Filed Pursuant to Rule 424(b)(4)
Registration No. 333-141700

5,000,000 Shares

Common Stock

This offering is our initial public offering of shares of our common stock. We are offering 5,000,000 shares of common stock.

Our Common Stock

The initial public offering price is $15.00 per share. Prior to this offering, there has been no public market for our shares, Our common stock has been approved for listing on The NASDAQ Global Market under the symbol “FOLD”.

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

	Per Share	Total
Public offering price	$15.00	$75,000,000
Underwriting discount	$ 1.05	$ 5,250,000
Proceeds, before expenses	$13.95	$69,750,000

The underwriters may also purchase up to an additional 750,000 shares of common stock from us at the public offering price, less the underwriting discount, within 30 days from the date of this prospectus to cover over-allotments.

Neither the Securities and Exchange Commission nor any state securities commission has approved or disapproved these securities, or determined if this prospectus is truthful or complete. Any representation to the contrary is a criminal offense.

The underwriters expect to deliver the shares to purchasers on or about June 5, 2007.

Morgan Stanley **Merrill Lynch & Co.**

JPMorgan

Lazard Capital Markets **Pacific Growth Equities, LLC**

May 30, 2007

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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, Inc.

Until June 24, 2007, 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.

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 8 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. Our lead products in development are Amigal for Fabry disease, Plicera 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 five approved therapeutics to treat Fabry, Gaucher and Pompe disease totaled more than $1.5 billion in 2006, as publicly reported by companies that market these therapeutics. We hold worldwide commercialization rights to Amigal, Plicera and AT2220 and we intend to establish a commercial infrastructure and targeted sales force to market some or all of our products. Currently, none of our product candidates are approved for commercial sale or have generated any revenue from commercial sales.

We have completed enrollment of our Phase II clinical trials of Amigal, and have obtained initial results in the first eleven patients who have completed at least 12 weeks of treatment. These initial results suggest that treatment with Amigal causes an increase in the activity of α-galactosidase A, or α-GAL, the enzyme deficient in Fabry disease. We believe this increase is likely to be clinically meaningful for a wide range of Fabry patients. Data for the three patients from whom we have kidney biopsies suggest that the increased level of α-GAL that occurs after treatment with Amigal may result in a decrease of globotriaosylceramide, or GL-3. GL-3 is the substrate that accumulates in the cells of patients with Fabry disease and is believed to cause the majority of disease symptoms. Reduction of the level of GL-3 in a specific cell type of the kidney was the basis of prior regulatory approval by the FDA of an enzyme replacement therapy for the treatment of Fabry disease. We expect to complete our Phase II clinical trials of Amigal by the end of 2007.

We are currently conducting two Phase II clinical trials of Plicera in Type I Gaucher patients. We expect to obtain preliminary results from the first of these Phase II clinical trials by the end of 2007. We are currently conducting Phase I trials of AT2220 for Pompe disease and expect to initiate a Phase II clinical trial by the end of 2007.

Certain human diseases result from mutations in specific genes that, in many cases, lead to the production of proteins with reduced stability. Proteins with such mutations may not fold into their correct three-dimensional shape and are generally referred to as misfolded proteins. 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 to cellular dysfunction and disease.

Our novel approach to the treatment of human genetic diseases consists of using pharmacological chaperones that selectively bind to the target protein, increasing the stability of the protein and helping it fold into the correct three-dimensional shape. This allows proper trafficking of the protein, thereby increasing protein activity, improving cellular function and potentially reducing cell stress.

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 of recombinant enzyme. 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 and ease of use, potentially improving treatment of these diseases. In addition, we believe our technology is broadly applicable to other diseases for which protein stabilization and improved folding may be beneficial, 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 and increased risk of heart attack and stroke. We are currently conducting multiple Phase II clinical trials of Amigal. We expect to complete our Phase II trials of Amigal by the end of 2007.
- *Plicera for Gaucher disease.* We are developing Plicera for the treatment of Gaucher disease, which commonly causes an enlarged liver and spleen, abnormally low levels of red blood cells and platelets, and skeletal complications. Some patients also present with neurological complications. We are currently conducting two Phase II clinical trials of Plicera in Type I Gaucher patients. We expect to obtain preliminary results from the first of these two trials by the end 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. We are currently conducting Phase I clinical trials of AT2220 and expect to initiate a Phase II clinical trial by the end of 2007.

Preliminary Data from our Ongoing Phase II Clinical Trials in Fabry Disease

We have completed enrollment of our four Phase II clinical trials of Amigal and have obtained initial results for the first eleven patients that have completed at least 12 weeks of treatment. Each of these patients has been treated with various doses and regimens of Amigal for various periods of time in accordance with the Phase II protocols. Amigal has been well-tolerated to date with no reported drug-related serious, adverse events.

The eleven patients represent ten different genetic mutations and have baseline levels of α-GAL in white blood cells of between 0% and 30% of normal. An increase in α-GAL enzyme levels in white blood cells has been observed in ten out of the eleven patients. These initial results suggest that treatment with Amigal causes an increase in the level of α-GAL, the enzyme deficient in Fabry disease, in a wide range of Fabry patients. In addition, we believe that this increase is likely to be therapeutically meaningful because it is generally believed that even small increases in lysosomal enzyme levels may have clinical benefits.

GL-3, the lipid substrate broken down by α-GAL in the lysosome, accumulates in the cells of patients with Fabry disease and is believed to be the cause of the majority of disease symptoms. Reduction of the level of GL-3 in a specific cell type of the kidney was the basis of prior regulatory approval by the FDA of an enzyme replacement therapy for the treatment of Fabry disease. Kidney GL-3 levels are available for three patients and were assessed by an independent expert using light and electron microscopy. A decrease in GL-3 was observed in multiple cell types of the kidney of one patient after 12 weeks of treatment. A second patient showed a decrease of GL-3 levels in the same kidney cell types after 24 weeks of treatment, but these decreases were not independently conclusive because of the patient's lower levels of GL-3 at baseline. An increase in the level of α-GAL in white blood cells was observed in both of these two patients after treatment

with Amigal. A third patient showed an increase in GL-3 levels in some cell types of the kidney and no change or a decrease in others after 12 weeks of treatment. Of the eleven patients who have completed at least 12 weeks of treatment to date in our ongoing clinical trials, this is the one patient who did not show an increase in the level of α-GAL in white blood cells after treatment with Amigal.

Amigal has been well-tolerated to date with no reported drug-related serious adverse events. Four patients have been on Amigal for over a year. Adverse events were mostly mild and reported by the investigators as unlikely to be related to Amigal. One 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 trials to date do not necessarily predict final results for our Phase II clinical trials. The results from additional patients in our ongoing Phase II clinical studies or additional data from these first eleven patients may cause the results of our Phase II studies to differ from or be less favorable than the preliminary results presented above. We cannot guarantee that our Phase II clinical studies will ultimately be successful.

Data from our Phase I Clinical Trials in Gaucher Disease

We recently completed two double-blind, placebo-controlled, dose escalation Phase I clinical trials in healthy volunteers. These trials were designed to evaluate the safety, tolerability and pharmacokinetics of Plicera. In the first study, 36 subjects received a single dose of one of five dose levels of Plicera. This was followed by a multiple-dose study in which 18 subjects received one of three dose levels of Plicera once daily for 7 consecutive days. The data from our Phase I clinical trials in healthy volunteers showed that Plicera was generally safe and well tolerated at all doses. There were no serious adverse events and no subjects withdrew or discontinued due to an adverse event. The trials also demonstrated that Plicera has good oral bioavailability, and linear pharmacokinetics with a terminal half-life in plasma of approximately fourteen hours. Also, the data from the multiple-dose Phase I clinical trial showed a statistically significant, dose-related increase in β-glucocerebrosidase, or GCase, levels in the white blood cells of normal, healthy volunteers who received oral administration of Plicera for seven days. GCase is the enzyme deficient in Gaucher disease.

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 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 called lysosomal storage disorders;
- rapidly advance our lead programs;
- leverage our proprietary approach to the discovery and development of 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 $65.9 million for the year ended December 31, 2006 and $9.7 million for the three months ended March 31, 2007. As of March 31, 2007, we had an accumulated deficit of $93.4 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.

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

THE OFFERING

Common stock we are offering	5,000,000 shares
Common stock to be outstanding after this offering	22,234,426 shares
Over-allotment option	750,000 shares
Use of proceeds	The net proceeds from this offering will be approximately $67.9 million, or approximately $78.3 million if the underwriters exercise their over-allotment option in full, after deducting underwriting discounts and commissions and estimated 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.
NASDAQ Global Market symbol	FOLD

The number of shares of common stock to be outstanding immediately after the offering is based on 1,162,502 shares of common stock outstanding as of April 25, 2007 and the issuance of 16,071,924 shares of common stock issuable upon the automatic conversion of all shares of our redeemable convertible preferred stock outstanding upon the closing of this offering. The number of shares of common stock to be outstanding after this offering excludes:

- 2,549,950 shares of common stock issuable upon the exercise of stock options outstanding as of April 25, 2007, with a weighted average exercise price of $7.56 per share;
- 5,333 shares of common stock issuable upon exercise of a warrant to purchase common stock at an exercise price of $5.63 per share;
- 40,797 shares of common stock issuable in connection with the exercise of outstanding warrants to purchase shares of series B redeemable convertible preferred stock. Upon the closing of this offering, these warrants will be automatically exercised and the shares of series B redeemable convertible preferred stock automatically converted into shares of common stock on a one for one basis;
- an aggregate of 966,667 shares of common stock reserved for future issuance under our 2007 equity incentive plan as of the closing of this offering;
- an aggregate of 200,000 shares of common stock reserved for future issuance under our 2007 director option plan as of the closing of this offering; and
- an aggregate of 200,000 shares of common stock reserved for future issuance under our 2007 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 warrants to purchase capital stock described above;
- no exercise by the underwriters of their option to purchase shares of common stock to cover over-allotments; and
- a 1-for-7.5 reverse split of our common stock and preferred stock effected on May 24, 2007.

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 and pro forma net loss per share data for the year end December 31, 2006, and the three month period ended March 31, 2007, give effect, as of the beginning of each such period, to the issuance in March 2007 of 1,976,527 shares of our series D redeemable convertible preferred stock, and the automatic conversion of all outstanding shares of our redeemable convertible preferred stock into 16,071,924 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, 2007, to the foregoing events and the elimination of our warrant liability.

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 the initial public offering price of $15.00 per share, after deducting underwriting discounts and commissions and estimated offering expenses payable by us.

	Year Ended December 31,			Three Months Ended March 31,		Period from February 4, 2002 (Inception) to March 31, 2007
	2004	2005	2006	2006	2007	(unaudited)
				(unaudited)	(unaudited)	
	(in thousands, except shares and per share data)					
Statement of Operations Data:						
Operating expenses:						
Research and development	$ 6,301	$ 13,652	$ 33,630	$ 6,028	$ 7,085	$ 65,889
General and administrative	2,081	6,877	12,277	1,900	2,850	25,642
Impairment of leasehold improvements	—	—	—	—	—	1,030
Depreciation and amortization	146	303	952	199	297	1,854
In-process research and development	—	—	—	—	—	418
Total operating expenses	8,528	20,831	46,859	8,127	10,232	94,833
Loss from operations	(8,528)	(20,831)	(46,859)	(8,127)	(10,232)	(94,833)
Other income (expenses):						
Interest income	190	610	1,991	238	693	3,501
Interest expense	(550)	(82)	(273)	(52)	(92)	(1,175)
Change in fair value of warrant liability	(2)	(280)	(22)	(343)	(64)	(368)
Other expense	—	—	(1,182)	(3)	—	(1,182)
Loss before tax benefit	(8,890)	(20,584)	(46,345)	(8,287)	(9,695)	(94,057)
Income tax benefit	83	612	—	—	—	695
Net loss	(8,807)	(19,972)	(46,345)	(8,287)	(9,695)	(93,362)
Deemed dividend	—	—	(19,424)	—	—	(19,424)
Preferred stock accretion	(125)	(139)	(159)	(41)	(41)	(492)
Net loss attributable to common stockholders	$(8,932)	$(20,111)	$(65,928)	$ (8,328)	$ (9,736)	$ (113,278)

	Year Ended December 31,			Three Months Ended March 31,	
	2004	2005	2006	2006	2007
				(unaudited)	(unaudited)
	(in thousands, except shares and per share data)				
Net loss attributable to common stockholders per common shares – basic and diluted	$ (29.05)	$ (49.02)	$ (89.58)	$ (15.43)	$ (10.21)
Weighted-average common shares outstanding – basic and diluted	307,539	410,220	735,967	539,789	953,959
Unaudited pro forma net loss			$ (46,345)		$ (9,695)
Unaudited pro forma basic and diluted net loss per share			$ (2.76)		$ (0.57)
Unaudited shares used to compute pro forma basic and diluted net loss per share			16,807,933		17,025,885

	As of March 31, 2007		
	Actual	Pro Forma	Pro Forma as Adjusted
		(unaudited) (in thousands)	
Balance Sheet Data:			
Cash and cash equivalents and marketable securities	$ 67,706	$ 67,706	$ 135,570
Working capital	59,526	59,526	127,390
Total assets	73,048	73,048	140,912
Total liabilities	11,146	10,474	10,474
Redeemable convertible preferred stock	148,184	—	—
Deficit accumulated during the development stage	(93,362)	(92,690)	(92,690)
Total stockholders' (deficiency) equity	(86,282)	62,574	130,439

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

Since inception, we have incurred significant operating losses. Our net loss attributable to common stockholders was $65.9 million and $9.7 million for the year ended December 31, 2006 and the three months ended March 31, 2007, respectively. As of March 31, 2007, we had an accumulated deficit of $93.4 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 II clinical trials of Plicera for the treatment of Gaucher disease and potentially conduct later-stage clinical trials of Plicera;
- continue our ongoing Phase I clinical trials of AT2220 for the treatment of Pompe disease and potentially conduct later-stage clinical trials of AT2220;
- continue the research and development of additional product candidates;
- seek regulatory approvals for our product candidates that successfully complete clinical trials;
- establish a sales and marketing infrastructure to commercialize products for which we may obtain regulatory approval; and
- add operational, financial and management information systems and personnel, including personnel to support our product development efforts and our obligations as a public company.

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

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

We believe that the net proceeds from 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 early 2010. 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 clinical trials of Amigal, Plicera and AT2220;
- the scope, progress, results and costs of preclinical development, laboratory testing and clinical trials for our other product candidates;
- the costs, timing and outcome of regulatory review of our product candidates;
- the number and development requirements of other product candidates that we pursue;
- the costs of commercialization activities, including product marketing, sales and distribution;
- the emergence of competing technologies and other adverse market developments;
- the costs of preparing, filing and prosecuting patent applications and maintaining, enforcing and defending intellectual property related claims;
- the extent to which we acquire or invest in businesses, products and technologies; and
- our ability to establish collaborations and obtain milestone, royalty or other payments from any such collaborators.

Any 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.

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

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

- obtaining supplies of Amigal, Plicera and AT2220 for completion of our clinical trials on a timely basis;
- successful completion of preclinical studies and clinical trials;
- obtaining marketing approvals from the United States Food and Drug Administration, or FDA, and similar regulatory authorities outside the United States;
- establishing commercial-scale manufacturing arrangements with third party manufacturers whose manufacturing facilities are operated in compliance with current good manufacturing practice, or cGMP, regulations;
- launching commercial sales of the product, whether alone or in collaboration with others;
- acceptance of the product by patients, the medical community and third party payors;
- competition from other companies and their therapies;
- successful protection of our intellectual property rights from competing products in the United States and abroad; and
- a continued acceptable safety and efficacy profile of our product candidates following approval.

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

Each of the diseases that our product candidates are being developed to address is relatively rare. Our projections of both the number of people who have these diseases, as well as the subset of people with these diseases who have the potential to benefit from treatment with our product candidates, are based on estimates. Currently, most reported estimates of the prevalence of these diseases are based on studies of small subsets of the population of specific geographic areas, which are then extrapolated to estimate the prevalence of the diseases in the broader world population. In addition, as new studies are performed the estimated prevalence of these diseases may change. In fact, as a result of some recent studies, we believe that previously reported studies do not accurately account for the prevalence of Fabry disease and that the prevalence of Fabry disease could be many times higher than previously reported. There can be no assurance that the prevalence of Fabry

disease, Gaucher disease or Pompe disease in the study populations, particularly in these newer studies, accurately reflect the prevalence of these diseases in the broader world population.

