposed to significant credit risk on cash and cash equivalents or its marketable securities.

Fair Value of Financial Instruments

SFAS No. 107, *Disclosures about Fair Value of Financial Instruments*, requires disclosures of fair value information about financial instruments, whether or not recognized in the balance sheet, for which it is practicable to estimate that value. Due to the short-term nature, the carrying amounts reported in the financial statements approximate the fair value for cash and cash equivalents, accounts payable and accrued expenses. The estimated fair value of the Company s redeemable convertible preferred stock at March 31, 2006 is approximately $77.7 million based on the August 2005 series C redeemable convertible preferred stock price of $1.26 per share. The redeemable convertible preferred stock will be converted into common stock of the Company upon consummation of a qualified initial public offering.

Income Taxes

The Company accounts for income taxes under the liability method. Under this method deferred income tax liabilities and assets are determined based on the difference between the financial statement carrying amounts and tax basis of assets and liabilities and for operating losses and tax credit carryforwards, using enacted tax rates in effect in the years in which the differences are expected to reverse. A valuation allowance is recorded if it is more likely than not that a portion or all of a deferred tax asset will not be realized.

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Comprehensive Loss

SFAS No. 130, *Reporting Comprehensive Income*, requires components of other comprehensive loss, including unrealized gains and losses on available-for-sale securities, to be included as part of total comprehensive loss. The components of comprehensive loss are included in the statements of changes in stockholders deficiency.

Stock-Based Compensation

At December 31, 2005 and March 31, 2006, the Company has one stock-based employee compensation plan, which is described more fully in Note 7. Prior to December 31, 2005, the Company accounted for those plans under the recognition and measurement provisions of Accounting Principles Board Opinion (APB) No. 25, *Accounting for Stock Issued to Employees*, and related Interpretations, as permitted by FASB Statement No. 123 (SFAS No. 123), *Accounting for Stock-Based Compensation*. Stock-based employee compensation cost was recognized in the Statements of Operations for the years ended December 31, 2003, 2004, and 2005 and for the three month period ended March 31, 2005 to the extent the options granted under the plan had an exercise price that was less than the market value of the underlying common stock on the date of grant. Effective January 1, 2006, the company adopted the fair value recognition provisions of FASB Statement No. 123(R), *Share-Based Payment* (SFAS No. 123(R)), using the prospective transition method. Under that transition method, compensation cost is recognized for all share-based payments granted subsequent to January 1, 2006, based on the grant-date fair value estimated in accordance with the provisions of SFAS No. 123(R). Results for prior periods have not been restated. As a result of adopting SFAS No. 123(R) on January 1, 2006, the Company s net loss is larger than had it continued to account for share-based compensation under Opinion 25.

Prior to the adoption of SFAS No. 123(R), the Company presented its unamortized portion of deferred compensation cost for non-vested stock options in the statement of changes in stockholders deficiency with a corresponding credit to additional paid in capital. Upon the adoption of SFAS No. 123(R), these amounts were offset against each other. Under SFAS No. 123(R), an equity instrument is not considered to be issued until the instrument vests. As a result, compensation cost is recognized over the requisite service period with an offsetting credit to additional paid in capital, and the deferred compensation balance of $2.5 million at January 1, 2006 was net against additional paid in capital during the first quarter of 2006.

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The following table illustrates the effect on net loss and net loss per share if the company had applied the minimum value recognition provisions of SFAS No. 123 to options granted under the company s stock option plans in all periods presented prior to adoption of SFAS No. 123(R). For purposes of this pro forma disclosure, the value of the options is estimated using a minimum value option-pricing formula and amortized to expense over the options vesting periods.

	Years Ended December 31,			Three Months Ended March 31,
	2003	2004	2005	2005
Numerator				
Net loss attributable to common stockholders, as reported	$ (6,784,589)	$ (8,930,924)	$ (19,830,557)	$ (3,441,673)
Add: Non-cash employee compensation	70,340	59,842	364,551	91,138
Less: Total stock-based employee compensation expense determined under the minimum value method for all awards	(76,207)	(74,499)	(437,296)	(109,324)
Pro forma net loss attributable to common stockholders	$ (6,790,456)	$ (8,945,581)	$ (19,903,302)	$ (3,459,859)
Net loss attributable to common stockholders per common share:				
Basic and fully diluted:				
As reported	$ (2.94)	$ (3.87)	$ (6.45)	$ (1.49)
Pro forma	$ (2.94)	$ (3.88)	$ (6.47)	$ (1.49)

Pro forma information regarding net loss is required by SFAS No. 123 and has been determined as if the Company has been accounting for its stock options awards under the minimum value option pricing method as of that statement. The value of these options was estimated at the date of grant using a minimum value method with the following weighted-average assumptions:

Employee Stock Options

	Years Ended December 31,			Three Months Ended March 31,
	2003	2004	2005	2005
Expected term (in years)	6.5	6.5	6.0	6.0
Risk-free interest rate	4.26%	3.92%	4.15%	4.15%
Dividend yield	0.00%	0.00%	0.00%	0.00%

Upon adoption of SFAS No. 123(R), the Company selected the Black-Scholes option pricing model as the most appropriate model for determining the estimated fair value for stock-based awards. The fair value is then amortized on a straight-line basis over the requisite service periods of the awards, which is generally the vesting period. Use of a valuation model requires management to make certain assumptions with respect to selected model inputs. Expected volatility was calculated based on a blended weighted average of historical information of the Company s stock and the weighted average of historical information of similar public entities for which historical information was available. The Company will continue to use a weighted average approach using its own historical volatility and other similar public entity volatility information until historical volatility of the Company is relevant to measure expected

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volatility for future option grants. The average expected life was determined according to the SEC shortcut approach as described in Staff Accounting Bulletin (SAB) No. 107, *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. Forfeitures are estimated based on voluntary termination behavior, as well as a historical analysis of actual option forfeitures. The weighted-average assumptions used in the Black-Scholes option pricing model are as follows:

		Three Months Ended March 31, 2006
Expected stock price volatility		72.70%
Risk free interest rate		4.59%
Expected life of options (years)		6.25
Expected annual dividend per share	$	0.00

The weighted-average grant date fair value for options granted during the three months ended March 31, 2006 was approximately $1.52 per share.

During the three months ended March 31, 2006, the Company recorded compensation expense of approximately $152,000 ($0.04 per basic and diluted share) related to the expensing of the Company s options under SFAS No. 123(R) during the quarter. The compensation expense had no impact on the Company s cash flows from operations and financing activities. The total compensation cost related to non-vested stock option awards not yet recognized as of March 31, 2006 was approximately $7.1 million. This expense will be recorded on a straight basis over approximately 4 years.

Beneficial Conversion Feature

When the Company issues debt or equity which is convertible into common stock at a discount from the common stock fair value at the date the debt or equity is issued, a beneficial conversion feature for the difference between the closing price and the conversion price multiplied by the number of shares issuable upon conversion is recognized. The beneficial conversion feature is presented as a discount to the related debt or a deemed dividend to the related equity, with an offsetting amount increasing additional paid in capital. The Company recorded a beneficial conversion charge for its bridge loan financing of $135,000 which was initially recorded as debt discount and amortized to interest expense through May 2004. The Company expects to record a beneficial conversion charge (deemed dividend) during the second quarter of 2006 of approximately $19.4 million related to the issuance of certain shares of series C redeemable convertible preferred stock. The estimated fair value of the common stock was approximately $2.15 per share at the measurement date based on estimates of the projected initial public offering price of the Company s common stock which is planned in mid-2006.