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

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

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

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

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

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

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

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

Each of the diseases that our lead product candidates are intended to treat is relatively rare and we expect only a subset of the patients with these diseases to be eligible for our clinical trials. Given that each of our product candidates is in the early stages of required testing, we may not be able to initiate or continue clinical trials for each or all of our product candidates if we are unable to locate a sufficient number of eligible patients to participate in the clinical trials required by the FDA or other non-United States regulatory agencies. The requirements of our clinical testing 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. Further, if we are required to include patients in our clinical trials who have never received enzyme replacement therapy, we may experience yet further difficulty and delay enrolling patients in our trials. Our inability to enroll a sufficient number of patients for any of our current or future clinical trials would result in significant delays or may require us to abandon one or more clinical trials altogether.

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

Before obtaining regulatory approval for the sale of our product candidates, we must conduct, at our own expense, extensive preclinical tests to demonstrate the safety of our product candidates in animals, and clinical trials to demonstrate the safety and efficacy of our product candidates in humans. Preclinical and clinical testing is expensive, difficult to design and implement, and can take many years to complete. A failure of one or more of our preclinical studies or clinical trials can occur at any stage of testing. We may experience numerous unforeseen events during, or as a result of, preclinical testing and the clinical trial process that could delay or prevent our ability to obtain regulatory approval or commercialize our product candidates, including:

- our preclinical tests or clinical trials may produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional preclinical testing or clinical trials or we may abandon projects that we expect to be promising;
- regulators or institutional review boards may not authorize us to commence a clinical trial or conduct a clinical trial at a prospective trial site;

- conditions imposed on us by the FDA or any non-United States regulatory authority regarding the scope or design of our clinical trials or may require us to resubmit our clinical trial protocols to institutional review boards for re-inspection due to changes in the regulatory environment;
- the number of patients required for our clinical trials may be larger than we anticipate or participants may drop out of our clinical trials at a higher rate than we anticipate;
- our third party contractors or clinical investigators may fail to comply with regulatory requirements or fail to meet their contractual obligations to us in a timely manner;
- we might have to suspend or terminate one or more of our clinical trials if we, the regulators or the institutional review boards determine that the participants are being exposed to unacceptable health risks;
- regulators or institutional review boards may require that we hold, suspend or terminate clinical research for various reasons, including noncompliance with regulatory requirements;
- the cost of our clinical trials may be greater than we anticipate;
- the supply or quality of our product candidates or other materials necessary to conduct our clinical trials may be insufficient or inadequate or we may not be able to reach agreements on acceptable terms with prospective clinical research organizations; and
- the effects of our product candidates may not be the desired effects or may include undesirable side effects or the product candidates may have other unexpected characteristics.

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

- be delayed in obtaining, or may not be able to obtain, marketing approval for one or more of our product candidates;
- obtain approval for indications that are not as broad as intended or entirely different than those indications for which we sought approval; or
- have the product removed from the market after obtaining marketing approval.

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

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

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

- the prevalence and severity of any side effects, including any limitations or warnings contained in a product’s approved labeling;
- the efficacy and potential advantages over alternative treatments;
- the pricing of our product candidates;

- relative convenience and ease of administration;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- the strength of marketing and distribution support and timing of market introduction of competitive products;
- publicity concerning our products or competing products and treatments; and
- sufficient third party insurance coverage or reimbursement.

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

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

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

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

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

on our ability to set a price we believe is fair for our products and may affect our ability to generate revenue and achieve or maintain profitability.

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

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

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

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

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

- our inability to recruit and retain adequate numbers of effective sales and marketing personnel;
- the inability of sales personnel to obtain access to or persuade adequate numbers of physicians to prescribe our products;
- the lack of complementary products to be offered by our sales personnel, which may put us at a competitive disadvantage against companies with broader product lines;
- unforeseen costs associated with creating our own sales and marketing team or with entering into a partnering agreement with an independent sales and marketing organization; and
- efforts by our competitors to commercialize products at or about the time when our product candidates would be coming to market.

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

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

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

- business combinations or significant changes in a distributor's business strategy may also adversely affect a distributor's willingness or ability to complete its obligations under any arrangement; and
- these arrangements are often terminated or allowed to expire, which could interrupt the marketing and sales of a product and decrease our revenue.

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

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

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

- decreased demand for any product candidates or products that we may develop;
- damage to our reputation;
- regulatory investigations that could require costly recalls or product modifications;
- withdrawal of clinical trial participants;
- costs to defend the related litigation;
- substantial monetary awards to trial participants or patients, including awards that substantially exceed our product liability insurance, which we would then be required to pay from other sources, if available, and would damage our ability to obtain liability insurance at reasonable costs, or at all, in the future;
- loss of revenue;
- the diversion of management's attention from managing our business; and
- the inability to commercialize any products that we may develop.

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

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

The development and commercialization of new drugs is highly competitive and competition is expected to increase. We face competition with respect to our current product candidates and any products we may seek to develop or commercialize in the future from major pharmaceutical companies, specialty pharmaceutical companies and biotechnology companies worldwide. For example, several large pharmaceutical and biotechnology companies currently market and sell products for the treatment of Fabry disease. These products include Genzyme Corporation's Fabrazyme and Shire PLC's Replagal. In addition, Genzyme Corporation and

Actelion, Ltd. market and sell Cerezyme and Zavesca, respectively, for the treatment of Gaucher disease, and Genzyme Corporation markets and sells Myozyme for the treatment of Pompe disease. We are also aware of other enzyme replacement and substrate reduction therapies in development by third parties.

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

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

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

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

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

Risks Related to Our Dependence on Third Parties

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

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

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

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

- reliance on the third party for regulatory compliance and quality assurance;
- limitations on supply availability resulting from capacity and scheduling constraints of the third parties;
- impact on our reputation in the marketplace if manufacturers of our products, once commercialized, fail to meet the demands of our customers;
- the possible breach of the manufacturing agreement by the third party because of factors beyond our control; 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 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 certain preclinical development activities and our clinical trials and those third parties may not perform satisfactorily, including failing to meet established deadlines for the completion of such activities and trials.

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

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

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

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

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

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

- our collaboration agreements are likely to be for fixed terms and subject to termination by our collaborators in the event of a material breach or lack of scientific progress by us;
- our collaborators are likely to have the first right to maintain or defend our intellectual property rights and, although we would likely have the right to assume the maintenance and defense of our intellectual property rights if our collaborators do not, our ability to do so may be compromised by our collaborators' acts or omissions; and
- our collaborators may utilize our intellectual property rights in such a way as to invite litigation that could jeopardize or invalidate our intellectual property rights or expose us to potential liability.

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

Risks Related to Our Intellectual Property

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

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

to stop competitors from marketing similar products or reduce the term of patent protection we may have for our products. Changes in either patent laws or in interpretations of patent laws in the United States and other countries may diminish the value of our intellectual property or narrow the scope of our patent protection.

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

- we or our licensors were the first to make the inventions covered by each of our pending patent applications;
- we or our licensors were the first to file patent applications for these inventions;
- others will not independently develop similar or alternative technologies or duplicate any of our technologies;
- any patents issued to us or our licensors will provide a basis for commercially viable products, will provide us with any competitive advantages or will not be challenged by third parties;
- we will develop additional proprietary technologies that are patentable;
- we will file patent applications for new proprietary technologies promptly or at all;
- our patents will not expire prior to or shortly after commencing commercialization of a product; or
- the patents of others will not have a negative effect on our ability to do business.

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

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

- We do not hold composition of matter patents covering Amigal and AT2220, two of our three lead product candidates. Composition of matter patents can provide protection for pharmaceutical products to the extent that the specifically covered compositions are important. For our product candidates for which we do not hold composition of matter patents, competitors who obtain the requisite regulatory approval can offer products with the same composition as our products so long as the competitors do not infringe any method of use patents that we may hold.
- For some of our product candidates, the principal patent protection that covers, or 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 United States patent application covering our product candidates or a similar invention, we may have to participate in an adversarial proceeding, known as an interference, declared by the United States Patent and Trademark Office to determine priority of invention in the United States. The costs of these proceedings could be substantial and it is possible that our efforts could be unsuccessful, resulting in a loss of our United States patent position.

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

We are a party to a number of license agreements including agreements with the Mount Sinai School of Medicine of New York University, the University of Maryland, Baltimore County and Novo Nordisk A/S, pursuant to which we license key intellectual property relating to our lead product candidates. We expect to enter into additional licenses in the future. Under our existing licenses, we have the right to enforce the licensed patent rights. Our existing licenses impose, and we expect that future licenses will impose, various diligence, milestone payment, royalty, insurance and other obligations on us. If we fail to comply with these obligations, the licensor may have the right to terminate the license, in which event we might not be able to market any product that is covered by the licensed patents.

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

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

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

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

Further, if a patent infringement suit were brought against us, we or they could be forced to stop or delay research, development, manufacturing or sales of the product or product candidate that is the subject of the suit.

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

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

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

Others may sue us for infringing their patent or other intellectual property rights or file nullity, opposition or interference proceedings against our patents, even if such claims are without merit, which would similarly harm our business. For example, by letter dated April 10, 2007, we received a notice from a third party alleging trademark infringement in connection with our intended use of The NASDAQ Global Market ticker symbol "FOLD" for our common stock. Furthermore, during the course of litigation, confidential information may be disclosed in the form of documents or testimony in connection with discovery requests, depositions or trial testimony. Disclosure of our confidential information and our involvement in intellectual property litigation could materially adversely affect our business.

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

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

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

paying monetary damages, we may jeopardize valuable intellectual property rights, disclose confidential information or lose personnel.

Risks Related to Regulatory Approval of Our Product Candidates

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

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

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

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

- our failure to demonstrate to the satisfaction of the FDA or comparable regulatory authorities that a product candidate is safe and effective for a particular indication;
- the results of clinical trials may not meet the level of statistical significance required by the FDA or 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-United States regulatory authorities have substantial discretion in the approval process and may refuse to accept any application or may decide that our data is insufficient for approval and require additional preclinical, clinical or other studies. In addition, varying interpretations of the data obtained from preclinical and clinical testing could delay, limit or prevent regulatory approval of a product candidate. Any regulatory approval we ultimately obtain may be limited or subject to restrictions or post approval commitments that render the approved product not commercially viable. Any FDA or other regulatory approval of our product

candidates, once obtained, may be withdrawn, including for failure to comply with regulatory requirements or if clinical or manufacturing problems follow initial marketing.

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

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

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

- regulatory authorities may require the addition of restrictive labeling statements;
- regulatory authorities may withdraw their approval of the product; and
- we may be required to change the way the product is administered or conduct additional clinical trials.

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

We may not be able to obtain orphan drug exclusivity for our product candidates. If our competitors are able to obtain orphan drug exclusivity for their products that are the same drug as our product candidates, we 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 the active ingredient in Plicera 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 Plicera 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 Plicera may be important to each of the product candidate’s success. Even if we obtain orphan drug exclusivity for Amigal or Plicera for these indications, we may not be able to maintain it. For example, if a competitive product that is the same drug as our product candidate is shown to be clinically superior to our product candidate, any orphan drug exclusivity we have obtained will not block the approval of such competitive product and we may effectively lose what had previously been orphan drug exclusivity.

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

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

- restrictions on such products, manufacturers or manufacturing processes;
- warning letters;
- withdrawal of the products from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- voluntary or mandatory recall;
- 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;
- product seizure or detentions;
- injunctions or the imposition of civil or criminal penalties; and
- adverse publicity.

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

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

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

jurisdictions or by the FDA. We may not be able to file for regulatory approvals and may not receive necessary approvals to commercialize our products in any market.

Risks Related to Employee Matters and Managing Growth

Our future success depends on our ability to retain our chief executive officer and other key executives and to attract, retain and motivate qualified personnel.

We are highly dependent on John F. Crowley, our President and Chief Executive Officer, Matthew R. Patterson, our Chief Operating Officer, James E. Dentzer, our Chief Financial Officer, and David J. Lockhart, Ph.D., our Chief Scientific Officer. These executives each have significant pharmaceutical industry experience, including Mr. Crowley, with whom we have entered into an employment agreement that runs for successive one year terms until either we or Mr. Crowley elect to terminate the agreement. We may terminate Mr. Crowley's employment without cause at any time, or we may decide not to extend Mr. Crowley's agreement at the end of any term, or he may terminate his employment for good reason at any time, in each case subject to certain severance payments and benefits as described elsewhere in this prospectus. Mr. Crowley is a commissioned officer in the United States Navy (Reserve). The United States recently called Mr. Crowley to service, which he fulfilled, from September 11, 2006 to March 5, 2007, and he may be called to active duty service again at any time. The loss of Mr. Crowley for protracted military duty could materially adversely affect our business. We are also parties to employment agreements with each of Messrs. Patterson and Dentzer and Dr. Lockhart. These employment agreements each provide for an initial term of two years, and will continue thereafter for successive two-year periods until we provide the executive with written notice of the end of the agreement in accordance with its terms. We may terminate any of these executives without cause at any time, or one of these executives may quit for good reason within six months of the occurrence of certain corporate changes, in each case subject to certain severance payments and benefits as described elsewhere in this prospectus. The loss of the services of any of these executives might impede the achievement of our research, development and commercialization objectives and materially adversely affect our business. We do not maintain "key person" insurance on Mr. Crowley or on any of our other executive officers.

Recruiting and retaining qualified scientific personnel, clinical personnel and sales and marketing personnel will also be critical to our success. Our industry has experienced a high rate of turnover in recent years. We may not be able to attract and retain these personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar personnel, particularly in New Jersey and surrounding areas. Although we believe we offer competitive salaries and benefits, we may have to increase spending in order to retain personnel.

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

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

We are a development stage company with 76 full-time employees as of April 25, 2007. Of these employees, 53 work primarily in research and development and 23 provide administrative services. We expect to experience significant growth in the number of our employees and the scope of our operations, particularly in the areas of drug development, regulatory affairs and sales and marketing. Assuming our plans and business conditions progress consistent with our current projections, we plan to grow to a total of 90-100 employees by the end of 2007 and to a total of 100-120 employees by the end of 2008. To manage our anticipated future growth, we must continue to implement and improve our managerial, operational and financial systems, expand our facilities and continue to recruit and train additional qualified personnel. Due to our limited resources, we may not be able to effectively manage the expansion of our operations or recruit and train additional qualified personnel. The physical expansion of our operations may lead to significant costs and may

divert our management and business development resources. Any inability on the part of our management to manage growth could delay the execution of our business plans or disrupt our operations.

Risks Related to Our Common Stock 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 69.2% 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;
- 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.

The initial public offering price of our common stock is 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 the initial public offering price of $15.00 per share, you will experience immediate dilution of $9.15 per share, representing the difference between our pro forma as adjusted net tangible book value per share after giving effect to this offering and the initial public offering price. In addition, purchasers of common stock in this offering will have contributed approximately 33.2% of the aggregate price paid by all purchasers of our common stock but will own only approximately 22.5% 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 has been determined through negotiations with the underwriters. Although our common stock has been approved for quotation on The NASDAQ Global Market, an active trading market for our common stock may never develop or be sustained following its listing on The NASDAQ Global Market. 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.

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.

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 22,234,426 shares of common stock based on the number of shares outstanding as of April 25, 2007. Of these shares, 5,005,333 may be resold in the public market immediately and the remaining 17,229,093 shares are currently restricted under securities laws or as a result of lock-up agreements 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 16,570,855 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 1,366,667 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.

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,” “predict,” “project,” “will,” “would” and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.

The forward-looking statements in this prospectus include, among other things, statements about:

- our plans to develop and commercialize Amigal, Plicera and AT2220;
- our ongoing and planned discovery programs, preclinical studies and clinical trials;
- our ability to enter into selective collaboration arrangements;
- the timing of and our ability to obtain and maintain regulatory approvals for our product candidates;
- the rate and degree of market acceptance and clinical utility of our products;
- our ability to quickly and efficiently identify and develop product candidates;
- the extent to which our scientific approach may potentially address a broad range of diseases across multiple therapeutic areas;
- our commercialization, marketing and manufacturing capabilities and strategy;
- our intellectual property position; and
- our estimates regarding expenses, future revenues, capital requirements and needs for additional financing.

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

USE OF PROCEEDS

The net proceeds from the sale of 5,000,000 shares of common stock in this offering will be approximately $67.9 million, or $78.3 million if the underwriters exercise their over-allotment option in full, based on the initial public offering price of $15.00 per share, and after deducting the 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:

- approximately $20.0 million for clinical development of Amigal for the treatment of Fabry disease;
- approximately $20.0 million for clinical development of Plicera for the treatment of Gaucher disease;
- approximately $20.0 million for clinical development of AT2220 for the treatment of Pompe disease;
- approximately $5.0 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 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.

We expect that the net proceeds from this offering, along with our existing cash resources, will be sufficient to enable us to complete Phase III clinical trials of Amigal for the treatment of Fabry disease, initiate Phase III clinical trials of Plicera for the treatment of Gaucher Disease, and complete Phase II clinical trials of AT2220 for the treatment of Pompe Disease. We also believe that the funds from the offering will enable us to advance our preclinical studies of different pharmacological chaperones for the treatment of Parkinson’s disease and possibly other programs. As to our clinical programs, it is possible that we will not achieve the progress that we anticipate because the actual costs and timing of development are difficult to predict, are subject to substantial risks, and often vary depending on the particular indication and development strategy. As a result, we may need to raise additional funds from external sources to achieve the expected development progress described in this paragraph.

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.

CAPITALIZATION

The following table sets forth our capitalization as of March 31, 2007:

- on an actual basis;
- on a pro forma basis to give effect to elimination of our warrant liability of $672,418 and the automatic conversion of all shares of our redeemable convertible preferred stock into an aggregate of 16,071,924 shares of common stock outstanding upon the completion of this offering; and
- on a pro forma as adjusted basis to give further effect to our issuance and sale of 5,000,000 shares of common stock in this offering at the initial public offering price of $15.00 per share, after deducting underwriting discounts and commissions and estimated offering expenses payable by us.

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, 2007		
	Actual (unaudited)	Pro Forma (unaudited)	Pro Forma As Adjusted (unaudited)
		(in thousands)	
Capital lease obligations	$ 3,250	$ 3,250	$ 3,250
Series A redeemable convertible preferred stock, par value $0.01 per share; 444,443 shares authorized, issued and outstanding, actual; no shares authorized, issued or outstanding, pro forma and pro forma as adjusted	2,477	—	—
Series B redeemable convertible preferred stock, par value $0.01 per share; 4,936,730 shares authorized, actual, 4,877,056 shares issued and outstanding, actual; no shares authorized, issued or outstanding, pro forma and pro forma as adjusted	30,895	—	—
Series C redeemable convertible preferred stock, par value $0.01 per share; 5,820,020 shares authorized, issued and outstanding, actual; no shares authorized, issued or outstanding, pro forma and pro forma as adjusted	54,878	—	—
Series D redeemable convertible preferred stock, par value $0.01 per share; 4,930,405 shares authorized, issued and outstanding, actual; no shares authorized, issued or outstanding, pro forma and pro forma as adjusted	59,934	—	—
Stockholders' equity:			
Common stock, par value $0.01 per share; 21,333,333 shares authorized, actual and pro forma; 1,152,331 shares issued and outstanding, actual; 17,224,255 shares issued and outstanding, pro forma; 50,000,000 shares authorized and 22,224,255 shares issued and outstanding, pro forma as adjusted	82	1,288	1,338
Additional paid-in capital	6,981	153,959	221,774
Accumulated other comprehensive income	17	17	17
Deficit accumulated during the development stage	(93,362)	(92,690)	(92,690)
Total stockholders' (deficiency) equity	$ (86,282)	$ 62,574	$ 130,439
Total capitalization	$ 65,152	$ 65,824	$ 133,689

The table above does not include:

- 1,714,087 shares of common stock issuable upon exercise of options outstanding as of March 31, 2007 at a weighted average exercise price of $4.57 per share;
- 5,333 shares of common stock issuable upon exercise of a warrant to purchase common stock at an exercise price of $5.63 per share;
- 40,797 shares of common stock issuable in connection with the exercise of outstanding warrants to purchase shares of series B redeemable convertible preferred stock. Upon the closing of this offering, these warrants will be automatically exercised and the shares of series B redeemable convertible preferred stock automatically converted into shares of common stock on a one for one basis;
- an aggregate of 966,667 shares of common stock reserved for future issuance under our 2007 equity incentive plan as of the closing of this offering;
- an aggregate of 200,000 shares of common stock reserved for future issuance under our 2007 director option plan as of the closing of this offering; and
- an aggregate of 200,000 shares of common stock reserved for future issuance under our 2007 employee stock purchase plan as of the closing of this offering.

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, 2007 was approximately $(86.7) million or $(75.24) per share, based on 1,152,331 shares of common stock outstanding, as adjusted to reflect the 1-for-7.5 reverse split of our common stock and preferred stock effected on May 24, 2007. Historical net tangible book value per share represents the amount of our total tangible assets less total pro forma liabilities and redeemable convertible preferred stock, divided by the number of shares of common stock outstanding. Our pro forma net tangible book value as of March 31, 2007 was approximately $62.2 million, or $3.61 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 pro forma liabilities, divided by the pro forma number of shares of common stock outstanding after giving effect, as of March 31, 2007, to the elimination of our warrant liability of $672,418, and the automatic conversion of all shares of our redeemable convertible preferred stock into an aggregate of 16,071,924 shares of common stock outstanding upon completion of this offering.

After giving effect to our issuance and sale of 5,000,000 shares of our common stock in this offering at the initial public offering price of $15.00 per share, less the underwriting discounts and commissions and estimated offering expenses payable by us, our pro forma as adjusted net tangible book value as of March 31, 2007, would have been approximately $130.0 million, or $5.85 per share of our common stock. This represents an immediate increase in pro forma net tangible book value of $2.24 per share to our existing stockholders and an immediate dilution in pro forma net tangible book value of $9.15 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 as adjusted 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:

Initial public offering price per share		$15.00
Historical net tangible book value per shares as of March 31, 2007	(75.24)	
Increase attributable to the conversion of outstanding preferred stock	78.85	
Pro forma net tangible book value per share as of March 31, 2007	3.61	
Increase per share attributable to new investors	2.24	
Pro forma as adjusted net tangible book value per share after this offering		5.85
Dilution per share to new investors		$ 9.15

If the underwriters exercise their over-allotment option in full to purchase 750,000 additional shares of common stock in this offering, the proforma as adjusted net tangible book value per share after the offering would be $6.11 per share, the increase in net tangible book value per share to existing stockholders would be $0.26 per share and the dilution to new investors, in this offering would be $8.89 per share.

The following table sets forth, as of March 31, 2007, on a pro forma basis to give effect to the automatic conversion of all shares of our redeemable convertible preferred stock into an aggregate of 16,071,924 shares of common stock outstanding 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 the initial public offering price of $15.00 per share, before deducting underwriting discounts and commissions.

	Shares Purchased		Total Consideration		Average Price Per Share
	Number	Percent	Amount	Percent	
Existing stockholders	17,234,426	77.5%	150,791,267	66.8%	$ 8.75
New investors	5,000,000	22.5%	75,000,000	33.2%	15.00
Total	22,234,426	100.0%	225,791,267	100.0%	10.16

The discussion and tables above exclude:

- 1,714,087 shares of common stock issuable upon exercise of stock options outstanding as of March 31, 2007 at a weighted average exercise price of $4.57 per share;
- 5,333 shares of common stock issuable upon exercise of a warrant to purchase common stock at an exercise price of $5.63 per share;
- 40,797 shares of common stock issuable in connection with the exercise of outstanding warrants to purchase shares of series B redeemable convertible preferred stock. Upon the closing of this offering, these warrants will be automatically exercised and the shares of series B redeemable convertible preferred stock automatically converted into shares of common stock on a one for one basis;
- an aggregate of 966,667 shares of common stock reserved for future issuance under our 2007 equity incentive plan as of the closing of this offering;
- an aggregate of 200,000 shares of common stock reserved for future issuance under our 2007 director option plan as of the closing of this offering; and
- an aggregate of 200,000 shares of common stock reserved for future issuance under our 2007 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 75.0% 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 5,750,000, or approximately 25% of the total pro forma as adjusted number of shares of our common stock outstanding after this offering.

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, 2004, 2005 and 2006 and the balance sheet data at December 31, 2005 and 2006 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 year ended December 31, 2003 and the balance sheet data at December 31, 2002, 2003 and 2004, 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, 2006 and 2007, and for the period February 4, 2002 (inception) to March 31, 2007 and the balance sheet data at March 31, 2007 from our unaudited financial statements included in this prospectus. 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, 2007
		2003	2004	2005	2006	2006 (unaudited)	2007 (unaudited)	(unaudited)
	(in thousands, except shares and per share data)							
Statement of Operations Data:								
Operating expenses:								
Research and development	$ 788	$ 4,433	$ 6,301	$ 13,652	$ 33,630	$ 6,028	7,085	65,889
General and administrative	552	1,005	2,081	6,877	12,277	1,900	2,850	25,642
Impairment of leasehold improvements	—	1,030	—	—	—	—	—	1,030
Depreciation and amortization	24	132	146	303	952	199	297	1,854
In-process research and development	418	—	—	—	—	—	—	418
Total operating expenses	1,783	6,600	8,528	20,831	46,859	8,127	10,232	94,833
Loss from operations	(1,783)	(6,600)	(8,528)	(20,831)	(46,859)	(8,127)	(10,232)	(94,833)
Other income (expenses):								
Interest income	13	5	190	610	1,991	238	693	3,501
Interest expense	(6)	(172)	(550)	(82)	(273)	(52)	(92)	(1,175)
Change in fair value of warrant liability	—	—	(2)	(280)	(22)	(343)	(64)	(368)
Other expense	—	—	—	—	(1,182)	(3)	—	(1,182)
Loss before tax benefit	(1,776)	(6,768)	(8,890)	(20,584)	(46,345)	(8,287)	(9,695)	(94,057)
Income tax benefit	—	—	83	612	—	—	—	695
Net loss	(1,776)	(6,768)	(8,807)	(19,972)	(46,345)	(8,287)	(9,695)	(93,362)
Deemed dividend	—	—	—	—	(19,424)	—	—	(19,424)
Preferred stock accretion	(10)	(17)	(126)	(139)	(159)	(41)	(41)	(492)
Net loss attributable to common stockholders	$ (1,786)	$ (6,785)	$ (8,933)	$ (20,111)	$ (65,928)	$ (8,328)	$ (9,736)	(113,278)
Net loss attributable to common stockholders per common share – basic and diluted		$ (22.05)	$ (29.05)	$ (49.02)	$ (89.58)	$ (15.43)	$ (10.21)	
Weighted-average common shares outstanding – basic and diluted		307,539	307,539	410,220	735,967	539,789	953,959	
Unaudited pro forma net loss					$ (46,345)		$ (9,695)	
Unaudited pro forma basic and diluted net loss per share					$ (2.76)		$ (0.57)	
Unaudited shares used to compute pro forma basic and diluted net loss per share					16,807,933		17,025,885	

	As of December 31,					As of March 31,
	2002	2003	2004	2005	2006	2007
						(unaudited)
			(in thousands)			
Balance Sheet Data:						
Cash and cash equivalents and marketable securities	$ 1,341	$ 15	$ 4,336	$ 24,418	$ 54,699	$ 67,706
Working capital	947	(5,588)	3,569	22,267	44,814	59,526
Total assets	1,919	501	5,073	28,670	59,646	73,048
Total liabilities	752	5,776	1,346	4,031	13,071	11,146
Redeemable convertible preferred stock	2,416	2,432	20,013	60,469	124,091	148,184
Deficit accumulated during the development stage	(1,775)	(8,503)	(17,351)	(37,322)	(83,667)	(93,362)
Total stockholders' deficiency	$(1,249)	$(7,708)	$(16,287)	$(35,830)	$ (77,515)	$ (86,282)

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

Overview

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

We have generated significant losses to date and expect to continue to generate losses as we continue the clinical development of Amigal, Plicera, and AT2220. From our inception in February 2002 through March 31, 2007, we have accumulated a deficit of $93.4 million. Because we do not generate revenue from any of our product candidates, our losses will continue as we conduct our research and development activities. These activities are budgeted to expand over time and will require further resources if we are to be successful. As a result, our operating losses are likely to be substantial over the next several years. We will need to obtain additional funds to further develop our research and development programs.

Financial Operations Overview

Revenue

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

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;
- technology and intellectual property license costs;
- manufacturing development costs;
- personnel related expenses, including salaries, benefits, travel, and related costs for the personnel involved in drug discovery and development;
- activities relating to regulatory filings and the advancement of our product candidates through preclinical studies and clinical trials; and
- facilities and other allocated expenses, which include direct and allocated expenses for rent, facility maintenance, as well as laboratory and other supplies.

We have multiple research and development projects ongoing at any one time. We utilize our internal resources, employees and infrastructure across multiple projects. We do not believe that allocating internal costs on the basis of estimates of time spent by our employees would accurately 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, 2007, we have incurred research and development expense in the aggregate of $65.9 million, including stock-based compensation expense of approximately $2.3 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).

Product Candidate	Year Ended December 31,			Three Months Ended March 31,		Period from February 4, 2002 (Inception) to March 31,
	2004	2005	2006	2006	2007	2007
Third party direct project expenses						
Amigal (Fabry Disease — Phase II)	$4,547	$ 5,579	$ 3,361	$ 849	$ 591	$ 16,973
Plicera (Gaucher Disease — Phase II)	26	2,109	9,905	1,360	2,027	13,757
AT2220 (Pompe Disease — Phase I)	—	374	4,427	129	938	5,701
Total third party direct project expenses	4,573	8,062	17,693	2,338	3,556	36,431
Other project costs[1]						
Personnel costs	1,363	3,581	8,187	1,642	2,299	17,009
Other costs[2]	365	2,009	7,750	2,048	1,230	12,449
Total other project costs	1,728	5,590	15,937	3,690	3,529	29,458
Total research and development costs	$6,301	$13,652	$33,630	$ 6,028	$ 7,085	$ 65,889

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

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

The successful development of our product candidates is highly uncertain. At this time, we cannot reasonably estimate or know the nature, timing and costs of the efforts that will be necessary to complete the remainder of the development of, or the period, if any, in which material net cash inflows may commence from Amigal, Plicera, AT2220 or any of our other preclinical product candidates. This uncertainty is due to the numerous risks and uncertainties associated with the duration and cost of clinical trials which vary significantly over the life of a project as a result of differences arising during clinical development, including:

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

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

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

General and Administrative Expense

General and administrative expense consists primarily of salaries and other related costs, including stock-based compensation expense, for persons serving in our executive, finance, accounting, information technology and human resource functions. Other general and administrative expense includes facility-related costs not otherwise included in research and development expense, promotional expenses, costs associated with industry and trade shows, and professional fees for legal services, including patent-related expense, and accounting services. We expect that our general and administrative expenses will increase as we add personnel and become subject to the reporting obligations applicable to public companies. From our inception in February 2002 through March 31, 2007, we spent $25.6 million, including stock-based compensation expense of approximately $2.5 million, on general and administrative expense.

Beneficial Conversion Charges

When we issue debt or equity securities which are convertible into common stock at a discount from the common stock fair value at the date the debt or equity financing is committed, a beneficial conversion charge is measured as for the difference between the closing price and the conversion price at the commitment date. The beneficial conversion charge is presented as a discount or reduction to the related security, with an offsetting amount increasing additional paid-in capital. We recorded a beneficial conversion charge for a bridge loan financing of $0.1 million which was initially recorded as debt discount and amortized to interest expense through May 2004. We also recorded a beneficial conversion charge (deemed dividend) during April of 2006 of approximately $19.4 million related to the issuance of certain shares of series C redeemable convertible preferred stock. The beneficial conversion charge for our equity instruments is recorded with offsetting charges and credits to additional paid in capital with no effect on total shareholder equity. The beneficial conversion charge (deemed dividend) increases the loss applicable to our common stockholders in the calculation of basic net loss per share for the year ended December 31, 2006. The Series C investors committed to finance the second tranche of the series C redeemable convertible preferred stock on March 31, 2006. The estimated fair value of the common stock was approximately $16.13 per share at the commitment date of the second tranche and the beneficial conversion charge was recognized upon issuance of the series C redeemable convertible preferred stock as such stock could be converted upon issuance. We did not record a beneficial conversion charge for any other redeemable convertible preferred stock issuances as the common stock fair value was less than the conversion price of each offering on the respective commitment dates of those offerings.