The accompanying financial statements for the quarter ended March 31, 2006 were restated as the Company determined that its deemed dividend related to the Series C redeemable convertible preferred stock should have been recorded as a second quarter event instead of a first quarter event. The deemed dividend was initially recorded in the financial statements as of the measurement date instead of the date the shares were issued, which occurred in April 2006. The change had no impact on net loss for the three months ended March 31, 2006 and decreased net loss attributable to common shareholders for the three months ended March 31, 2006 by $19.4 million; resulting in an increase to basic and diluted earnings per share attributable to common stockholders of $4.60, from ($6.41) to ($1.81). This change was confined to the first quarter of 2006 only and had no impact on the related balance sheet accounts and statement of

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cash flows. The statement of changes in stockholders deficiency were revised to remove the beneficial conversion charges, however, the net impact was zero.

The effects of the restatement on the financial statement items were as follows:

Statement of Operations	Previously Reported Three Months Ended March 31, 2006	As Restated Three Months Ended March 31, 2006
Net loss attributable to common stockholders	$ (27,096,407)	$ (7,672,040)
Net loss attributable to common stockholders per common share basic and diluted	$ (6.41)	$ (1.81)

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, and C redeemable convertible preferred stock represent participating securities in accordance with Emerging Issue Task Force (EITF) 03-6 *Participating Securities and the Two Class Method under FASB Statement No. 128*. However, since 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 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.

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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:

	Years Ended December 31,			Three Months Ended March 31,	
	2003	2004	2005	2005	2006
				(unaudited)	(unaudited) (Restated)
Historical					
Numerator:					
Net loss	$ (6,767,696)	$ (8,805,191)	$ (19,691,815)	$ (3,409,950)	$ (7,631,429)
Accretion of series B redeemable convertible preferred stock	(16,893)	(125,733)	(138,742)	(31,723)	(40,611)
Net loss attributable to common stockholders	$ (6,784,589)	$ (8,930,924)	$ (19,830,557)	$ (3,441,673)	$ (7,672,040)
Denominator:					
Weighted average common shares outstanding basic and diluted	2,306,541	2,306,541	3,076,649	2,314,804	4,228,564
Unaudited Pro forma					
Numerator:					
Net loss			$ (19,691,815)		$ (7,631,429)
Denominator:					
Pro forma weighted average common shares outstanding basic and diluted			87,085,839		88,237,754

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 approximately 5,496,110, 28,749,798 and 70,948,031 for the years ended December 31, 2003, 2004 and 2005, respectively and 47,059,954 and 76,243,031 for the three months ended March 31, 2005 and 2006, respectively. Potentially dilutive common stock equivalents were excluded from the diluted earnings per share denominator for all periods because of their anti-dilutive effect.

Recent Accounting Pronouncements

In February 2006, FASB issued SFAS No. 155, *Accounting for Certain Hybrid Instruments*. SFAS 155 allows financial instruments that have embedded derivatives to be accounted for as a whole (eliminating the need to bifurcate the derivative from its host) if the holder elects to account for the whole instrument on a fair value basis. This

statement is effective for all financial instruments acquired or issued after the beginning of an entity s first fiscal year that begins after September 15, 2006. The Company believes the adoption of SFAS No. 155 will not have a material impact on its financial statements.

In May 2005, FASB issued SFAS No. 154, *Accounting Changes and Error Corrections* (SFAS No. 154), a replacement of APB No. 20, Accounting Changes and SFAS No. 3, *Reporting Accounting Changes in Interim Financial Statements*. SFAS No. 154 applies to all voluntary changes in accounting principle and changes the requirements for accounting for and reporting of a change in accounting

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principle. This statement establishes that, unless impracticable, retrospective application is the required method for reporting a change in accounting principle in the absence of explicit transition requirements specific to the newly adopted accounting principle. It also requires the reporting of an error correction which involves adjustments to previously issued financial statements similar to those generally applicable to reporting an accounting change retrospectively. SFAS No. 154 is effective for accounting changes and corrections of errors made in fiscal years beginning after December 15, 2005.

Segment Information

The Company currently operates in one business segment focusing on the development and commercialization of small molecule, orally administered therapies to treat a range of human genetic diseases. The Company is not organized by market and is managed and operated as one business. A single management team reports to the chief operating decision maker who comprehensively manages the entire business. The Company does not operate any separate lines of business or separate business entities with respect to its products. Accordingly, the Company does not accumulate discrete financial information with respect to separate service lines and does not have separately reportable segments as defined by SFAS No. 131, *Disclosure About Segments of an Enterprise and Related Information.*

3. Investments in Marketable Securities

The following is a summary of available for sale securities held by the Company:

	Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
March 31, 2006 (unaudited)				
Corporate Bonds	$ 9,258,812	$	$ (5,320)	$ 9,253,492
December 31, 2005				
Corporate Bonds	$ 17,985,235	$	$ (16,139)	$ 17,969,096
December 31, 2004				
Corporate Bonds	$ 4,088,008	$	$ (9,083)	$ 4,078,925

The Company s available for sale investments have the following maturities at:

	December 31, 2004	December 31, 2005	March 31, 2006 (unaudited)
Due in one year or less	$4,078,925	$17,969,096	$ 9,253,492

Unrealized gains and losses are reported as a component of accumulated other comprehensive loss in stockholders deficiency. For the years ended December 31, 2003, 2004, 2005, and for the three months ended March 31, 2005 and 2006, realized losses were $0, $704, $1,228, $1,228, and $0, respectively. The cost of securities sold is based on specific identification method.

Unrealized loss positions for which other than temporary impairments have not been recognized at December 31, 2004, 2005, and March 31, 2006 are summarized as follows:

	December 31,		March 31,
	2004	2005	2006
			(unaudited)
Less than 12 months	$(9,083)	$(16,139)	$ (5,320)

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Unrealized losses in the Company s portfolio relate to fixed income debt securities. For these securities, the unrealized losses are due to increases in interest rates and not changes in credit risk. The Company has concluded that the unrealized losses in its marketable securities are not other-than-temporary as the Company has the ability to hold the securities to maturity or a planned forecasted recovery.

4. Property and Equipment

Property and equipment consist of the following:

	December 31,		March 31,
	2004	2005	2006
			(unaudited)
Computer equipment	$ 105,637	$ 284,913	$ 307,609
Computer software		15,921	80,143
Research equipment	737,672	1,790,873	1,929,599
Furniture and fixtures		251,703	291,514
Leasehold improvements		109,345	1,891,819
Construction in progress		1,430,996	
	843,309	3,883,751	4,500,684
Less accumulated depreciation and amortization	(302,032)	(604,864)	(804,088)
	$ 541,277	$ 3,278,887	$ 3,696,596

In 2003, the Company capitalized costs related to an additional facility that it had leased in Cranbury, New Jersey. However, because the Company was not able to raise the necessary capital it required to continue the construction of the leasehold improvements in a timely manner, it decided to cease activities related to the construction. As a result, the Company expensed all capitalized leasehold improvements amounting to $1,029,696 in 2003.

Included in property and equipment is costs capitalized pursuant to a capital lease obligation of $0, $1,146,007, and $3,247,858 at December 31, 2004, 2005, and March 31, 2006. Depreciation and amortization expense was $0, $0 and $137,504 for the years ended December 31, 2003, 2004, and 2005, respectively; and $0 and $152,097 for the three months ended March 31, 2005 and 2006, respectively.