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.

Other Income and Expenses

During the second and third quarter of 2006, we deferred and capitalized $1.2 million of costs directly attributable to the planned initial public offering of our common stock as other non-current assets. These costs were recorded as non-operating expenses when the planned offering was officially withdrawn during the third quarter of 2006.

Change in Warrant Liability

We account for warrants to purchase shares of our series B redeemable convertible preferred stock in accordance with FASB Staff Position 150-5: *Issuer's Accounting under FASB Statement No. 150 for Freestanding Warrants and Other Similar Instruments on Shares That Are Redeemable ("FSP150-5").* As the

Series B Preferred shares underling the warrants have redemption rights, the warrants to purchase Series B shares are classified as a liability. We recognize changes in the fair value of the warrants in the statements of operations as non-operating income or expense.

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 filing, we believe that the following accounting policies are the most critical to aid you in fully understanding and evaluating our financial condition and results of operations.

Accrued Expenses

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

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

Adoption of SFAS No. 123(R)

Effective January 1, 2006, we adopted the fair value recognition provisions of FASB Statement No. 123(R), *Share-Based Payment*, or SFAS No. 123(R), using the prospective transition method. Under the prospective transition method, compensation expense is recognized in the financial statements on a prospective basis for all share-based payments granted subsequent to December 31, 2005, based upon the grant-date fair value estimated in accordance with the provisions of SFAS 123(R). Options granted prior to January 1, 2006, as a non-public company and accounted for using the intrinsic value method, will continue to be expensed over the vesting period. The fair value of awards expected to vest, as measured at grant date, is expensed on a straight-line basis over the vesting period of the related awards. Under the prospective transition method, results for prior periods are not restated.

Stock-Based Compensation

At December 31, 2006 and March 31, 2007, 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 Accounting

Principles Board Opinion No 25, *Accounting for Stock Issued to Employees*, or APB 25, and related interpretations, as permitted by SFAS 123. Stock-based employee compensation cost was recognized in the statement of operations for periods prior to January 1, 2006, 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 stock-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 year ended December 31, 2006 was less than it would have been had we continued to account for stock-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.

We recognized employee stock-based compensation expense of $0.1 million, $0.4 million, $2.8 million, and $0.7 million for the years ended 2004, 2005, 2006, and the three month period ended March 31, 2007, respectively.

During the year ended December 31, 2006, we recorded incremental compensation expense of approximately $2.2 million ($2.99 per basic and diluted share) related to the expensing of our options under SFAS 123(R) during the year. The compensation expense had no impact on our cash flows from operations and financing activities. The total unrecognized compensation cost related to non-vested stock option awards as of December 31, 2006 was approximately $8.1 million. This expense will be recorded on a straight-line basis over approximately 2.7 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 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:

	Year Ended December 31, 2006	Three Months Ended March 31, 2006	Three Months Ended March 31, 2007
Expected stock price volatility	74.8%	72.7%	78.8%
Risk free interest rate	4.7%	4.6%	4.7%
Expected life of options (years)	6.25	6.25	6.25
Expected annual dividend per share	$ 0.00	$ 0.00	$ 0.00

The weighted-average fair value (as of the date of grant) of the options granted during the year ended December 31, 2006 and three months ended March 31, 2006 and 2007 was $10.20, $11.40, and $7.13, respectively.

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 board's 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 a public offering, we performed a retrospective determination of fair value for financial reporting purposes of our common stock underlying stock option grants in 2005 and the first quarter of 2006 utilizing a combination of valuation methods described in the AICPA *Technical Practice Aid, Valuation of Privately-Held-Company Equity Securities Issued as Compensation*, or the Practice Aid. We utilized the same combination of valuation methods to perform contemporaneous valuations of our common stock for each quarter subsequent to March 31, 2006. Information on stock option grants during 2005, 2006, and 2007 are 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	404,941	$ 0.68	$ 2.33	$ 1.65
June - July	235,838	0.68	5.78	5.10
August - September	42,071	1.65	7.13	5.48
October - November	313,477	5.33	8.55	3.23
December	13,934	5.33	10.80	5.48
	1,010,261			

Date of 2006 Issuance	Number of Options Granted	Average Exercise Price	Average Fair Value Estimate per Common Share	Average Intrinsic Value per Share
January - March	786,019	$ 5.33	$ 13.73(1)	$ 8.40
June	119,940	8.18	8.18	—
July - September	54,006	8.18	8.18	—
October - December	45,203	9.15	9.15	—
	1,005,168			

(1) Retrospectively determined fair value for financial reporting purposes.

Date of 2007 Issuance	Number of Options Granted	Average Exercise Price	Average Fair Value Estimate per Common Share	Average Intrinsic Value per Share
January - March	17,870	9.90	$ 9.90	$ —
April	856,292	13.43	13.43	—
	874,162			

Determining the fair value of the common stock of a private enterprise requires complex and subjective judgments. Our retrospective and contemporaneous estimates of enterprise value at each of the grant dates during 2005, 2006, and 2007 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, Plicera and AT2220. Estimated operating expenses were based on our internal assumptions, including continuing research and development activities for Amigal, Plicera, 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 and contemporaneous reviews, 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 retrospective review, the future outcomes included two scenarios: (i) we become a public company and; (ii) we remain a private company. In our contemporaneous review, the future outcomes included three scenarios: (i) we become a public company, (ii) we merge or are acquired by another company, and; (iii) 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 over time 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 while a decrease in that probability has the opposite effect on the value ascribed to our common stock.

For each of the 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 an IPO versus remaining a private company or completing a merger or acquisition; 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 the 2006 principally reflects increases resulting from achieving significant clinical milestones and a significant increase in our probability weighting for the IPO scenario until we withdrew our offering in the third quarter of 2006. The following is a summary of the significant factors that resulted in changes in the fair value of our common stock since January 2005:

- The reassessed fair value for financial reporting purposes of common stock underlying 404,941 options granted to employees during the period from January 2005 through May 2005 was $2.33 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 235,838 options granted to employees during the period from June 2005 through July 2005 was determined to be $5.78 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 due to progress made on our preclinical programs.
- The reassessed fair value for financial reporting purposes of common stock underlying 42,071 options granted to employees during the period from August 2005 through September 2005 was determined to be $7.13 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 313,477 options granted to employees during the period from October 2005 through November 2005 was determined to be $8.55 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 13,934 options granted to employees in December 2005 and 12,335 options granted to employees in the period from January 1, 2006 to February 22, 2006 was determined to be $10.80 per share. This increase was primarily based on preclinical development of Plicera and AT2220, as well as an acceleration of our IPO planning associated with early internal discussions regarding a potential IPO.
- The reassessed fair value for financial reporting purposes of common stock underlying 773,684 options granted to employees and directors in the period from February 28, 2006 to March 27, 2006 was determined to be $13.80 per share. This increase was primarily based on initial data from our Phase II studies in Fabry disease, leading to an increased probability of the IPO scenario in the PWER method and a further acceleration of our IPO timeline.
- The reassessed fair value for financial reporting purposes of common stock at March 31, 2006 was determined to be $16.13 per share. No options were granted on this date. This increase was primarily based on our board of director’s resolution to pursue an IPO and an increase in probability of the IPO scenario under the PWER method. During this timeframe, we believed that an IPO was imminent and that the common stock price was set at what we believed was 90% of the midpoint of the expected IPO price range.
- The fair value of common stock underlying 173,946 options granted to employees during the period from June to September of 2006 was determined to be $8.18 per share. This decrease was primarily the result of slower than anticipated enrollment in our Phase II clinical trials for Fabry and worsening market conditions as evidenced by the valuations of Biotech IPOs in the second quarter of 2006, the decline in the Nasdaq Biotechnology Index during the same period, and our extended delay and subsequent withdrawal of a planned IPO in 2006 which significantly reduced the probability of what we had previously believed to be an imminent IPO event.
- The fair value of common stock underlying 45,203 options granted to employees during the fourth quarter of 2006 was determined to be $9.15 per share. This increase was primarily based on a comparison to improved pre-money IPO values of biotechnology and emerging pharmaceutical companies at a similar stage of development to ours, an increased probability estimate for the IPO scenario under the PWER method subsequent to the completion of our Series D financing and an increase in the probability that we merge with or are acquired by another company.
- The fair value of common stock underlying 17,870 options granted to employees during the first quarter of 2007 was determined to be $9.90 per share. This increase was primarily based on an increase of the

probability estimate for the IPO scenario under the PWER method associated with the commencement of Phase I clinical trials for AT2220.

- The fair value of common stock underlying 856,292 options granted to employees during April of 2007 was determined to be $13.43 per share. This increase was primarily based on a significant increase of the probability estimate for the IPO scenario under the PWER method attributable to the completion of enrollment for our Phase II clinical trials for Amigal, data from our preclinical and Phase I clinical trials of Amigal, data from our preclinical and Phase I clinical trials from Plicera, and our board of directors resolution to pursue an IPO and file a Form S-1 with the SEC. In connection with the increase of the probability of the IPO scenario, the probability of a merger or acquisition occurring was reduced. During this timeframe, we believed that an IPO was imminent and that the common stock price was set at what we believed was 90% of the midpoint of the expected IPO price range.

The intrinsic value of all outstanding vested and unvested options based on the initial public offering price of $15.00 was $17.9 million based on 1,714,087 common stock options outstanding at March 31, 2007.

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

We calculated net loss per share in accordance with SFAS No. 128, *Earnings Per Share*. We have determined that the series A, B, C, and D redeemable convertible preferred stock represent participating securities in accordance with Emerging Issue Task Force, or EITF, 03-6 *Participating Securities and the Two — Class Method under FASB Statement No. 128*. However, since we operate at a loss, and losses are not allocated to the redeemable convertible preferred stock, the two class method does not affect our calculation of earnings per share. We had a net loss for all periods presented; accordingly, the inclusion of common stock options and warrants would be anti-dilutive. Therefore, the weighted average shares used to calculate both basic and diluted earnings per share are the same.

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

	Years Ended December 31,			Three Months Ended March 31, 2006	Three Months Ended March 31, 2007
	2004	2005	2006		
Historical					
Numerator:					
Net loss	$ (8,807,102)	$ (19,972,289)	$ (46,344,910)	$ (8,287,253)	$ (9,694,939)
Deemed dividend	—	—	(19,424,367)	—	—
Accretion of redeemable convertible preferred stock	(125,733)	(138,743)	(158,802)	(40,611)	(40,988)
Net loss attributable to common stockholders	$ (8,932,835)	$ (20,111,032)	$ (65,928,079)	$ (8,327,864)	$ (9,735,927)
Denominator:					
Weighted average common shares outstanding — basic and diluted	307,539	410,220	735,967	539,789	953,959

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 3,833,306, 9,459,737, 16,530,450 and 18,345,127 for the years ended December 31, 2004, 2005, 2006 and for the three months ended March 31, 2007, respectively. Potentially dilutive common stock equivalents were excluded from the diluted earnings per share denominator for all periods because of their anti-dilutive effect.

Results of Operations

Three Months Ended March 31, 2007 Compared to Three Months Ended March 31, 2006

Research and Development Expense. Research and development expense was $7.1 million for the three months ended March 31, 2007 representing an increase of $1.1 million, or 18%, from $6.0 million for the three months ended March 31, 2006. The increase was primarily attributable to third party direct project expenses, including a rise in contract research and manufacturing costs of $1.1 million due to our continued development of Plicera and AT2220, increases in personnel costs of $0.7 million associated with headcount growth, partially offset by reductions in other costs such as consulting and non-program specific research.

General and Administrative Expense. General and administrative expense was $2.8 million for the three months ended March 31, 2007, an increase of $0.9 million, or 47.4%, from $1.9 million from the three months ended March 31, 2006. The increase resulted from an increase of personnel costs of $0.9 million attributable to increased headcount in finance, information technology, human resources, and general management.

Interest Income and Interest Expense. Interest income was $0.7 million in the three months ended March 31, 2007, compared to $0.2 million in the three months ended March 31, 2006. Interest expense was $0.1 million in the three months ended March 31, 2007, compared to $0.1 million in the three months ended March 31, 2006. The increase in interest income resulted from higher average cash and cash equivalent balances and higher average interest rates.

Year Ended December 31, 2006 Compared to Year Ended December 31, 2005

Research and Development Expense. Research and development expense was $33.6 million in 2006, an increase of $19.9 million, or 145%, from $13.7 million in 2005. The increase was primarily attributable to third party direct project expenses, including increased contract research and manufacturing costs for Plicera and AT2220 of $9.6 million, an increase in personnel costs of $4.6 million associated with headcount and salary increases in our research, clinical, and regulatory functions and the impact of adopting SFAS 123(R), and other costs associated with licenses totaling $2.5 million as well as higher facility, supply, overhead, and non-program specific research.

General and Administrative Expense. General and administrative expense was $12.3 million in 2006, an increase of $5.4 million, or 78%, from $6.9 million in 2005. The increase resulted principally from an increase in personnel costs of $3.7 million attributable to increased headcount, a rise in salaries, and the impact of adopting SFAS 123(R).

Depreciation and Amortization. Depreciation and amortization expense was $1.0 million in 2006, an increase of $0.7 million or 233%, from $0.3 million in 2005. The increase is primarily due to leasehold improvements completed in late 2005 and early 2006 as well as purchases of equipment during 2006.

Interest Income and Interest Expense. Interest income was $2.0 million in 2006, compared to $0.6 million in 2005. The increase in interest income resulted from higher average cash and cash equivalents balances and higher average interest rates in 2006. Interest expense was $0.3 million in 2006, compared to $0.1 million in 2005. The increase in interest expense resulted from additional capital lease borrowings during 2006.

Other Expense. During 2006, we capitalized $1.2 million of costs directly attributable to the planned offering of our anticipated IPO. These costs were expensed when we withdrew our offering in the third quarter of 2006.

Tax Benefit. In 2005, we recognized tax benefits related to our sale of net operating losses in the New Jersey Tax Transfer Program. Our tax benefit was $0.6 million in 2005. We sold $6.7 million of net operating losses in 2005. We did not sell net operating losses in the New Jersey Tax Transfer Program in 2006 and therefore we did not recognize any tax benefits in 2006.

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, Plicera, and AT2220 of $3.5 million during 2005, and a rise in personnel costs of $2.2 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 $0.6 million in 2005, compared to $0.2 million in 2004. Interest expense was $0.1 million in 2005, compared to $0.6 million 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 $0.6 million in 2005 and $0.1 million in 2004. We sold $6.7 million and $1.1 million of net operating losses in 2005 and 2004, respectively.

Liquidity and Capital Resources

Source of Liquidity

As a result of our significant research and development expenditures and the lack of any approved products to generate product sales revenue, we have not been profitable and have generated operating losses since we were incorporated in 2002. We have funded our operations principally with $148.6 million of gross proceeds from redeemable convertible preferred stock offerings through March 31, 2007. The following table summarizes our funding sources as of March 31, 2007:

Issue	Year	No. Shares	Approximate Amount(1)
Series A Redeemable Convertible Preferred Stock	2002	444,443	$ 2,500,000
Series B Redeemable Convertible Preferred Stock	2004, 2005, 2006	4,877,056	31,091,307
Series C Redeemable Convertible Preferred Stock	2005, 2006	5,820,020	54,999,332
Series D Redeemable Convertible Preferred Stock	2006, 2007	4,930,405	59,999,999
		16,071,924	$ 148,590,638

(1) Represents gross proceeds.

As of March 31, 2007, we had cash and cash equivalents and marketable securities of $67.7 million. We hold our cash and investment balances in a variety of high quality interest-bearing instruments, including obligations of U.S. government agencies and money market accounts. We invest cash in excess of our immediate requirements with regard to liquidity and capital preservation. Wherever possible, we seek to minimize the potential effects of concentration and degrees of risk.

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

Net Cash Used in Operating Activities

Net cash used in operations was $33.9 million for the year ended December 31, 2006. The net loss for the year ended December 31, 2006 of $46.3 million was offset primarily by non-cash charges for depreciation and amortization of $1.0 million, stock-based compensation of $3.3 million, stock-based license payment of $1.2 million and changes in operating assets and liabilities of $7.0 million.