5. Accrued Expenses

Accrued expenses consist of the following:

	December 31,		March 31,
	2004	2005	2006
			(unaudited)
Accrued construction costs	$	$ 592,594	$
Accrued professional fees	85,930	312,244	57,593
Accrued contract manufacturing & contract research costs		53,163	1,796,713
Accrued compensation and benefits		14,719	230,185

Accrued facility costs		182,303	196,600
Accrued other	71,890	252,002	158,641
	$ 157,820	$ 1,407,025	$ 2,439,732

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6. Capital Structure

Redeemable Convertible Preferred Stock

At March 31, 2006 the Company is authorized to issue 3,333,334 shares of series A redeemable convertible preferred stock (Series A), 37,025,594 shares of series B redeemable convertible preferred stock (Series B) and 43,650,262 shares of series C redeemable convertible preferred stock (Series C). At December 31, 2005 and March 31, 2006, the Company had outstanding 3,333,334 shares, 36,470,591 shares, and 21,825,131 shares of Series A, B, and C, respectively.

Voting

Series A, Series B and Series C stockholders are entitled to vote on substantially all matters based on the number of votes equal to the number of shares of common stock into which each share of preferred stock is convertible.

Dividends

Dividends are payable when, as and if declared by the board of directors and are non-cumulative. Series A, Series B and Series C stockholders shall be entitled to receive dividends at the same rate as dividends paid with respect to the common stock. Such preferred dividends will be determined by the number of shares of common stock into which each share of redeemable convertible preferred stock is convertible.

Conversion

Series A, Series B and Series C stockholders are entitled, at any time, to cause their shares to be converted into fully-paid and non-assessable shares of common stock on a one-for-one basis. However, if there is a stock dividend, stock split or a capital reorganization of the common stock before conversion of preferred stock, the conversion factor will be adjusted in accordance with the Company s amended and restated certificate of incorporation. Additionally, the Series A, Series B and Series C will convert automatically immediately upon the closing of a firmly underwritten public offering pursuant to an effective registration statement under the Securities Act of 1933, as amended, covering the offer and sale of common stock for the account of the Company, which results in aggregate net proceeds to the Company of at least $40,000,000 and a per share price of at least $2.52 and the common stock is listed on a U.S. national securities exchange or admitted for quotation on the NASDAQ National Market.

Liquidation

In the event of any liquidation, dissolution or winding up of the Company (including a merger or sale of all or substantially all of the assets of the Company), either voluntary or involuntary, the Series A, Series B and Series C holders are entitled to receive, in preference to common stock, an amount equal to $0.75 per share, $0.85 per share and $1.26 per share, respectively, adjusted for any combinations, splits, and other recapitalizations plus all declared but unpaid dividends. For any remaining assets, the Series A, Series B and Series C shareholders shall participate with the holders of common stock on an as-converted basis.

Redemption Rights

The holders of the redeemable convertible preferred stock are entitled to require the Company to redeem all shares of the redeemable convertible preferred stock at any time after the fifth anniversary of the Series C original issue date. The redeemable convertible preferred stock may be redeemed at an

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amount equal to the liquidation preference upon receipt by the Company of a request from the holders of at least 60% of the then outstanding shares of Series C that the redeemable convertible preferred stock be redeemed.

As of December 31, 2004, 2005, and March 31, 2006, Series A, Series B and Series C are recorded at its stated values (estimated fair value of $0.75 per share, $0.85 per share and $1.26, respectively, less issuance costs, plus accrued but unpaid dividends, if any, and accretion adjustments).

	Series A		Series B		Series C	
	Shares	Amount	Shares	Amount	Shares	Amount
Balance at February 4, 2002 (inception)		$		$		$
Issuance of Series A at $0.75 per share	3,333,334	2,500,000				
Issuance costs		(95,185)				
Accretion to redemption value		10,720				
Balance at December 31, 2002	3,333,334	2,415,535				
Accretion to redemption value		16,893				
Balance at December 31, 2003	3,333,334	2,432,428				
Issuance of Series B at $0.85 per share			21,176,472	18,000,000		
Issuance cost				(122,402)		
Issuance of warrants with Series B				(421,802)		
Accretion to redemption value		16,893		108,840		
Balance at December 31, 2004	3,333,334	2,449,321	21,176,472	17,564,636		
Issuance of Series B at $0.85 per share			15,294,119	13,000,000		
Issuance cost				(5,793)		
Issuance of Series C at $1.26 per share					21,825,131	$ 27,499,665
Issuance cost						(177,757)
Accretion to redemption value		16,893		109,999		11,850
Balance at December 31, 2005	3,333,334	2,466,214	36,470,591	30,668,842	21,825,131	27,333,758

Accretion to redemption value		4,223		27,500		8,888
Balance at March 31, 2006 (unaudited)	3,333,334	$ 2,470,437	36,470,591	$ 30,696,342	21,825,131	$ 27,342,646

On April 15, 2002, the Company issued 666,668 shares of Series A. On July 15, 2002, the Company issued 2,666,666 shares of Series A. Subsequently, on May 3, 2004, the Company amended its certificate of incorporation to create the Series B and to set forth the rights and preferences of such stock. On May 4, 2004, the Company issued 21,176,472 shares of its Series B at $0.85 per share and 555,003 warrants with a gross proceeds (exclusive of proceeds from the potential future exercise of the warrants)

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amounting to approximately $18 million. The values of the warrants were classified as a cost of issuance and are being accreted through the earliest redemption date. In April of 2005, the Company issued 15,294,119 shares of its Series B and in August of 2005, 21,825,131 shares of Series C.

Bridge Loans for Series B redeemable convertible preferred stock

During 2003 and 2004, prior to the closing of the issuance of the Series B, the Company issued a series of notes and warrants in connection with short-term loans (Bridge Loans) to help fund the Company s operations prior to the closing of the Series B shares. The principal owed on all of these notes issued in 2003 and in the first quarter 2004 totaled $5.5 million. $5.0 million of principal outstanding under the Bridge Loans was converted into 5,882,353 Series B shares and $500,000 of principal outstanding under the Bridge Loans was repaid, in each case in May 2004 at the closing of the Series B financing. Approximately $193,000 in interest payable at such closing was waived by the holders. The interest was recorded and charged to expense and credited to additional paid-in capital during 2004. The interest owed on such notes was waived by the holders thereof and recorded as additional paid-in capital.

In addition, the Company issued warrants for 999,999 shares of common stock in connection with some of the Bridge Loans. These warrants were valued using a Black-Scholes option pricing model, amortized over the term of the notes, and charged to interest expense. The total interest charge related to these warrants was $210,000. In addition, the Company recognized a beneficial conversion charge of $135,000 related to the conversion feature in the Bridge Loans.

Common Stock and Stock Options

As of March 31, 2006 the Company was authorized to issue 115,000,000 shares of common stock.

Dividends on common stock will be paid when, and if declared by the board of directors. Each holder of common stock is entitled to vote on all matters and is entitled to one vote for each share held. The Company will, at all times, reserve and keep available out of its authorized but unissued shares of common stock sufficient shares to affect the conversion of the shares of the redeemable convertible preferred stock and the exercise of outstanding warrants and stock options.

Restricted Common Stock Issuances

In connection with the formation of the Company, the Company issued 1,742,000 shares of common stock to the Mount Sinai School of Medicine of New York University in exchange for exclusive license rights for certain intellectual property. The value of the shares was accounted for as in-process research and development (see Note 11).

In connection with a 2002 consulting arrangement, the Company issued 562,041 shares of common stock in return for services. The shares are fully vested and the Company recorded $83,863 as compensation expense in the financial statements during the year ended 2002.

Warrants

During 2002, the Company issued 40,000 common stock warrants to a vendor as part of a capital lease agreement. These warrants were outstanding at December 31, 2003, 2004 and 2005. The warrants have an exercise price of $0.75 per share (adjusted for stock splits, stock dividends, etc.). The value of the warrants was calculated using the Black-Scholes option pricing model and was capitalized as debt issuance cost and amortized to interest expense over the term of the obligation. The value of the warrants and total charge to interest expense was not material for each of the years presented.

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In 2003, the Company issued 999,999 common stock warrants to certain investors in connection with its Bridge Loans. The warrants had an exercise price of $0.075 per share (adjusted for stock splits, stock dividends, etc.). The value of the warrants of $210,000 was calculated using the Black-Scholes option pricing model and was accounted for as debt discount and amortized to interest expense over the term of the loans. These same warrant shares were exercised in 2005. The total charge to interest expense was $84,000 and $126,000 for the years ended December 31, 2003 and 2004, respectively.

In 2004, the Company issued warrants to purchase 555,003 Series B shares to certain investors as part of the Series B financing. These warrants were outstanding at March 31, 2006. The warrants have an exercise price of $0.85 per share (adjusted for stock splits, stock dividends, etc.). The value of the warrants of $422,000 was calculated using the Black-Scholes option pricing model and was capitalized as issuance cost and is being accreted through the earliest redemption date. The total accretion amount related to the warrants was $84,360, for each of the years ended December 31, 2004, 2005, and $21,090 for the three months ended March 31, 2006, respectively.

7. Stock Option Plan

In April 2002, the Company s board of directors and shareholders approved the Company s 2002 Stock Option Plan (the 2002 Plan). The 2002 Plan provides for the granting of options to purchase common stock in the Company to employees, advisors and consultants at a price to be determined by the Company s board of directors. The 2002 Plan is intended to encourage ownership of stock by employees and consultants of the Company and to provide additional incentives for them to promote the success of the Company s business. The Options may be incentive stock options (ISO s) or non-statutory stock options (NSO s). Under the provisions of the 2002 Plan, no option will have a term in excess of 10 years.

The board of directors, or its committee, is responsible for determining the individuals to be granted options, the number of options each individual will receive, the option price per share, and the exercise period of each option. Options granted pursuant to the 2002 Plan generally vest over a four year term.

As of March 31, 2006, the Company reserved up to 17,500,000 shares for issuance under the 2002 Plan.

In establishing its estimates of fair value of its common stock, the Company considered the guidance set forth in the AICPA Practice Aid, *Valuation of Privately-Held-Company Equity Securities Issued as Compensation*, and performed a retrospective determination of the fair value of its common stock utilizing a combination of valuation methods.

In December 2004, the FASB issued SFAS No. 123(R), which requires compensation costs related to share-based transactions, including employee share options, to be recognized in the financial statements based on fair value. SFAS No. 123(R) revises SFAS No. 123, as amended, *Accounting for Stock-Based Compensation,* and supersedes APB Opinion No. 25, *Accounting for Stock Issued to Employees*.

On January 1, 2006, the Company adopted SFAS No. 123(R) using the prospective method. Under SFAS No. 123(R), the Company has elected to recognize the compensation cost of all share-based awards on a straight-line basis over the vesting period of the award. Benefits of tax deductions in excess of recognized compensation expense will be reported as a financing cash flow, rather than an operating cash flow as prescribed under the prior accounting rules.

Prior to January 1, 2006, the Company applied APB No. 25 to account for its stock-based compensation plans. Under APB No. 25 the Company used the minimum value method of calculating unrecognized compensation expense for disclosure purposes in the Financial Statements.

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Notes To Financial Statements (Continued)

Stock Options

The following table summarizes information about stock options outstanding:

		Options Outstanding			
	Shares Available For Grant	Number of Shares	Option Price Per Share Range	Weighted-Average Exercise Price	Weighted-Average Grant Date Fair Value or Calculated Value
Balance at February 4, 2002 (inception)			$	$	$
Shares authorized	1,212,611				
Options granted	(961,111)	961,111	0.01 0.08	0.02	0.23
Balance at December 31, 2002	251,500	961,111	0.01 0.08	0.02	
Shares authorized	100,000				
Options exercised		(2,500)	0.01	0.01	
Options granted	(164,166)	164,166	0.08	0.08	0.23
Balance at December 31, 2003	187,334	1,122,777	0.01 0.08	0.02	
Shares authorized	6,401,492				
Options granted	(2,083,882)	2,083,882	0.08 0.09	0.08	0.12
Options forfeited	6,666	(6,666)	0.08	0.08	
Balance at December 31, 2004	4,511,610	3,199,993	0.01 0.09	0.06	
Shares authorized	4,125,000				
Options granted	(7,576,785)	7,576,785	0.09 0.71	0.29	0.72
Options exercised		(728,691)	0.01 0.09	0.03	
Options forfeited	769,112	(769,112)	0.01 0.09	0.06	
Balance at December 31, 2005	1,828,937	9,278,975	0.01 0.09	0.28	
Shares authorized	5,660,897				
Options granted		5,895,000	0.71	0.71	1.84
Options exercised		(600,000)	0.09	0.09	
Balance at March 31, 2006 (unaudited)	7,489,834	14,573,975	$ 0.01 $0.71	$ 0.43	
Exercisable at December 31, 2003		116,731			

Exercisable at December 31, 2004	221,315
Exercisable at December 31, 2005	930,397
Vested and Exercisable at March 31, 2006	1,213,538

As of March 31, 2006, there was approximately $9.8 million of total unrecognized compensation cost related to non-vested share-based compensation arrangements granted under the Plan. Of this amount approximately $2.7 million is related to stock option grants issued prior to January 1, 2006 and approximately $7.1 million is related to 2006 grants. That cost is expected to be recognized over a weighted-average period of 3.5 years and 3.92 years for 2005 and 2006, respectively.

As of March 31, 2006, outstanding options had aggregate intrinsic value amounting to $8.6 million. Exercisable options had aggregate intrinsic value amounting to $121,370. The Company estimates that 13,845,276 of the options outstanding at March 31, 2006 will vest over the remaining vesting periods for such options.

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Notes To Financial Statements (Continued)

Following is a summary of the status of stock options outstanding at March 31, 2006:

	Outstanding Options			Exercisable Options	
Exercise Price per Range	Number	Weighted Average Remaining Contractual Life	Weighted Average Exercise Price	Number	Weighted Average Exercise Price
$0.01	160,000	6.44	$ 0.01	132,292	$ 0.01
$0.075 - $0.085	6,012,475	8.85	0.08	1,081,246	0.08
$0.71	8,401,500	9.81	$ 0.71		$ 0.71
	14,573,975			1,213,538	

As of December 31, 2005 and March 31, 2006, the average remaining contractual life of outstanding options was approximately 9.0 and 9.4 years, respectively. The total intrinsic value of options exercised during the years ended December 31, 2003, 2004, and 2005, was $575, $0, and $140,235, respectively, and $1,832 and $135,000 for the three months ended March 31, 2005 and 2006. For the three months ended March 31, 2006, the fair value of the options granted, based upon the Black-Scholes calculation ranged from $1.14 to $1.52 per share.

Options may be exercised in whole or in part for 100% of the shares subject to vesting at any time after the date of grant. Options generally vest 25% on the first year anniversary date of grant plus an additional 1/48 for each month thereafter.