Net cash used in operations was $6.1 million and $10.7 million for the three months ended March 31, 2006 and 2007. The net loss for the three months ended March 31, 2007 of $9.7 million was offset primarily

by non-cash charges for depreciation and amortization of $0.3 million, stock-based compensation expense of $0.8 million offset by changes in operating assets and liabilities of $2.2 million.

Net Cash Used in Investing Activities

Net cash used in investing activities was $26.6 million for the year ended December 31, 2006. Net cash used in investing activities reflects $62.0 million for the purchase of marketable securities and $2.0 million for the acquisition of property and equipment, partially offset by $37.4 million for the sale and redemption of marketable securities.

Net cash used in investing activities for the three months ended March 31, 2007 was $5.5 million and consisted primarily of $21.6 million from the sale and redemption of marketable securities and $26.8 million of purchases of marketable securities.

Net Cash Provided by Financing Activities

Net cash provided by financing activities was $66.2 million for the year ended December 31, 2006. Net cash provided by financing activities mainly reflects $27.5 million of proceeds from the issuance of our series C redeemable convertible preferred stock, $35.9 million of proceeds from the issuance of our series D redeemable convertible preferred stock, and $3.4 million of proceeds from our capital asset financing arrangement, partially offset by $0.9 million of payments of capital lease obligations.

Net cash provided by financing activities for the three months ended March 31, 2007 was $23.9 million, consisting primarily of $24.1 million from issuance of preferred stock and $0.2 million proceeds from exercise of stock options offset by payments of equipment debt financing obligations of $0.3 million.

Funding Requirements

We expect to incur losses from operations for the foreseeable future. We expect to incur increasing research and development expenses, including expenses related to the hiring of personnel and additional clinical trials. We expect that our general and administrative expenses will also increase as we expand our finance and administrative staff, add infrastructure, and incur additional costs related to being a public company, including directors' and officers' insurance, investor relations programs, and increased professional fees. Our future capital requirements will depend on a number of factors, including the continued progress of our research and development of products, the 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 early 2010. We have based this estimate on assumptions that may prove to be wrong, and we could use our available capital resources sooner than we currently expect. Because of the numerous risks and uncertainties associated with the development and commercialization of our product candidates, we are unable to estimate the amounts of increased capital outlays and operating expenditures associated with our current and anticipated clinical trials.

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.

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.

Financial Uncertainties Related to Potential Future Milestone Payments

We have acquired rights to develop and commercialize our product candidates through licenses granted by various parties. Two of these agreements contain milestone payments that are due with respect to Plicera only if certain specified pre-commercialization events occur. Our other products currently under development do not trigger such milestone payments. Upon the satisfaction of certain milestones and assuming successful development of Plicera, we may be obligated, under the agreements that we have in place, to make future milestone payments aggregating up to approximately $7.9 million. In general, potential milestone payments for Plicera may or may not be triggered under these licenses, and may vary in size, depending on a number of variables, almost all of which are currently uncertain.

The events that trigger these payments include:

- completion of Phase II clinical trials;
- commencement of Phase III clinical trials;
- submission of an NDA to the FDA or foreign equivalents; and
- receipt of marketing approval from the FDA or foreign equivalents.

Under our license agreements, if we owe royalties on net sales for one of our products to more than one of the above licensors, then we have the right to reduce the royalties owed to one licensor for royalties paid to another. The amount of royalties to be offset is generally limited in each license and can vary under each agreement. For Amigal and AT2220, we will owe royalties only to Mt. Sinai School of Medicine. We expect to pay royalties to all three licensors with respect to Plicera. To date, we have not made any royalty payments on sales of our products and believe we are several years away from selling any products that would require us to make any such royalty payments. Whether we will be obligated to make milestone or royalty payments in the future is subject to the success of our product development efforts and, accordingly, is inherently uncertain.

Contractual Obligations

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

	Total	Less than 1 Year	1-3 Years	3-5 Years	Over 5 Years
Operating lease obligations	$ 7,631,820	$ 1,629,181	$ 4,477,324	$ 1,525,315	—
Capital lease obligations	4,113,425	1,624,727	2,488,698	—	—
Employment agreement	1,850,669	1,388,002	462,667	—	—
Total fixed contractual obligations(1)	$ 13,595,914	$ 4,641,910	$ 7,428,689	$ 1,525,315	—

(1) This table does not include (a) any milestone payments which may become payable to third parties under license agreements as the timing and likelihood of such payments are not known, (b) any royalty payments to third parties as the amounts of such payments, timing and/or the likelihood of such payments are not known, (c) amounts, if any, that may be committed in the future to construct additional facilities, and (d) contracts that are entered into in the ordinary course of business which are not material in the aggregate in any period presented above.

In May 2005, we entered into a seven-year, non-cancelable operating sublease agreement for office and laboratory space in Cranbury, New Jersey. The operating sublease will expire by its terms in February 2012. In August 2006, we entered into a sublease agreement for office space in an adjacent building. This sublease will expire by its terms in August 2009.

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 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 5,333 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.

On April 28, 2006, we entered into an employment agreement with our president and chief executive officer that provides for an annual base salary of $400,000, a cash bonus of up to 50% of base salary, an executive medical reimbursement contract, annual reimbursement up to $220,000 for medical expenses not covered by the executive medical reimbursement contract or our medical or health insurance policies, and gross up for federal and state income taxes of income tax incurred in connection with medical reimbursement. The agreement will continue for successive one-year terms until either party provides written notice of termination to the other in accordance with the terms of the agreement. The table above includes costs associated with the remainder of the first one-year term and second one-year term ending April 28, 2008. The cost of the executive medical reimbursement contract is estimated based on current premiums. This employment agreement is more fully described in the Compensation Discussion and Analysis section of this prospectus.

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, Plicera and 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.

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 2004, 2005, 2006 or through March 31, 2007.

Off-Balance Sheet Arrangements

We have no off-balance sheet arrangements as of December 31, 2005 and 2006 or March 31, 2007.

Recent Accounting Pronouncements

In July 2006, FASB issued FSAB Interpretation No. 48, *Accounting for Uncertainty in Income Taxes — an Interpretation of FASB Statement No. 109*, or FIN No. 48, which clarifies the accounting for uncertainty in tax positions. This Interpretation prescribes a recognition threshold and measurement attribute for the financial statement recognition and measurement of a tax position taken or expected to be taken in a tax return. It also provides guidance on derecognition, clarification, interest and penalties, accounting in interim periods, disclosures and transitions. The provision of FIN 48 are effective as of the beginning of our 2007 fiscal year, with the cumulative effect, if any, of the change in accounting principle recorded as an adjustment to opening retained earnings. FIN 48 was adopted on January 1, 2007 and did not impact our financial statements.

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

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, 2007, we had cash and cash equivalents and investments in marketable securities of $67.7 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.

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 products in development are Amigal for Fabry disease, Plicera for Gaucher disease and AT2220 for Pompe disease. We have completed enrollment of our Phase II clinical trials of Amigal, and are currently conducting Phase II clinical trials of Plicera and Phase I clinical trials of AT2220. 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 five approved therapeutics to treat Fabry, Gaucher and Pompe disease were more than $1.5 billion in 2006, as publicly reported by companies that market these therapeutics. We hold worldwide commercialization rights to Amigal, Plicera 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 such mutations may not fold into their correct three-dimensional shape and are generally referred to as misfolded proteins. Misfolded proteins are often recognized by cells as having defects and, as a result, may be eliminated prior to reaching their intended location in the cell. The reduced biological activity of these proteins leads to impaired cellular function and ultimately to disease.

Our novel approach to the treatment of human genetic diseases consists of using pharmacological chaperones that selectively bind to the target protein, increasing the stability of the protein and helping it fold into the correct three-dimensional shape. This allows proper trafficking of the protein, thereby increasing protein activity, improving cellular function and potentially reducing cell stress.

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 of recombinant enzyme. 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 and ease of use, potentially improving treatment of these diseases.

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 diseases. Our initial clinical efforts are currently focused on developing pharmacological chaperones for the treatment of lysosomal storage disorders, which are chronic genetic diseases, such as Fabry, Gaucher and Pompe, that frequently result in severe symptoms. We believe our technology also is broadly applicable to other diseases for which protein stabilization and improved folding may be beneficial, 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. Each of these disorders results from the deficiency of a single enzyme.

- *Amigal for Fabry disease.* We are developing Amigal for the treatment of Fabry disease and are currently conducting multiple Phase II clinical trials of Amigal. We expect to complete these trials by the end of 2007.
- *Plicera for Gaucher disease.* We are developing Plicera for the treatment of Gaucher disease and are currently conducting two Phase II clinical trials of Plicera in Type I Gaucher patients. We expect to obtain preliminary results from the first of these two trials by the end of 2007.

- *AT2220 for Pompe disease.* We are developing AT2220 for the treatment of Pompe disease, and are currently conducting Phase I clinical trials of AT2220. We expect to initiate a Phase II clinical trial of AT2220 by the end of 2007.

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 that cause changes in the amino acid sequence of a protein which reduce its stability and may prevent it from folding properly. The majority of genetic mutations that lead to the production of less stable or 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 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 before 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. 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, improving cellular function and potentially reducing cell stress.

Pharmacological chaperones represent a new way of increasing the levels of specific proteins to improve cellular function and treat disease. Our proprietary approach to the discovery of pharmacological chaperone drug candidates involves the use of rapid molecular and cell-based screening methods combined with our understanding of the intended biological function of proteins implicated in disease. We use this knowledge to select and develop compounds with desirable 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 our technology is broadly applicable to other diseases for which protein stabilization and improved folding may be beneficial, including certain types of neurological disease, metabolic disease, cardiovascular disease and cancer. 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.

The current therapeutic standard of care for the most common lysosomal storage disorders is enzyme replacement therapy. Enzyme replacement therapy involves regular infusions of recombinant human enzyme to compensate for the deficient lysosomal enzyme. We believe that pharmacological chaperone therapy may have

advantages relative to enzyme replacement therapy for the treatment of lysosomal storage disorders. The following table compares some features of enzyme replacement therapy to pharmacological chaperone therapy.

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

An additional therapeutic approach to the treatment of certain lysosomal storage disorders is called substrate reduction therapy. 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, if synthesis of the substrate is inhibited it cannot perform its normal biological functions. Additionally, the enzyme that is inhibited is needed to make other molecules that are used in other biological processes. As a result, inhibiting this enzyme may have adverse effects that are difficult to predict. By contrast, our pharmacological chaperones are designed to bind 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
Plicera	Gaucher Disease	Phase II	Amicus
AT2220	Pompe Disease	Phase I	Amicus

Amigal for Fabry Disease

Overview

Our most advanced product candidate, Amigal, is an orally-administered, small molecule pharmacological chaperone for the treatment of Fabry disease. We have completed enrollment of our four Phase II clinical trials of Amigal and have obtained initial results for the first eleven patients who have completed at least 12 weeks of treatment. Each of these patients has been treated with various doses and regimens of Amigal for various periods of time in accordance with the Phase II protocols. Amigal has been well-tolerated to date with no reported drug-related serious adverse events.

The eleven patients represent ten different genetic mutations and have baseline levels of α-GAL in white blood cells of between 0% and 30% of normal. An increase in α-GAL enzyme levels in white blood cells has been observed in ten out of the eleven patients. These initial results suggest that treatment with Amigal causes an increase in the level of α-galactosidase A, or α-GAL, the enzyme deficient in Fabry disease, in a wide range of Fabry patients. In addition, we believe that this increase is likely to be therapeutically meaningful because it is generally believed that even small increases in lysosomal enzyme levels may have clinical benefits.

Globotriaosylceramide, or GL-3, the lipid substrate broken down by α-GAL in the lysosome, accumulates in the cells of patients with Fabry disease and is believed to be the cause of the majority of disease symptoms. Reduction of the level of GL-3 in a specific cell type of the kidney was the basis of prior regulatory approval by the FDA of an enzyme replacement therapy for the treatment of Fabry disease. Kidney GL-3 levels are available for two patients treated in our Phase II clinical trials and were assessed by a blinded independent expert using light and electron microscopy. A decrease of GL-3 levels was observed in multiple cell types of the kidney of one patient after 12 weeks of treatment. A second patient also showed a decrease of GL-3 levels in these same kidney cell types after 24 weeks of treatment, but these decreases were not independently conclusive because of the patient's lower levels of GL-3 at baseline. An increase in the level of α-GAL in white blood cells was observed in both of these two patients after treatment with Amigal. A third patient showed an increase in GL-3 levels in some cell types of the kidney and no change or a decrease in others after 12 weeks of treatment. Of the eleven patients who have completed at least 12 weeks of treatments to date in our ongoing clinical trials, this is the one patient who did not show an increase in the level of α-GAL in white blood cells after treatment with Amigal.

We expect to complete our Phase II clinical trials of Amigal by the end of 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 α-GAL. Symptoms can be severe and debilitating, including kidney failure and increased risk of heart attack and stroke. The deficiency of α-GAL in Fabry patients is caused by inherited genetic mutations. Certain of these mutations cause changes in the amino acid sequence of α-GAL that may result in the production of α-GAL with reduced stability that does not fold into its correct three-dimensional shape. Although α-GAL produced in patient cells often retains the potential for some level of biological activity, the cell's quality control mechanisms recognize and retain misfolded α-GAL in the endoplasmic reticulum, or ER, until it is ultimately moved to another part of the cell for degradation and elimination. Consequently, little or no α-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 be the cause of the symptoms of Fabry disease. In addition, accumulation of the misfolded α-GAL enzyme in the ER may lead to stress on cells and inflammatory-like responses, which may contribute to cellular dysfunction and disease.

Amigal is designed to act as a pharmacological chaperone for α-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 α-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 α-GAL from the ER to the lysosome, Amigal also 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 increases levels of a patient's naturally produced α-GAL, those Fabry disease patients with a missense mutation or other genetic mutations that result in production of α-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 α-GAL enzyme or α-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 spectrum of severity and roughly correlate with a patient's residual α-GAL levels. 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 males and females that have a range of Fabry disease symptoms, such as impaired cardiac or renal function and strokes, that usually first appear in adulthood. Individuals with this type of Fabry disease, referred to as later-onset Fabry disease, tend to have higher residual α-GAL levels than classic Fabry disease patients. Although the symptoms of Fabry disease span a spectrum of severity, it is useful to classify patients as having classic or later-onset Fabry disease when discussing the disease and the associated treatable population.

Classic Fabry Disease

Individuals with classic Fabry disease are in most instances males. They have little or no detectable α-GAL levels 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 eye lenses. 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

Individuals with later-onset Fabry disease can be male or female. They 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 and Journal of the American Heart Association (March 2002 and August 2004), estimated that 6-12% of patients between 40 and 60 years of age with 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 Dialysis Transplant (2003), Clinical and Experimental Nephrology (2005) and Nephrology Clinical Practice (2005) estimate that 0.20% to 0.94% of 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 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 with stroke of unknown cause have Fabry disease.

It was previously believed to be rare for female Fabry disease patients to develop overt clinical manifestations of Fabry disease. Fabry disease is known as an X-linked disease because the inherited α-GAL gene mutation is located only on the X chromosome. 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 females as males that have Fabry disease mutations. Recently, 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 Genetics and Genomic Sciences, report that, while the majority of females with Fabry disease mutations have mild symptoms, many have severe symptoms, including enlargement of the left ventricle of the heart and/or renal failure.

In a recent study reported in the American Journal of Human Genetics, more than thirty-seven thousand newborn males in Italy were screened for α-GAL activity and mutations. The incidence of Fabry mutations in this study was 1:3100, over ten times higher than previous estimates. This high incidence was attributed to a large number of newborn males with α-GAL mutations often associated with later-onset Fabry disease, which may not have been identified in previous screening studies that relied on diagnosis based on development of symptoms of classic Fabry disease.

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.

We expect that as awareness of later-onset Fabry disease grows, the number of patients diagnosed with the 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 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 specialist physicians, we intend to provide support for testing for the disease, which is performed using a simple blood test for the level of α-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 approximate 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) suggest 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 α-GAL with some residual enzyme activity, we believe patients with these mutations may benefit from treatment with Amigal. We also believe that other types of genetic mutations may result in misfolded α-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

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 2006 were approximately $359 million and $118 million, respectively, as publicly reported by Genzyme Corporation and Shire PLC, respectively.