The Company performed a retrospective determination of the fair value of the Company s common stock and granted stock options with exercise prices as follows:

2005 Grant Date	Number of Options Granted	Weighted Average Exercise Price	Retrospective Determination of Fair Value of Underlying Stock	Intrinsic Value
January - May	3,037,037	$ 0.09	$ 0.31	$ 0.22
June - July	1,768,748	0.09	0.77	0.68
August - September	315,500	0.22	0.95	0.73
October - November	2,351,000	0.71	1.14	0.43
December	104,500	$ 0.71	$ 1.44	$ 0.73
	7,576,785			

The intrinsic value of options granted prior to 2005 is not significant.

The Company recorded approximately $2.8 million in gross deferred compensation expense and recognized compensation expense of approximately $364,551 during the year ended December 31, 2005 in connection with these stock grants which is net of approximately $47,000 related to employee terminations during the year ended December 31, 2005.

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During the three months ended March 31, 2006 the Company granted stock options with exercise prices as follows:

Grant Date	Number of Options Granted	Exercise Price	Fair Value of Underlying Stock	Intrinsic Value
January 2, 2006	17,000	$ 0.71	$ 1.44	$ 0.73
January 12, 2006	5,000	0.71	1.44	0.73
February 6, 2006	5,000	0.71	1.44	0.73
February 9, 2006	23,000	0.71	1.44	0.73
February 13, 2006	7,500	0.71	1.44	0.73
February 22, 2006	35,000	0.71	1.44	0.73
February 28, 2006	5,752,500	0.71	1.84	1.13
March 27, 2006	50,000	$ 0.71	$ 1.84	$ 1.13
	5,895,000			

Compensation expense of $70,340, $59,842, $364,551, and $91,138 and $315,671 was recognized for the years ended 2003, 2004 and 2005 and for the three months ended March 31, 2005 and 2006, respectively.

8. 401(k) Plan

The Company has a 401(k) plan (the Plan) covering all eligible employees. The Plan allows for a discretionary employer match. Through March 31, 2006 the Company has not made any match on employee contributions.

9. Leases

Operating Leases

On May 12, 2005, the Company entered into a Sublease Agreement for its Corporate Office in Cranbury, NJ. The sublease term will expire on February 28, 2012 or on such earlier date upon mutual agreement of both parties. At March 31, 2006, aggregate annual future minimum lease payments under this lease are as follows:

Years ending December 31:	
2006	$ 1,018,859
2007	1,451,463
2008	1,470,060
2009	1,470,060
2010	1,470,060
2011 and thereafter	1,715,070
	$ 8,595,572

Rent expense for the years ended December 31, 2003, 2004, 2005 and for the three months ended March 31, 2005 and 2006 were $110,000, $152,668, $971,687, $48,385, and $353,377, respectively.

Capital Leases

In August 2002, the Company entered into capital lease agreements that provides for up to $1 million of equipment financing through August 2004. The facility was increased to $3 million in May of 2005 and

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Notes To Financial Statements (Continued)

to $5 million in November 2005. These financing arrangements include interest of approximately 9-12%, and lease terms of 36 or 48 months. Eligible assets under the lease lines include laboratory and scientific equipment, computer hardware and software, general office equipment, furniture, and tenant improvements.

At December 31, 2005 and March 31, 2006, the total amount available to the Company under these agreements is $4.0 million and $2.2 million, respectively.

The remaining future minimum payments due in 2006 for all non-cancelable capital leases as of March 31, 2006 are as follows:

Years ending December 31:	
2006	$ 842,013
2007	1,122,684
2008	1,056,522
2009	311,274
2010	8,317
	3,340,810
Less payments for interest	(494,323)
Total principal obligation	2,846,487
Less short-term portion	(852,527)
Long-term portion	$ 1,993,960

The capital lease obligation is secured by the related assets financed by the leases.

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Notes To Financial Statements (Continued)

10. Income Taxes

Deferred income taxes reflect the net effect of temporary difference between the carrying amounts of assets and liabilities for financial reporting purposes and the amounts used for income tax purposes. The significant components of the deferred tax assets and liabilities are as follows:

	December 31,			March 31,
	2003	2004	2005	2006
				(unaudited)
Current deferred tax asset				
Non-cash stock issue to consultants	$	$	$ 63,747	$ 91,951
Others			32,983	27,663
			96,730	119,614
Non-current deferred tax assets				
Amortization	236,957	198,941	132,097	129,246
Research tax credit	62,513	730,903	1,344,230	1,572,505
Net operating loss carryforwards	3,143,749	6,387,827	14,463,790	17,374,472
Others	42,411	75,165	28,829	22,029
Total deferred tax asset	3,485,630	7,392,836	16,065,676	19,217,866
Non-current deferred tax liability:				
Depreciation	(27,896)	(29,865)	(57,027)	(66,169)
Total net deferred tax asset	3,457,734	7,362,971	16,008,649	19,151,697
Less valuation allowance	(3,457,734)	(7,362,971)	(16,008,649)	(19,151,697)
Net deferred tax asset	$	$	$	$

The Company records a valuation allowance for temporary differences for which it is more likely than not that the Company will not receive future tax benefits. At December 31, 2003, 2004, 2005, and at March 31, 2006, the Company recorded valuation allowances of $3,457,734, $7,362,971, $16,008,649, and $19,151,697, respectively, representing a change in the valuation allowance of $3,905,237 and $8,645,678 for the two previous fiscal year-ends and $3,143,048 for the three months ended March 31, 2006, due to the uncertainty regarding the realization of such deferred tax assets, to offset the benefits of net operating losses generated during those years.

Utilization of the net operating loss carryforwards and credits may be subject to a substantial annual limitation due to the ownership change limitations provided by the Internal Revenue Code of 1986, as amended and similar state provisions. The annual limitation may result in the expiration of net operating losses and credits before utilization. The company has not performed an analysis to determine if there has been a change in ownership as defined by the Tax Reform Act of 1986.

The Company recognized a tax benefit of approximately $83,000 and $612,000 in connection with the sale of New Jersey state net operating loss during the years ended December 31, 2004 and 2005.

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Notes To Financial Statements (Continued)

A reconciliation of the statutory tax rates and the effective tax rates for the three years ended December 31, 2003, 2004, 2005, and for the three months ended March 31, 2005 and 2006 are as follows:

	Year Ended December 31,			Three Months Ended March 31,	
	2003	2004	2005	2005	2006
				(unaudited)	(unaudited)
Statutory rate	(34)%	(34)%	(34)%	(34)%	(34)%
State taxes, net of federal benefit	(6)	(6)	(6)	(6)	(6)
Permanent adjustments			1		
Non deductible interest		1			
R&D Credit		(5)	(3)	(3)	(3)
Other		(2)	(1)	(4)	2
Benefit sale of NOL		1	(3)		
Valuation allowance	40	44	43	47	41
Net	%	(1)%	(3)%	%	%

Income tax expense (benefit) consisted of the following components:

	Year Ended December 31,			Three Months Ended March 31,	
	2003	2004	2005	2005	2006
				(unaudited)	(unaudited)
Current payable (benefit):					
Federal	$	$	$	$	$
State		(83,015)	(611,797)		
Deferred:					
Federal					
State					
Income tax benefit	$	$ (83,015)	$ (611,797)	$	$

11. Licenses

The Company acquired rights to develop and commercialize its product candidates through licenses granted by various parties. The following summarizes the Company s material rights and obligations under those licenses:

Mt. Sinai School of Medicine The Company acquired exclusive worldwide patent rights to develop and commercialize Amigal, AT2101 and AT2220 and other pharmacological chaperones for the treatment of diseases caused by misfolded proteins pursuant to a license agreement with Mt. Sinai School of Medicine of New York University. Under this agreement, the Company has no milestone or future payments other than royalties on net sales.

This agreement expires upon expiration of the last of the licensed patent rights, which will be in 2019 if a foreign patent is granted and 2018 otherwise.

University of Maryland, Baltimore County The Company acquired exclusive U.S. patent rights to develop and commercialize AT2101 for the treatment of Gaucher disease from the University of Maryland, Baltimore County. Under this agreement, the Company paid upfront and annual license fees of $24,500, which were expensed as research and development expense. Upon the satisfaction of certain milestones and assuming successful development of AT2101, the Company could be required to make up

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to $175,000 in aggregate payments. The Company is also required to pay royalties on net sales. In connection with this agreement, the Company issued 1,742,000 shares of common stock to Mt. Sinai School of Medicine in April 2002. This agreement expires upon expiration of the last of the licensed patent rights in 2015.

Novo Nordisk A/S The Company acquired exclusive patent rights to develop and commercialize AT2101 for all human indications. Under this agreement, to date the Company paid $400,000 in license fees which were expensed as research and development expense. Upon the satisfaction of certain milestones and assuming successful development of AT2101, the Company could be required to make up to $4,750,000 in aggregate payments. The Company is also required to pay royalties on net sales. This license will terminate in 2016.

Under our license agreements, if the Company owes royalties on net sales for one of its 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, the Company will owe royalties only to Mt. Sinai School of Medicine and will owe no milestone payments. The Company expects to pay royalties to all three licensors with respect to AT2101.

The Company s rights with respect to these agreements to develop and commercialize Amigal, AT2101 and AT2220 may terminate, in whole or in part, if the Company fails to meet certain development or commercialization requirements or if the Company does not meet its obligations to make royalty payments.

12 In-Process Research and Development

During 2002, the Company acquired certain development rights to intellectual property in the form of patent rights owned by Mount Sinai School of Medicine of New York University in exchange for 1,742,000 shares of common stock. The patent rights cover compounds that improve protein folding and protein stability.

The patent rights were reviewed to determine the stage of their development, the achievement of technological feasibility, and the technical milestones needed before commercialization is possible. It was determined, as of the acquisition date, that each patent had significant technical risk associated with achieving the technological feasibility needed for FDA approval and each patent has significant milestones to reach before commercialization is reasonably certain. It was also determined that all of the patents had no alternative future uses if they were not successful. Accordingly, the license was classified as in-process research and development and expensed immediately as of the acquisition date and included in research and development expense. The Company valued the acquired patents using fair value techniques, as a quoted market price was not available. The estimated fair value of the transfer at the date of the transaction was approximately $418,000.

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13. Selected Quarterly Financial Data (Unaudited)

	Quarter Ended			
	March 31	**June 30**	**September 30**	**December 31**
2004				
Net loss	$ (3,339,570)	$ (1,657,368)	$ (1,047,599)	$ (2,760,654)
Net loss attributable to common stockholders	$ (3,343,793)	$ (1,697,871)	$ (1,088,102)	$ (2,801,158)
Basic and diluted net loss per common share(1)	$ (1.45)	$ (0.74)	$ (0.47)	$ (1.21)

	Quarter Ended			
	March 31	**June 30**	**September 30**	**December 31**
2005				
Net loss	$ (3,409,950)	$ (5,348,166)	$ (5,215,161)	$ (5,718,538)
Net loss attributable to common stockholders	$ (3,441,673)	$ (5,379,889)	$ (5,252,809)	$ (5,756,186)
Basic and diluted net loss per common share(1)	$ (1.49)	$ (2.13)	$ (1.54)	$ (1.43)

(1) Per common share amounts for the quarters and full years have been calculated separately. Accordingly, quarterly amounts do not add to the annual amounts because of differences on the weighted-average common shares outstanding during each period principally due to the effect of the Company s issuing shares of its common stock during the year.

14. Subsequent Event (Unaudited)

In April 2006, the Company received approximately $27.5 million from the issuance of 21,825,131 shares of series C redeemable convertible preferred stock at $1.26 per share. The Company expects to record a beneficial conversion charge (deemed dividend) during the second quarter of 2006 of approximately $19.4 million related to the issuance of the Series C redeemable convertible preferred stock.

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PART II
INFORMATION NOT REQUIRED IN PROSPECTUS

Item 13. ***Other Expenses of Issuance and Distribution.***

The following table indicates the expenses to be incurred in connection with the offering described in this Registration Statement, other than underwriting discounts and commissions, all of which will be paid by the Registrant. All of the amounts are estimated except the Securities and Exchange Commission registration fee and the National Association of Securities Dealers, Inc. filing fee.

Securities and Exchange Commission registration fee	$	9,229
National Association of Securities Dealers, Inc. filing fee	$	9,125
Nasdaq National Market listing fee	$	5,000
Accounting fees and expenses		*
Legal fees and expenses		*
Blue Sky fees and expenses		*
Transfer Agent s expenses		*
Printing and engraving fees		*
Miscellaneous		*
Total expenses	$	*

* To be filed by amendment.

Item 14. ***Indemnification of Directors and Officers.***

Section 102 of the Delaware General Corporation Law permits a corporation to eliminate the personal liability of directors of a corporation to the corporation or its stockholders for monetary damages for a breach of fiduciary duty as a director, except where the director breached his or her duty of loyalty, failed to act in good faith, engaged in intentional misconduct or knowingly violated a law, authorized the payment of a dividend or approved a stock repurchase in violation of Delaware corporate law or obtained an improper personal benefit. The Registrant s restated certificate of incorporation to be effective upon closing of this offering provides that no director of the Registrant shall be personally liable to it or its stockholders for monetary damages for any breach of fiduciary duty as director, notwithstanding any provision of law imposing such liability, except to the extent that the Delaware General Corporation Law prohibits the elimination or limitation of liability of directors for breaches of fiduciary duty.

Section 145 of the Delaware General Corporation Law provides that a corporation has the power to indemnify any person who was or is a party or is threatened to be made a party to any threatened, pending or completed action, suit or proceeding, whether civil, criminal, administrative or investigative, by reason of the fact that the person is or was a director, officer, employee or agent of the corporation, or is or was serving at the request of the corporation as a director, officer, employee or agent of another corporation, partnership, joint venture, trust or other enterprise, against expenses (including attorneys fees), judgments, fines and amounts paid in settlements actually and reasonably incurred by the person in connection with an action, suit or proceeding to which he or she is or is threatened to be made a party by reason of such position, if such person acted in good faith and in a manner he or she reasonably believed to be in or not opposed to the best interests of the corporation, and, in any criminal action or proceeding, had no reasonable cause to believe his or her conduct was unlawful, except that, in the case of actions brought by or in the right of the corporation, no indemnification shall be made with respect to any claim, issue or matter as to which such person shall have been adjudged to be liable to the corporation unless and only to the extent that the Delaware Court of Chancery or other adjudicating court determines that, despite the adjudication of liability but in view of all of the circumstances of the case, such person is fairly and reasonably entitled to indemnity for such expenses which the Delaware Court of Chancery or such other court shall deem proper.