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 and other symptoms, and beta-blockers, calcium channel blockers, ACE inhibitors, angiotensin receptor antagonists and other agents to treat blood pressure and vascular disease.

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 multiple cell types in the skin, heart and kidney. However, because they are large protein molecules, Fabrazyme and Replagal are believed to have difficulty penetrating some 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 intravenous infusions. See "Potential Advantages of Pharmacological Chaperones in the Treatment of Lysosomal Storage Disorders".

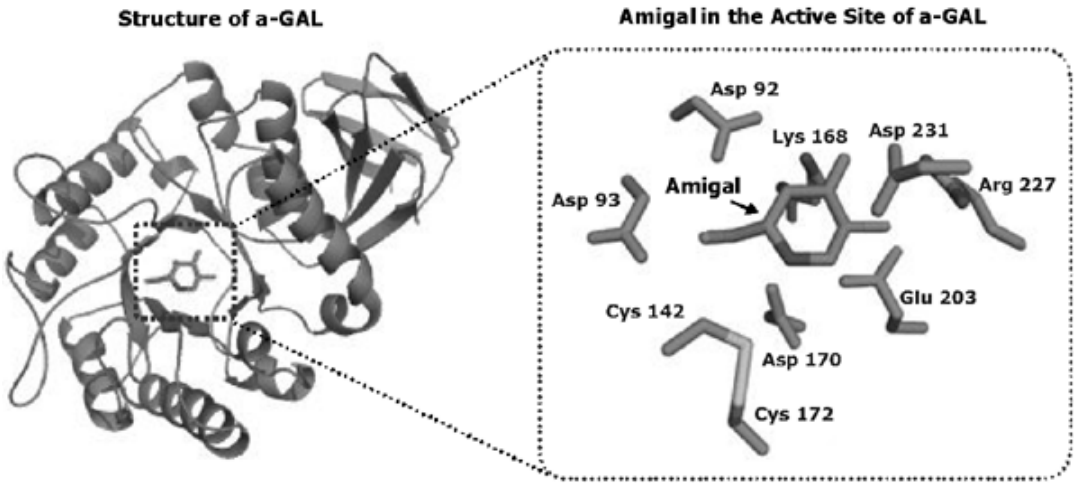
In February 2004, Amigal was granted orphan drug designation by the FDA for the treatment of Fabry disease and in March 2006 the EMEA recommended orphan medicinal product designation for Amigal. We believe that orphan drug designation of Fabrazyme in the United States and of Fabrazyme and Replagal in 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 completed experiments in collaboration with researchers in the field to better understand the mechanism of action of Amigal. In one experiment we crystallized α-GAL both alone and with Amigal. These data demonstrate that Amigal binds directly to the active site of α-GAL. See Figure 1 below.

Figure 1: Crystal Structure of α-GAL with Amigal



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

- Amigal increased α-GAL enzyme levels in cells derived from a variety of different Fabry disease patients. Over 60 different α-GAL missense mutations have been examined in cell culture assays with approximately 65% showing an increase in α-GAL enzyme levels after incubation with Amigal for several days.
- Treatment of normal mice and mice that produce a form of human α-GAL resulted in a dose-dependent increase in α-GAL enzyme levels in a variety of tissues including skin, liver, heart, kidney and spleen.
- Treatment of mice that produce a form of human α-GAL resulted in both an increase of α-GAL enzyme levels and a decrease in GL-3 levels in skin, heart and kidney.

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 effects 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. We are currently planning additional reproductive toxicity and carcinogenicity studies with Amigal in accordance with standard regulatory guidelines.

Phase I Clinical Trials

We have completed Phase I clinical trials of Amigal in a total of 48 healthy volunteers, of which 36 were treated with Amigal and 12 were given placebo.

- *Single Dose Phase I Trial.* Our single-dose Phase I trial 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 placebo or 25 mg, 75 mg, 225 mg or 675 mg of Amigal and were evaluated on Day 1 and on Day 8. The objectives of the study were to evaluate the safety and pharmacokinetics of Amigal in healthy volunteers.
- *Multiple-Dose Phase I Trial.* Our multiple-dose Phase I trial 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 placebo or 50 mg twice a day for seven days, and all subjects in the other group received placebo or 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 objectives of the study were to evaluate the safety and pharmacokinetics of Amigal in healthy volunteers and to measure α-GAL enzyme levels in white blood cells of healthy volunteers treated with Amigal.

The data from our Phase I clinical trials in healthy volunteers showed that Amigal was generally safe and well tolerated at all doses. There were no serious adverse events and no subjects withdrew or discontinued due to an adverse event. The studies also demonstrate that Amigal has high oral bioavailability with a terminal half-life in plasma of approximately three to four hours.

In addition, the data from the multiple-dose Phase I trial showed a dose-related increase in the level of α-GAL in the white blood cells of healthy volunteers administered Amigal for seven days. At the highest dose level there was approximately a 2-fold increase in levels of α-GAL, and this increase was maintained for at least seven days after the last dose. We believe normal enzyme levels can be increased because some fraction of normal protein molecules can also misfold and fail to pass the cell's quality control mechanisms. Normal α-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 levels following discontinuation of treatment occurs because the enzyme is stable for many days once it reaches the lysosome.

We believe these Phase I results are the first demonstration of an increase in enzyme levels in humans following oral administration of a pharmacological chaperone.

Phase II Clinical Trials

We have completed enrollment of our four open-label Phase II clinical trials of Amigal with a target aggregate enrollment for all four trials of between 20 and 25 patients, and have obtained initial results for the first eleven patients who have completed at least 12 weeks of treatment. These studies were open to male and female patients with all forms of Fabry disease, including both classic and later-onset Fabry disease.

In order to qualify for these clinical studies, patients must have a confirmed diagnosis of Fabry disease with a documented missense mutation in α-GAL and a positive result in either an in vitro or in vivo test of the effect of Amigal on α-GAL enzyme levels. The 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 α-GAL enzyme activity. The in vivo test involves measuring α-GAL enzyme activity from white blood cells before and after 2 weeks of treatment to assess response. For entry into the Phase II clinical trials, enzyme activity from a patient's white blood cells must show a relative increase of at least 20% to 100% after treatment in the in vitro or in vivo screen, depending on the amount of baseline α-GAL activity.

We have four ongoing Phase II clinical trials.

- *Phase II Study 201.* Nine patients have been treated in this study. Enrollment is complete and the study is expected to be finished by the end of 2007. The study consists of treatment with Amigal for a period of twelve weeks with a possible extension up to 48 weeks in male Fabry disease patients that are naïve to enzyme replacement therapy or have not had enzyme replacement therapy for at least one month. Eight patients received 25 mg of Amigal twice a day for two weeks, followed by 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. Patients participating in the extension portion of the study are receiving 50 mg of Amigal once per day and are expected to receive 150 mg of Amigal every other day after a planned protocol amendment is completed.
- *Phase II Study 202.* Three patients have been treated in this study. A fourth patient has completed screening and is expected to begin treatment in May 2007. Enrollment is complete and the study is expected to be finished by the end of 2007. The study consists of treatment with Amigal for a period of 12 weeks with a possible extension to 48 weeks in male Fabry disease patients that are naïve to enzyme replacement therapy or have not had enzyme replacement therapy for at least one month. All patients will receive 150 mg of Amigal every other day during the duration of the study.
- *Phase II Study 203.* Five patients have been treated in this study. Enrollment is complete and the study is expected to be finished by the end of 2007. The study consists of treatment with Amigal for a period of 24 weeks with a possible extension to 48 weeks in male Fabry disease patients that are naïve to enzyme replacement therapy or have not had enzyme replacement therapy for at least one month. All patients will receive 150 mg of Amigal every other day during the duration of the study.
- *Phase II Study 204.* Seven patients have been treated in this study. Two additional patients have completed screening and are expected to begin treatment in May 2007. Enrollment is complete and the study is expected to be finished by the end of 2007. The study consists of treatment with Amigal for a period of 12 weeks with a possible extension to 48 weeks in female Fabry disease patients that are naïve to enzyme replacement therapy or have not had enzyme replacement therapy for at least one month. 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 during the extension will be determined based on data from the first 12 weeks.

The primary objective of the Phase II clinical trials 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 including effects on α-GAL activity and GL-3 levels. GL-3 levels are measured from skin biopsies,

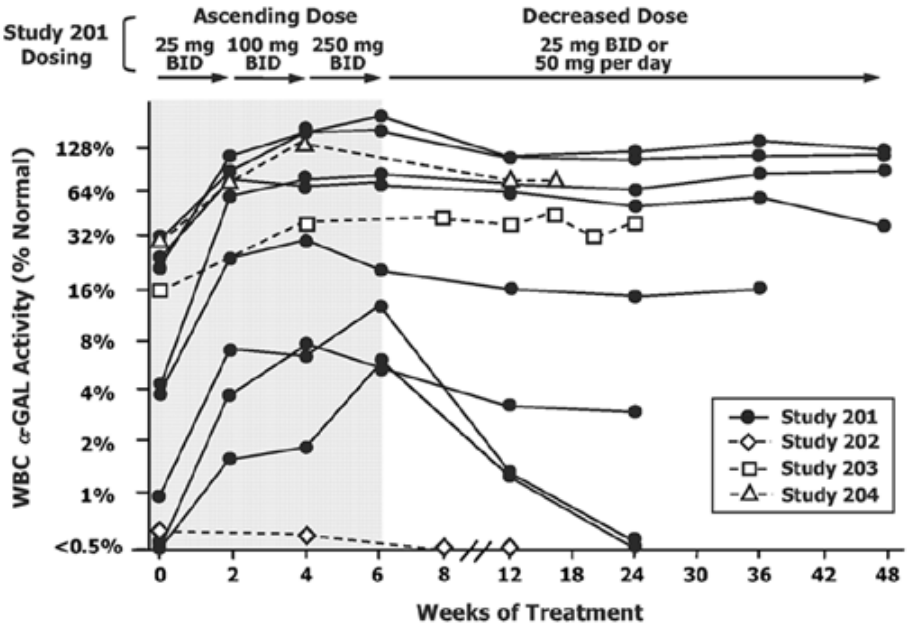
kidney biopsies, plasma and urine of patients in all four ongoing Phase II clinical studies of Amigal except Study 201 which does not include kidney biopsies. An additional objective of the Phase II clinical trials is the preliminary assessment of Amigal's effect on cardiac, renal and central nervous system function in Fabry disease patients.

Preliminary Data From Our Ongoing Phase II Clinical Trials

We have obtained initial results for the first eleven patients who have completed at least 12 weeks of treatment in our Phase II clinical trials of Amigal. Amigal has been well-tolerated to date with no reported drug-related serious adverse events. Four patients have been on Amigal for over a year. Adverse events were mostly mild and reported by the investigators as unlikely to be related to Amigal. One 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.

Initial results for the first eleven patients suggest that treatment with Amigal causes an increase in the level of α-GAL that we believe is likely to be clinically meaningful for a wide range of Fabry patients. Figure 2 below summarizes the available white blood cell α-GAL data for all eleven patients that have completed at least 12 weeks of treatment.

Figure 2: Enzyme Activity Response to Treatment with Amigal



Patients in the 202, 203 and 204 studies received 150 mg of Amigal every other day throughout the study.
For purposes of calculating the percentage of normal in the table, the level of α-GAL that is normal was derived by using the average of the levels of α-GAL in white blood cells of 15 healthy volunteers from the multiple-dose Phase I trial.

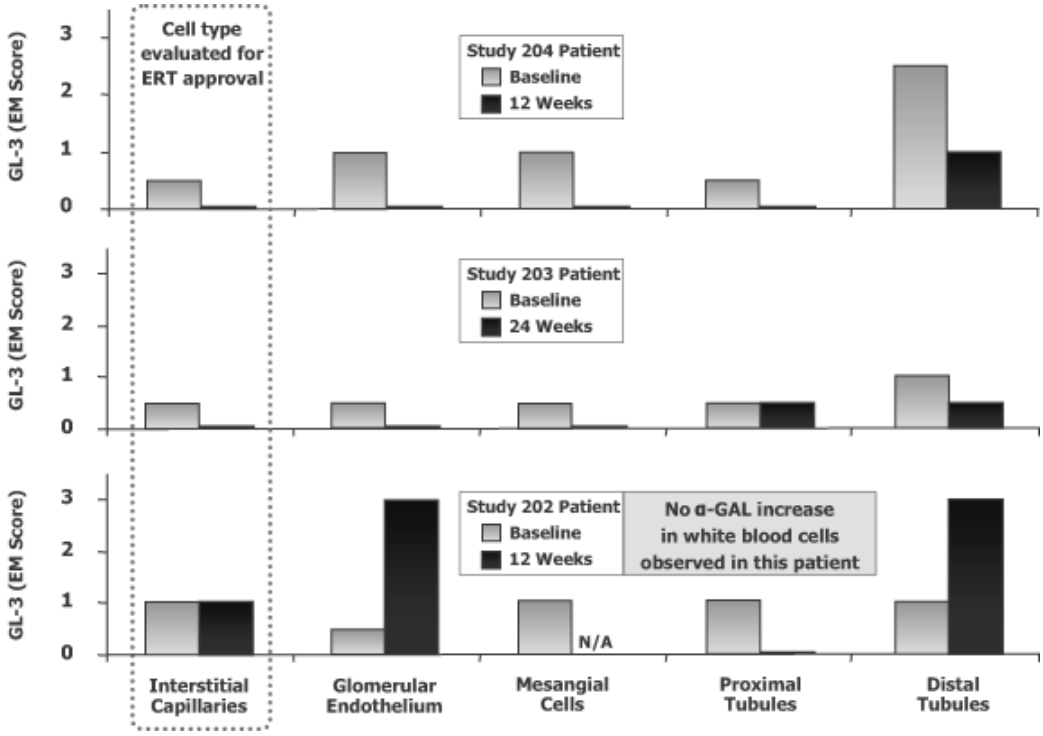
A summary of the preliminary data displayed in Figure 2 is provided below.

- The eleven patients represent ten different genetic mutations.
- The eleven patients consist of ten males and one female.
- The eleven patients have baseline levels of α-GAL enzyme activity in white blood cells that range from 0% to 30% of normal.
- Patients have been treated with various doses and regimens of Amigal for various periods of time in accordance with relevant protocols of our Phase II clinical trials.
- An increase in the level of α-GAL in white blood cells was observed in ten out of eleven patients.
- The results suggest a dose dependence particularly in several patients in Study 201, which included ascending doses through Week 6 and then a significantly decreased dose thereafter.
- We believe the α-GAL responses observed are likely to be therapeutically meaningful because it is generally believed that even small increases in lysosomal enzyme levels may have clinical benefits.
- We believe that these results provide the first evidence in patients of an effect of an orally administered pharmacological chaperone on its intended protein target.

GL-3, the lipid substrate broken down by α-GAL in the lysosome, accumulates in the cells of patients with Fabry disease and is believed to be the cause of the majority of disease symptoms. Reduction of the level of GL-3 in cells of the interstitial capillaries of the kidney was the basis of prior regulatory approval by the FDA of an enzyme replacement therapy for the treatment of Fabry disease. Initial data on kidney GL-3 levels before and after treatment with Amigal are available for three patients in our Phase II clinical trials.

Kidney GL-3 levels were assessed by an independent expert using light and electron microscopy. The expert was blinded to sample identification, including patient information and whether the sample came from a patient before or after treatment. GL-3 accumulation in each cell type was scored using a scale of 0-3 units, with 3 indicating severe GL-3, 2 indicating moderate GL-3, 1 indicating mild GL-3, and 0 indicating no GL-3. When the level of GL-3 in a cell was assessed to be in between scoring units, half point scores were used. For example, a score of 0.5 designates a cell with detectable GL-3, but at levels that are not as high as in a cell scored as 1. A change in GL-3 of at least 1 unit is considered conclusive. This same scoring system was used for the prior regulatory approval by the FDA of an enzyme replacement therapy for the treatment of Fabry disease.

Figure 3: GL-3 Response to Treatment with Amigal in Various Kidney Cell Types



A summary of the preliminary data displayed in Figure 3 is provided below.