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The Registrant s restated certificate of incorporation, which is to be effective upon the closing of this offering, provides that the Registrant will, to the fullest extent permitted by Section 145 of the Delaware General Corporation Law and the Registrant s by-laws (each as amended from time to time), indemnify each person who was or is a party or is threatened to be made a party to any threatened, pending or completed action, suit or proceeding, whether civil, criminal, administrative or investigative, by reason of the fact that he or she is or was, or has agreed to become, a director or officer of the Registrant, or is or was serving, or has agreed to serve, at the request of the Registrant, as a director, officer, partner, or trustee of, or in a similar capacity with, another corporation, partnership, joint venture, trust or other enterprise, including any employee benefit plan (all such persons being referred to hereafter as an Indemnitee), or by reason of any action alleged to have been taken or omitted in such capacity, against all expenses (including attorneys fees), judgments, fines and amounts paid in settlement actually and reasonably incurred by, or on behalf of, the Indemnitee in connection with such action, suit or proceeding and any appeal therefrom. Such indemnification may include payment by the Registrant of expenses in defending an action or proceeding in advance of the final disposition of such action or proceeding upon receipt of an undertaking by the Indemnitee (such undertaking acceptable by the Registrant without reference to the financial ability of the Indemnitee) to repay such payment if it is ultimately determined that the Indemnitee is not entitled to indemnification under the Registrant s restated certificate of incorporation; however, the Registrant will not indemnify any person seeking indemnification in connection with a proceeding (or part thereof) initiated by such person, unless such initiation was approved by the Registrant s board of directors. Also, the indemnification rights provided in the Registrant s restated certificate of incorporation (i) are not exclusive of any other rights to which those indemnified may be entitled under any law, agreement or vote of stockholders or disinterested directors or otherwise, and (ii) will inure to the benefit of the heirs, executors and administrators of such persons. The Registrant may, to the extent authorized from time to time by its board of directors, grant indemnification rights to other employees of the Registrant or other persons serving the Registrant and such rights may be equivalent to, or greater or less than, those set forth in the Registrant s restated certificate of incorporation.

The Registrant has entered into indemnification agreements with each of its directors. These agreements, among other things, require the Registrant to indemnify each director to the fullest extent permitted by Delaware law, including indemnification of expenses such as attorneys fees, judgments, fines and settlement amounts incurred by the director in any action or proceeding, including any action or proceeding by or in right of the Registrant, arising out of the person s services as a director.

The Registrant maintains a general liability insurance policy that covers certain liabilities of the Registrant s directors and officers arising out of claims based on acts or omissions in their capacities as directors or officers.

In any underwriting agreement that the Registrant enters into in connection with the sale of common stock being registered hereby, the underwriters will agree to indemnify, under certain conditions, the Registrant, its directors, its officers and persons who control the Registrant within the meaning of the Securities Act, against certain liabilities.

Item 15. ***Recent Sales of Unregistered Securities.***

Set forth below is information regarding shares of common stock and preferred stock issued, and options and warrants granted, by the Registrant within the past three years that were not registered under the Securities Act. Also included is the consideration, if any, received by the Registrant for such shares, options and warrants and information relating to the section of the Securities Act, or rule of the Securities and Exchange Commission, under which exemption from registration was claimed.

(a) Issuances of Securities

1. On June 20, 2003, the Registrant issued a promissory note in the amount of $936,875 to CHL Medical Partners, II, L.P. The Registrant also issued a promissory note in the amount of

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$63,125 to CHL Medical Partners Side Fund II, L.P. The principal outstanding under the notes was converted into shares of series B redeemable convertible preferred stock in May 2004.

2. On August 25, 2003, the Registrant issued a promissory note in the amount of $936,875, together with a warrant to purchase 312,291 shares of common stock at an exercise price of $0.075 per share, to CHL Medical Partners II, L.P. The Registrant also issued a promissory note in the amount of $63,125, together with a warrant to purchase 21,042 shares of common stock at an exercise price of $0.075 per share, to CHL Medical Partners Side Fund II, L.P. The principal outstanding under the notes was converted into shares of series B redeemable convertible preferred stock in May 2004. CHL Medical Partners II, L.P. and CHL Medical Partners Side Fund II, L.P. exercised their warrants in August 2005.

3. On November 26, 2003, the Registrant issued a promissory note in the amount of $936,875, together with a warrant to purchase 312,291 shares of common stock at an exercise price of $0.075 per share, to CHL Medical Partners II, L.P. The Registrant also issued a promissory note in the amount of $63,125, together with a warrant to purchase 21,042 shares of common stock at an exercise price of $0.075 per share, to CHL Medical Partners Side Fund II, L.P. CHL Medical Partners II, L.P. and CHL Medical Partners Side Fund II, L.P. exercised their warrants in August 2005.

4. On February 5, 2004, the Registrant issued a promissory note in the amount of $1,873,750, together with a warrant to purchase 312,291 shares of common stock at an exercise price of $0.075 per share, to CHL Medical Partners II, L.P. The promissory note issued on February 5, 2004 amended and restated in its entirety the promissory note issued to CHL Medical Partners II, L.P. on November 26, 2003. The Registrant also issued a promissory note in the amount of $126,250, together with a warrant to purchase 21,042 shares of common stock at an exercise price of $0.075 per share, to CHL Medical Partners Side Fund II, L.P. The promissory note issued on February 5, 2004 amended and restated in its entirety the promissory note issued to CHL Medical Partners Side Fund II, L.P. on November 26, 2003. CHL Medical Partners II, L.P. and CHL Medical Partners Side Fund II, L.P. exercised their warrants in August 2005.

5. On April 19, 2004, the Registrant issued a promissory note in the amount of $2,342,188 to CHL Medical Partners II, L.P. This promissory note amended and restated in its entirety the promissory note issued to CHL Medical Partners II, L.P. on February 5, 2004. The Registrant also issued a promissory note in the amount of $157,812 to CHL Medical Partners Side Fund II, L.P. This promissory note amended and restated in its entirety the promissory note issued to CHL Medical Partners Side Fund II, L.P. on February 5, 2004. The principal outstanding under the notes was converted into shares of Series B convertible preferred stock in May 2004.

6. On May 4, 2004 and March 24, 2005, the Registrant issued an aggregate of 36,470,591 shares of our series B redeemable convertible preferred stock at a price of $0.85 per share, together with warrants to purchase an aggregate of 555,003 shares of series B redeemable convertible preferred stock at an exercise price of $0.85 per share, to institutional investors for aggregate cash proceeds of approximately $31 million.

7. On August 17, 2005 and April 17, 2006, the Registrant issued an aggregate of 43,650,262 shares of our series C redeemable convertible preferred stock at a price of $1.26 per share to institutional investors for aggregate cash proceeds of approximately $55 million.

8. On August 23, 2005, the Registrant issued, pursuant to the exercise of common stock purchase warrants, (i) 936,873 shares of our common stock at a purchase price of $0.075 per share to CHL Medical Partners II, L.P., and (ii) 63,126 shares of our common stock at a purchase price of $0.075 per share to CHL Medical Partners II Side Fund, L.P., for aggregate cash proceeds of approximately $75,000.

No underwriters were involved in the foregoing sales of securities. The securities described in this section (a) of Item 15 were issued to a combination of foreign and U.S. investors in reliance upon the

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exemption from the registration requirements of the Securities Act, as set forth in Section 4(2) under the Securities Act and Rule 506 of Regulation D promulgated thereunder, relative to sales by an issuer not involving any public offering, to the extent an exemption from such registration was required. All purchasers of shares of convertible preferred stock described above represented to the Registrant in connection with their purchase that they were accredited investors and were acquiring the shares for investment and not distribution, that they could bear the risks of the investment and could hold the securities for an indefinite period of time. The purchasers received written disclosures that the securities had not been registered under the Securities Act and that any resale must be made pursuant to a registration statement or an available exemption from such registration.

(b) Stock Option Grants

Since inception, the Registrant has granted options to certain employees, consultants and others to purchase an aggregate of 16,030,166 shares of common stock as of May 3, 2006. As of May 3, 2006, options to purchase 2,202,984 shares of common stock had been exercised, options to purchase 1,060,840 shares of common stock had been forfeited, and options to purchase 13,552,120 shares of common stock remained outstanding at a weighted average exercise price of $0.48 per share.

The issuance of stock options and the common stock issuable upon the exercise of such options as described in this section (b) of Item 15 were issued pursuant to written compensatory plans or arrangements with the Registrant s employees, directors and consultants, in reliance on the exemption provided by Rule 701 promulgated under the Securities Act. All recipients either received adequate information about the Registrant or had access, through employment or other relationships, to such information.

All of the foregoing securities are deemed restricted securities for purposes of the Securities Act. All certificates representing the issued shares of common stock described in this Item 15 included appropriate legends setting forth that the securities had not been registered and the applicable restrictions on transfer.

Item 16. ***Exhibits and Financial Statement Schedules.***

(a) Exhibits

Exhibit Number	Description of Exhibit
1.1**	Form of Underwriting Agreement
3.1**	Certificate of Incorporation of the Registrant, as amended
3.2*	Form of Restated Certificate of Incorporation of the Registrant to be effective upon completion of this offering
3.3*	By-laws of the Registrant
3.4*	Form of Restated By-laws of the Registrant to be effective upon completion of this offering
4.1**	Specimen Stock Certificate evidencing shares of common stock
4.2*	Second Amended and Restated Investor Rights Agreement, dated as of August 17, 2005, as amended
4.3*	Warrant to purchase shares of common stock, dated August 28, 2002
5.1**	Opinion of Bingham McCutchen LLP
10.1*	2002 Equity Incentive Plan, as amended
10.2*	2006 Equity Incentive Plan
10.3*	2006 Employee Stock Purchase Plan
10.4*+	License Agreement, dated as of April 15, 2002, by and between the Registrant and Mount Sinai School of Medicine of New York University
10.5*+	License Agreement, dated as of June 26, 2003, by and between the Registrant and University of Maryland, Baltimore County
10.6*+	Exclusive License Agreement, dated as of June 8, 2005, by and between the Registrant and Novo Nordisk, A/ S

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Exhibit Number	Description of Exhibit
10.7*	Sublease Agreement, dated as of May 12, 2005, by and between the Registrant and Purdue Pharma, L.P.
10.8*	Amended and Restated Employment Agreement, dated as of April 28, 2006, by and between the Registrant and John F. Crowley
10.9*	Letter Agreement, dated as of November 9, 2004, by and between the Registrant and Matthew R. Patterson
10.10*	Letter Agreement, dated as of June 3, 2005, by and between the Registrant and Pedro Huertas, M.D.
10.11*	Letter Agreement, dated as of December 19, 2005, by and between the Registrant and David Lockhart, Ph.D.
10.12*	Letter Agreement, dated as of February 2, 2006, by and between the Registrant and Karin Ludwig, M.D.
10.13*	Change in Control Agreement, dated as of March 6, 2006, by and between the Registrant and David Palling, Ph.D.
10.14*	Change in Control Agreement, dated as of March 6, 2006, by and between the Registrant and S. Nicole Schaeffer
10.15*	Change in Control Agreement, dated as of March 6, 2006, by and between the Registrant and Gregory P. Licholai, M.D.
10.16*	Consulting Agreement, dated as of February 28, 2006, by and between the Registrant and Donald J. Hayden, Jr.
10.17*	Letter Agreement, dated as of May 12, 2006, by and between the Registrant and Douglas A. Branch
10.18*	Form of Director and Officer Indemnification Agreement
10.19	Letter Agreement, dated as of May 12, 2006, by and between the Registrant and Mark Simon.
23.1	Consent of Ernst & Young LLP
23.2**	Consent of Bingham McCutchen LLP (included in Exhibit 5.1)
24.1*	Powers of Attorney

* Previously filed.

** To be filed by amendment.

\+ Portions of this exhibit have been omitted pursuant to a confidential treatment request. This information has been filed or will be filed separately with the Securities and Exchange Commission.

Financial Statement Schedules

All schedules have been omitted because they are not required or are not applicable or the required information is shown in the financial statements or notes thereto.

Item 17. ***Undertakings***

(a) The undersigned Registrant hereby undertakes to provide to the underwriters at the closing specified in the Underwriting Agreement, certificates in such denominations and registered in such names as required by the underwriters to permit prompt delivery to each purchaser.

(b) Insofar as indemnification for liabilities arising under the Securities Act may be permitted to directors, officers and controlling persons of the Registrant pursuant to the provisions described under Item 14 above, or otherwise, the Registrant has been advised that in the opinion of the Securities and Exchange Commission such indemnification is against public policy as expressed in the Securities Act and is, therefore, unenforceable. In the event that a claim for indemnification against such liabilities (other than the payment by the Registrant of expenses incurred or paid by a director, officer or controlling person of the Registrant in the successful defense of any action, suit or proceeding) is asserted by such director, officer or controlling person in connection with the securities being registered, the Registrant will, unless in the opinion of its counsel the matter has been settled by controlling precedent, submit to a court of appropriate jurisdiction the question whether such indemnification by it is against public policy as expressed in the Securities Act and will be governed by the final adjudication of such issue.

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(c) The undersigned Registrant hereby undertakes that:

(1) For purposes of determining any liability under the Securities Act, the information omitted from the form of prospectus filed as part of this Registration Statement in reliance upon Rule 430A and contained in a form of prospectus filed by the Registrant pursuant to Rule 424(b)(1) or (4) or 497(h) under the Securities Act shall be deemed to be part of this Registration Statement as of the time it was declared effective.

(2) For the purpose of determining any liability under the Securities Act, each post-effective amendment that contains a form of prospectus shall be deemed to be a new Registration Statement relating to the securities offered therein, and the offering of such securities at that time shall be deemed to be the initial *bona fide* offering thereof.

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SIGNATURES

Pursuant to the requirements of the Securities Act, the Registrant certifies that it has duly caused this Registration Statement to be signed on its behalf by the undersigned, thereunto duly authorized, in the city of Cranbury, New Jersey, on the 19th day of June, 2006.

AMICUS THERAPEUTICS, INC.
By: /s/ Douglas A. Branch

Douglas A. Branch
Vice President Secretary and General Counsel

Pursuant to the requirements of the Securities Act, this Registration Statement has been signed by the following persons in the capacities and on the dates indicated.

Signature	Title	Date
*	President, Chief Executive Officer and Director (principal executive officer)	June 19, 2006
/s/ **John M. McAdam**	Director, Finance and Accounting, and Corporate Controller (principal financial and accounting officer)	June 19, 2006
*	Chairman of the Board	June 19, 2006
*	Director	June 19, 2006
*	Director	June 19, 2006
*	Director	June 19, 2006
*	Director	June 19, 2006
*	Director	June 19, 2006
*	Director	June 19, 2006

*By: /s/ **Douglas A. Branch**

Douglas A. Branch
Attorney-in-Fact

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