- A decrease in GL-3 of at least 1 unit was observed in the kidney of one patient in Study 204 after 12 weeks of treatment in mesangial cells and the cells of the glomerular endothelium and distal tubules.
- One patient in Study 203 also showed a decrease of GL-3 levels in these same kidney cell types. In this patient, some of the scores were zero after treatment, but the decreases cannot be considered conclusive on their own because they involved a change of less than 1 full unit due to the lower levels of GL-3 observed at baseline.
- Both patients showed a decrease of GL-3 levels in other kidney cell types including cells of the interstitial capillaries, but the decreases were less than 1 unit and, thus, even though the post-treatment GL-3 score was zero, cannot be considered independently conclusive.
- One patient in Study 202 showed an increase in GL-3 levels in some cell types of the kidney and no change or a decrease in others after 12 weeks of treatment. Of the eleven patients who have completed at least twelve weeks of treatment to date in our ongoing clinical trials, this is the one patient who did not show an increase in the level of α-GAL in white blood cells after treatment with Amigal.
- Some kidney cell types such as podocyte cells did not show signs of GL-3 reduction.
- Results are presented as determined by electron microscopy, however light and electron microscopy values were generally consistent with one another.
- We believe that these data are the first evidence in patients of treatment with a pharmacological chaperone resulting in an effect on the biological activity of the intended protein target.

A summary of additional preliminary data from the first eleven patients that have completed 12 weeks of treatment is provided below.

Skin GL-3 levels at baseline and after treatment as assessed by light and electron microscopy are available for 10 patients. Seven patients had skin GL-3 levels that were normal or near normal both before and after treatment. Results for the three other patients were difficult to interpret because they showed evidence of a decrease in GL-3 in some skin cell types and an increase in GL-3 in other skin cell types, with variability over time.

Urine and plasma GL-3 levels at baseline and after treatment as assessed by liquid chromatography mass spectrometry are available for 10 patients. Most patients had GL-3 levels in urine and plasma that were normal or near normal both before and after treatment. For the few patients that had elevated levels of GL-3 in urine or plasma at baseline, the results were difficult to interpret due to high intra-patient variability.

Most patients in these studies had normal or near normal cardiac, renal and central nervous system function before treatment, and no clinically meaningful changes have been observed after 12 to 48 weeks of treatment.

The available data from the first eleven patients suggest that treatment with Amigal causes an increase in the level of α-GAL for a wide range of Fabry patients. We believe that this increase is likely to be therapeutically meaningful because it is generally believed that even small increases in lysosomal enzyme levels may have clinical benefits. We also believe the initial kidney GL-3 data suggest that the increased level of α-GAL that occurs after treatment with Amigal may result in a decrease in the substrate believed to be the cause of the symptoms of Fabry disease. Reduction of the level of GL-3 in cells of the interstitial capillaries of the kidney was the basis of prior regulatory approval by the FDA of an enzyme replacement therapy for the treatment of Fabry disease. We believe the preliminary results from the first eleven Fabry patients support the continuation of our current Phase II clinical trials.

The results of our Phase II clinical trials to date do not necessarily predict final results for our Phase II clinical trials. The results from additional patients in our ongoing Phase II clinical trials or additional data from these first eleven patients may cause the assessment of our Phase II trials to differ from or be less favorable than the assessment based on the initial results presented above. We cannot guarantee that our Phase II clinical trials will ultimately be successful.

Plicera for Gaucher Disease

Overview

Our second most advanced clinical product candidate, Plicera, is an orally-administered, small molecule, pharmacological chaperone for the treatment of Gaucher disease. We completed Phase I clinical trials which demonstrated that Plicera was safe and well tolerated in healthy subjects at all doses tested. We are currently conducting Phase II clinical trials of Plicera in Type I Gaucher patients. We expect to complete enrollment and obtain preliminary results of our Phase II trials in 2007. In February 2006, the FDA granted orphan drug designation for Plicera for the treatment of Gaucher disease in the United States.

Causes of Gaucher Disease and Rationale for Use of Plicera

Gaucher disease is a lysosomal storage disorder resulting from a deficiency in the enzyme, β-glucocerebrosidase, or GCase. Signs and symptoms can be severe and debilitating, including an enlarged liver and spleen, abnormally low levels of red blood cells and platelets, and skeletal complications. In some forms of the disease there is also significant impairment of the central nervous system. 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 fold into its correct three-dimensional shape. Although GCase produced in patient cells often 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.

Plicera 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 lysosomes, Plicera 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 Plicera increases the cellular levels 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 Plicera. 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 Plicera.

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 affecting more than 90% of patients 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.
- *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 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. Type I Gaucher disease is, by far, the most common of the subtypes.

Published data, including data from the Human Gene Mutation Database, suggest that the substantial majority of patients with Gaucher disease have a missense mutation in at least one copy of the gene. The majority of the Type I Gaucher patients in the United States, Europe and Israel have at least one copy of either the N370S or the L444P mutation, both of which are missense mutations. Based on our experience in the field and studies we have completed, including a Gaucher Ex Vivo Response Study, we believe that the substantial majority of individuals with Gaucher disease may benefit from treatment with Plicera. In addition, we believe that Plicera may also benefit some patients with the neuronopathic forms of Gaucher disease (Type II and Type III) because of the ability of the small molecule to cross the blood-brain barrier.

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

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 which is an enzyme replacement therapy. One of the products is Cerezyme, an enzyme replacement therapy 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 2006 were approximately $1.0 billion and $20 million, respectively, as publicly reported by Genzyme Corporation and Actelion Ltd. respectively.

For Gaucher disease patients who respond to Plicera, we believe that the use of Plicera may have advantages relative to the use of Cerezyme. 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 increased levels of red blood cells and platelets. However, because it is a large protein molecule, Cerezyme is believed to have difficulty 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 Plicera distributes throughout the body. In particular, studies show that Plicera crosses the blood-brain barrier, suggesting that it may provide a clinical benefit to patients with Type II and Type III Gaucher disease. Additionally, treatment with Cerezyme requires intravenous infusions every other week, presenting an inconvenience to Gaucher disease patients. Oral treatment with Plicera 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 Plicera 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 molecules that are used for many types of biological processes. As a result, inhibiting this enzyme may have adverse effects that are difficult to predict. By contrast, Plicera is designed to bind 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. Plicera’s mechanism of action is very different from Zavesca’s, and we do not expect it to have the same side-effect profile.

In February 2006, the FDA granted orphan drug designation for the active ingredient in Plicera for the treatment of Gaucher disease in the United States. 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 Plicera in either geography. See “Government Regulation”.

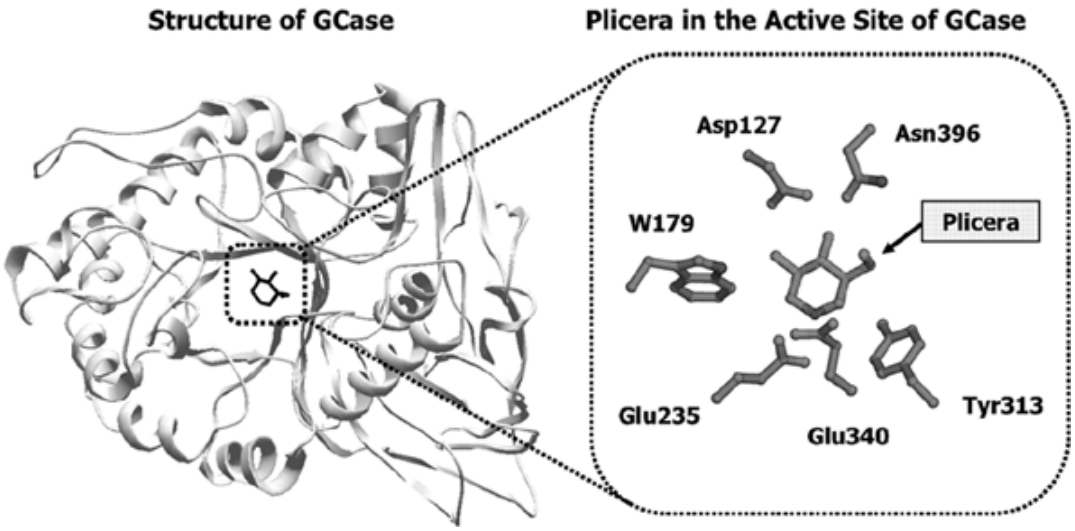
Plicera Development Activities

Preclinical Activities

We have conducted experiments in collaboration with researchers in the field to better understand the mechanism of action of Plicera. The primary conclusions of these experiments are summarized below.

- We have crystallized GCase both alone and with Plicera. These structural data demonstrate that Plicera binds directly to the active site of GCase. See Figure 4 below.
- In vitro exposure to Plicera increased transport of GCase to the lysosome in cells derived from a patient with the N370S mutation. Once in the lysosome, the enzyme was stable and active for more than 3 days after Plicera was removed. The N370S is the most common mutation associated with Gaucher disease in the western world.

Figure 4: Crystal Structure of GCase with Plicera



We have conducted several in vitro and in vivo preclinical studies of Plicera. Key findings of our studies are listed below.

- Oral administration of Plicera to both normal mice and mice expressing the L444P mutation resulted in a dose-dependent increase in GCase levels in the liver, spleen, brain and lungs. The L444P is one of the most common mutations associated with Gaucher disease.
- Oral administration of Plicera to L444P mice resulted in decreased spleen and liver weights and reduced plasma IgG and chitin III levels, which are biomarkers related to Gaucher disease.
- Oral administration of Plicera resulted in increased GCase levels in cells from hard bone and bone marrow in mice.

In 14-day, short-term, repeat dose, oral administration studies in rats and monkeys, no mortality or morbidity was observed at dose levels up to 1,500 mg/kg of Plicera. This dose was significantly higher than the human equivalent doses being considered for our future clinical studies. 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. The primary treatment-related toxicities were thickening of the lining of the forestomach of rats and mild reddening of the skin of monkeys. The forestomach is a region of the stomach that is only present in rodents and its lining is structurally similar to skin.

Six-month data from 9-month, repeat dose, oral administration studies in rats and monkeys showed that there was no mortality or morbidity at dose levels up to 200 mg/kg of Plicera. As in the 14-day toxicology studies, the primary treatment-related toxicities were thickening of the lining of the forestomach of rats and mild reddening of the skin of monkeys. All toxicities were found to be dose related and reversible or showed a trend toward reversibility. The clinical implications of these preclinical observations are unknown at this time. While the toxicities were observed at exposures comparable to the projected human exposure, the effect on the skin of the monkeys was very mild and any potential effect on the skin of humans could be readily monitored. In our 7-day, multiple-dose Phase I clinical trial of Plicera, no comparable effects on skin were observed.

Plicera has been tested for genotoxicity in a battery of both in vitro and in vivo genotoxicity assays. The results of these studies suggest that Plicera has an acceptable safety profile. We are currently conducting standard reproductive toxicity studies of Plicera and planning standard carcinogenicity studies.

Gaucher Ex Vivo Response Study

We have completed a study that corroborates our belief that a substantial majority of Gaucher patients may benefit from treatment with Plicera. The study evaluated and characterized the effects of Plicera in cells derived from patients with Gaucher disease. In this study, patients did not receive Plicera directly but provided blood samples from which certain cell types were isolated. We measured GCase levels in these cells before treatment and after incubation with Plicera for several days. We also measured biomarkers associated with Gaucher disease and other exploratory biomarkers. Preliminary data are available from 40 of the 53 patients who were enrolled in this study. These 40 patients included 21 males and 18 females with Type I Gaucher disease, the most common subtype of Gaucher disease which accounts for more than 90% of cases. In addition, preliminary data are available from one male with type III Gaucher disease. Out of these 40 patients, 34 (85%) had at least one copy of the GCase gene with the N370S mutation, the most common mutation in Type I Gaucher disease in the western world, found in more than 80% of the patient population. Patients ranged in age from 7 to 83 years, 38 of 40 patients were receiving enzyme replacement therapy and blood was drawn prior to infusion. We were able to derive usable cells from 34 of 40 subjects. A summary of the preliminary findings from the study is given below.

- Plicera increased GCase levels in cells derived from 32 of 34 patients (94%).
- Plicera increased GCase levels in cells derived from 28 of 29 patients (97%) with an N370S mutation and from 4 of 5 patients with mutations other than N370S.

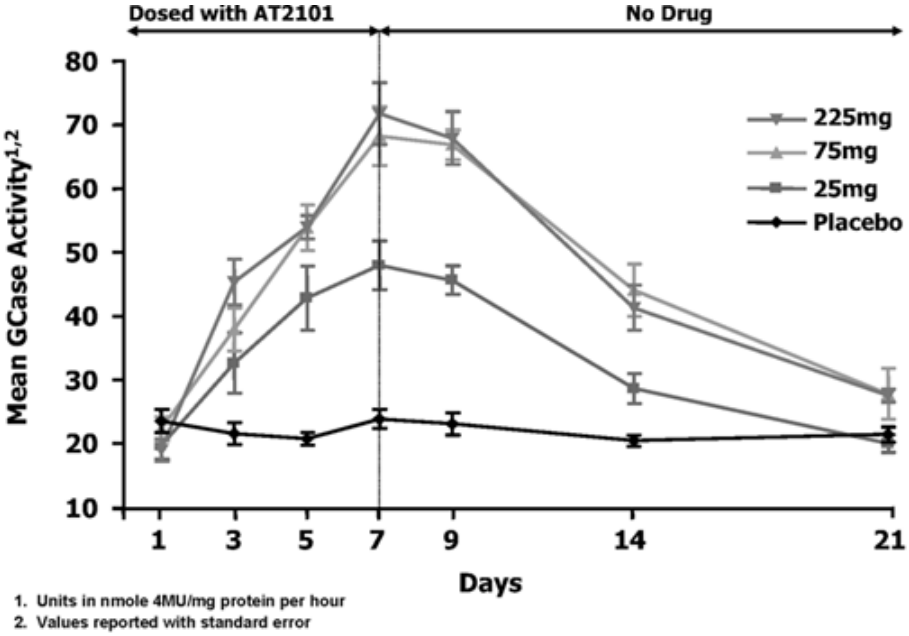
Phase I Clinical Trials

We have completed two Phase I clinical trials of Plicera in a total of 72 healthy volunteers, of which 54 were treated with Plicera and 18 were given placebo.

- *Single-Dose Phase I Trial.* Our single-dose Phase I trial was a single center, randomized, dose ranging study in healthy volunteers. The clinical phase began in June 2006 and was completed in September 2006. The study consisted of a total of 48 healthy volunteers divided into six groups of eight subjects. Six subjects in each group received oral administration of Plicera and two subjects received placebo. All subjects received single doses of placebo or 8 mg, 25 mg, 75 mg, 150 mg, 150 mg (repeat) or 300 mg of Plicera and were evaluated on Days 1 to 3 and on Day 7. The objectives of the study were to evaluate the safety and pharmacokinetics of Plicera in healthy volunteers.
- *Multiple-Dose Phase I Trial.* Our multiple-dose Phase I trial was a single center, randomized, dose ranging study in healthy volunteers. The clinical phase began in August 2006 and was completed in October 2006. The study consisted of a total of 24 healthy volunteers divided into three groups of eight subjects. Six subjects in each group received oral administration of Plicera and two subjects received placebo. All subjects received placebo or 25 mg, 75 mg or 225 mg of Plicera once a day for seven days. Subjects were evaluated on Days 1 to 7 and Days 9, 14 and 21. The objectives of the study were to evaluate the safety and pharmacokinetics of Plicera in healthy volunteers and to measure the level of GCase enzyme levels in white blood cells of healthy volunteers who received Plicera.

The data from our Phase I clinical trials in healthy volunteers showed that Plicera was generally safe and well tolerated at all doses. There were no serious adverse events and no subjects withdrew or discontinued due to an adverse event. In these studies, Plicera was shown to have good oral bioavailability and linear pharmacokinetics with a terminal half-life in plasma of approximately fourteen hours. Also, the data from the multiple-dose Phase I trial showed a statistically significant, dose-related increase in GCase levels in the white blood cells of normal, healthy volunteers who received oral administration of Plicera for seven days. The results are summarized below in Figure 5.

Figure 5: GCase Response to Plicera in Normal Volunteers



GCase activity was measured in white blood cells isolated from subjects receiving Plicera in daily oral doses for 7 days. Compared to placebo, GCase activity was significantly higher and increased over time in all treatment groups. GCase activity also increased with dose with the most marked increase, in absolute terms, between 25 and 75 mg. Relative percent increases at day 7 (time of maximal increase) compared to baseline were 147%, 209% and 279% at 25, 75 and 225 mg, respectively. Upon discontinuation of Plicera, GCase activity declined, returning to or near to baseline by day 21 (14 days of wash-out). The terminal half-life for decline of GCase activity upon removal of Plicera is about 4 to 5 days.

In addition to our findings in the Fabry disease studies, we believe these Phase I results are the only other demonstration of an increase in enzyme levels in humans following oral administration of a pharmacological chaperone. We believe normal enzyme levels can be increased because some fraction of normal protein molecules can also misfold and fail to pass the cell's quality control mechanisms. Normal GCase is stabilized by binding to the pharmacological chaperone, which results in an increase in the amount of enzyme successfully trafficked to the lysosome.

Phase II Clinical Trials

We are conducting two open-label Phase II clinical trials in up to 48 adult male and female patients with Type I Gaucher disease. In order to qualify for these clinical studies, patients must have a confirmed diagnosis of Type I Gaucher disease with a documented missense mutation in GCase. We expect to obtain preliminary results from the first of these two Phase II trials by the end of 2007.

- *Phase II Study 201.* We are conducting a Phase II trial in which we are seeking to enroll 32 patients with Type I Gaucher disease who are currently receiving enzyme replacement therapy and have agreed to discontinue their enzyme replacement therapy for a total of 7 weeks. The study is designed to assess the safety and pharmacodynamic effects of Plicera, particularly its effect on GCase levels. We will also monitor the effect of Plicera on parameters that are commonly abnormal in Gaucher disease including levels of red blood cells and platelets, although we do not expect to observe a change in these parameters in this 4-week trial because of its short duration. Patients will be assigned to one of four treatment arms and will receive Plicera for 4 weeks. Patients will receive 25 mg once per day, 150 mg once per day, 150 mg every four days, or 150 mg every seven days.
- *Phase II Study 202.* We are conducting a Phase II trial in which we are seeking to enroll 16 patients with Type I Gaucher disease who are naïve to enzyme replacement therapy and substrate reduction therapy. The study is designed to evaluate the safety of Plicera and its effect on parameters that are commonly abnormal in Gaucher disease including levels of red blood cells, platelets, liver and spleen volumes and other biomarkers related to Gaucher disease. Patients will be assigned to one of two treatment arms and will receive treatment with Plicera for approximately 6 months. Patients will receive 150 mg every four days or 150 mg every seven days.

AT2220 for Pompe Disease

Overview

Our third most advanced product candidate, AT2220, is an orally-administered small molecule pharmacological chaperone for the treatment of Pompe disease. We are currently conducting Phase I clinical trials of AT2220 for Pompe disease.

Causes of Pompe Disease and Rationale for Use of AT2220

Pompe disease is a neuromuscular and lysosomal storage disorder caused by a deficiency in the enzyme α-glucosidase, or Gaa. Symptoms can be severe and debilitating, including progressive muscle weakness throughout the body, particularly the heart and skeletal muscles. 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 that does not fold into its correct three-dimensional shape. Although Gaa produced in patient cells often retains the potential for 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. Certain other mutations cause changes in RNA processing that lead to the production of normal Gaa, but at levels that are much lower than in an unaffected individual. In either case, 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 majority of 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.

AT2220 is designed to act as a pharmacological chaperone for Gaa by selectively binding to Gaa and increasing its stability which helps the enzyme fold into its correct three-dimensional shape. We believe 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. We believe AT2220 may increase proper trafficking of Gaa in patients that produce unstable misfolded Gaa, and in patients that produce low levels of normal Gaa because some fraction of normal Gaa can also fail to pass the cell's quality control system. In addition, as a result of increasing the proper trafficking of unstable misfolded Gaa 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 is believed to increase the activity of a patient's naturally produced Gaa, those Pompe disease patients with a mutation that results in production of Gaa 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 with Pompe disease worldwide.

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 patients often experience progressive muscle weakness.

Pompe Disease Market Opportunity

Pompe disease is a relatively rare disorder. Most reported estimates project that there are 5,000 to 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 many of the known genetic mutations that cause Pompe disease are mutations that result in measurable residual enzyme activity. 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 benefit patients with some residual enzyme activity, we believe that a majority of the Pompe patient population may benefit from treatment with AT2220. There are a few mutations reported in Pompe disease that are more common in specific ethnic populations, including a splice-site mutation common in Caucasians with adult-onset disease. Studies published in the Journal of Medical Genetics, Human Mutation, and the Journal of Neurology suggest that over 70% of all Caucasians with adult-onset Pompe disease have at least one copy of this splice-site mutation. Because this splice-site mutation results in the production of normal Gaa protein, albeit at a level lower than in a non-affected individual, we believe patients with this mutation may be addressable with pharmacological chaperone therapy.

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 reported in infantile-onset disease. No data have been reported on the safety or efficacy of Myozyme in later-onset disease. The net product sales of Myozyme for 2006 were approximately $59 million as publicly reported by Genzyme Corporation.

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. Because AT2220 is a small molecule that has demonstrated high oral bioavailability and good biodistribution properties in preclinical testing, it 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. The label for Myozyme also indicates that the infusion has safety concerns, with infusion reactions observed in 51% of patients, and severe infusion-related reactions observed in 14% of 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 Development Activities

Preclinical Activities

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

- AT2220 increased levels of the active, mature form of Gaa in cells engineered to express different human Gaa missense mutations and in cells derived from patients with Pompe disease.
- Oral administration of AT2220 to normal mice resulted in an approximately 5-fold increase in the level of Gaa activity in most tissues examined, including heart, brain, diaphragm, soleus, tongue, and gastocnemius muscle. This increase in Gaa was assessed using a lysed cell enzyme activity assay and was correlated with increased levels of the mature form of Gaa in heart and gastrocnemius.

AT2220 demonstrated a favorable pharmacokinetic profile when tested in rats and monkeys, including good oral bioavailability and a terminal half-life of approximately 5 hours in rats, and 3 hours in monkeys. No mortality or morbidity was observed in the 14-day repeat dose, oral administration studies in rats and monkeys at dose levels up to 2,000 mg/kg of AT2220 in rats and up to 1,000 mg/kg of AT2220 in monkeys. The primary treatment-related toxicity observed in rats was decreased body weight gain which was correlated with decreased food consumption. These findings were modest and only occurred at the highest dose level. The primary treatment-related toxicities observed in monkeys were red blood cell, hemoglobin and hematocrit counts that were slightly lower relative to control. These toxicities were considered to be minimal and were observed in male and female monkeys at the highest dose, and male monkeys at the second highest dose. All of the observed toxicities in rats and monkeys were found to be reversible or showed a trend toward reversibility, and occurred only at doses that are significantly higher than the human equivalent doses being considered for clinical studies. The clinical implications of these preclinical observations are unknown at this time. Chronic toxicity testing of AT2220 is ongoing in 6-month rat studies and 9-month monkey studies. We are currently planning reproductive toxicity and carcinogenicity studies of AT2220.

Phase I Clinical Trials

We have completed a single-dose Phase I clinical trial of AT2220 and plan to initiate a multiple-dose Phase I clinical trial. Our single-dose Phase I study was a single center, randomized, dose-ranging study in healthy volunteers. The clinical phase began in December 2006 and was completed in February 2007. The study consisted of a total of 32 healthy volunteers divided into four groups of eight subjects. Six subjects in each group received AT2220 and two subjects received placebo. All subjects received single doses of placebo or 50 mg, 150 mg, 300 mg or 600 mg of AT2220 and were evaluated on Day 1 and on Day 8. The objectives of the study was to evaluate the safety and pharmacokinetics of AT2220 in healthy volunteers. The data from our single-dose Phase I clinical trial in healthy volunteers showed that AT2220 was well tolerated. The study also demonstrated that AT2220 has high oral bioavailability with a terminal half-life in plasma of approximately seven to eight hours.

If our Phase I trials are successful, we plan to initiate a Phase II trial by the end of 2007, and intend to develop AT2220 for the treatment of 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 currently researching the use of pharmacological chaperones for the treatment of diseases other than lysosomal storage disorders, including neurological diseases such as Parkinson's disease. We have an ongoing research program in Parkinson's disease and in January 2007, we received a grant from The Michael J. Fox Foundation for Parkinson's Research to further support this research program. Parkinson's disease is a chronic, progressive, degenerative disorder of the central nervous system. The disease affects an estimated 1 million people in the United States.

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 diseases. To achieve this objective, we intend to:

- *Focus our initial efforts on developing pharmacological chaperones for 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;
  - the novel mechanism 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 are currently conducting multiple Phase II clinical trials of Amigal for the treatment of Fabry disease. We expect to complete our current Phase II trials for Amigal by the end of 2007. We completed Phase I trials for Plicera in 2006 and are currently conducting Phase II trials for the treatment of Gaucher disease. We are currently conducting Phase I clinical trials of 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 our technology is broadly applicable to other diseases for which protein stabilization and improved folding may be beneficial, including certain types of neurological disease, metabolic disease, cardiovascular disease and cancer. 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. We plan to continue to apply our technologies to the discovery and development of treatments for genetic diseases as well as other conditions.
- *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 and Plicera, we believe that the clinical geneticists who are the key specialists in treating Fabry and Gaucher 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, unless this proprietary position would be better protected using trade secrets. 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 45 pending foreign applications, which are foreign counterparts of many of our U.S. patents. We also own or license rights to 22 pending U.S. applications, 7 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 three pending U.S. applications 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 α-GAL, and methods for the treatment of Fabry disease using Amigal and other specific competitive inhibitors of α-GAL. In addition, we own a pending U.S. application directed to specific treatment and monitoring regimens with Amigal and a pending U.S. application directed to dosing regimens with Amigal, which, if granted, may result in patents that expire in 2028; three pending U.S. applications directed to synthetic steps related to the commercial process for preparing Amigal, which may result in patents that expire in 2026. Lastly, we jointly own one pending U.S. application covering methods of diagnosing Fabry disease and determining whether Fabry patients will respond to treatment with Amigal, which, if granted, will expire in 2027. We have filed, or plan to file, foreign counterparts of these applications, where appropriate, by the applicable deadlines.
- We have an exclusive license to seven U.S. patents and two pending U.S. applications, and five foreign patents and a pending foreign application, that cover Plicera or its use. Two of the U.S. patents relating to Plicera compositions of matter expire in 2015 and 2016; the five composition of matter foreign patents and one pending foreign application, if granted, expire in 2015. The other five U.S. patents and two pending applications, which claim methods of increasing the activity of and preventing the degradation of GCase, and methods for the treatment of Gaucher disease using Plicera and other

specific competitive inhibitors of GCase, expire in 2018. We own two pending U.S. applications directed to the particular form of the active agent in Plicera, which, if granted, will expire in 2027. We own one pending U.S. application directed to dosing regimens for Plicera, which if granted, will expire in 2028. We own one pending U.S. application directed to specific treatment and monitoring regimens with Plicera. If granted, this also will expire in 2028. Lastly, we own one pending U.S. application directed to methods of synthesis of Plicera, which if granted, will expire in 2028. We have filed, or plan to file, foreign counterparts of these applications, where appropriate, by the applicable deadlines.

- We have an exclusive license to three U.S. patents that cover use of AT2220, two pending U.S. applications, 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 also includes patent applications we license or own relating to combination compositions or uses for our product candidates or new potential product candidates. Some of these applications are pending in the United States and foreign patent offices, and include one family of patents licensed from Mt. Sinai School of Medicine and one U.S. patent application and international application jointly owned with the Université of Montréal. Others have to date only been filed as provisional applications in the United States. We expect to file some of these as non-provisional applications in United States and in other countries at the appropriate time. These patent applications, assuming they issue as patents, would expire in the United States between 2023 and 2028.

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.

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 focus special attention on filing patent applications for formulations and delivery regimens for our products in development to further enhance our patent exclusivity for those products. 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, Plicera and AT2220 and other pharmacological chaperones for the prevention or treatment of human diseases or clinical conditions by increasing the activity of wild-type and mutant enzymes 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 royalties on net sales. In connection with this agreement, we issued 232,266 shares of our common stock to Mt. Sinai School of Medicine in April 2002. In October 2006 we issued Mt. Sinai School of Medicine an additional 133,333 shares of common stock and made a payment of $1,000,000 in consideration of an expanded field of use under that license. 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, or later subject to any patent term extension that may be granted.
- *University of Maryland, Baltimore County* — We have acquired exclusive U.S. patent rights to develop and commercialize Plicera 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 $29,500. We are required to make a milestone payment upon the demonstration of safety and efficacy of Plicera for the treatment of Gaucher disease in a Phase II study, and another payment upon receiving FDA approval for Plicera for the treatment of Gaucher disease. We are also required to pay royalties on net sales. Upon satisfaction of both milestones, we could be required to make up to $175,000 in aggregate payments. 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 Plicera for all human indications. Under this agreement, to date we have paid an aggregate of $400,000 in license fees. We are also required to make milestone payments based on clinical progress of Plicera, with a payment due after initiation of a Phase III clinical trial for Plicera for the treatment of Gaucher disease, and a payment due upon each filing for regulatory approval of Plicera for the treatment of Gaucher disease in any of the United States, Europe or Japan. An additional payment is due upon approval of Plicera for the treatment of Gaucher disease in the United States and a payment is also due upon each approval of Plicera for the treatment of Gaucher disease in either of Europe or Japan. Assuming successful development of Plicera for the treatment of Gaucher disease in the United States, Europe and Japan, total milestone payments would be $7,750,000. 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 Plicera.

Our rights with respect to these agreements to develop and commercialize Amigal, Plicera 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), AMIGAL and PLICERA. At present we have allowances as intent-to-use in the U.S., and some allowances or issued foreign registrations for all of these marks except PLICERA. In addition, we have filed an application in the United States to register PLICERA and we plan to file corresponding application abroad by the appropriate deadline. We have not yet obtained allowance for this mark. 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. For the allowed marks for our candidate products, it may be necessary to re-apply for registration if it becomes apparent that we will not use the mark in commerce within the prescribed time period. While we have received a notice from a third party alleging trademark infringement in connection with our intended use of the NASDAQ Global Market ticker symbol “FOLD” for our common stock, we believe this allegation is without merit and intend to vigorously contest it.

Manufacturing

We rely on contract manufacturers to supply the active pharmaceutical ingredients for Amigal, Plicera 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.

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	2006 Sales (in millions)
Genzyme Corporation	Fabry disease	Fabrazyme	Enzyme Replacement Therapy	Marketed	$ 359
	Gaucher disease	Cerezyme	Enzyme Replacement Therapy	Marketed	$ 1,007
	Pompe disease	Myozyme	Enzyme Replacement Therapy	Marketed	$ 59
	Gaucher disease	Genz-112638	Substrate Reduction Therapy	Phase II	N/A
Shire PLC	Fabry disease	Replagal	Enzyme Replacement Therapy	Marketed	$ 118
	Gaucher disease	GA-GCB	Enzyme Replacement Therapy	Phase III	N/A
Actelion, Ltd.	Gaucher disease	Zavesca	Substrate Reduction Therapy	Marketed	$ 20

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

FDA Approval Process

In the United States, pharmaceutical products are subject to extensive regulation by the FDA. The Federal Food, Drug, and Cosmetic Act, or the FDC Act, and other federal and state statutes and regulations, govern, among other things, the research, development, testing, manufacture, storage, recordkeeping, approval, labeling, promotion and marketing, distribution, post-approval monitoring and reporting, sampling, and import and export of pharmaceutical products. Failure to comply with applicable U.S. requirements may subject a company to a variety of administrative or judicial sanctions, such as FDA refusal to approve pending new drug applications or NDAs, warning letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, civil penalties, and criminal prosecution.

Pharmaceutical product development in the U.S. typically involves preclinical laboratory and animal tests, the submission to the FDA of a notice of claimed investigational exemption or an investigational new drug application or IND, which must become effective before clinical testing may commence, and adequate and well-controlled clinical trials to establish the safety and effectiveness of the drug for each indication for which FDA approval is sought. Satisfaction of FDA pre-market approval requirements typically takes many years and the actual time required may vary substantially based upon the type, complexity and novelty of the product or disease. Preclinical tests include laboratory evaluation of product chemistry, formulation and toxicity, as well as animal trials to assess the characteristics and potential safety and efficacy of the product. The conduct of the preclinical tests must comply with federal regulations and requirements including good laboratory practices. The results of preclinical testing are submitted to the FDA as part of an IND along with other information including information about product chemistry, manufacturing and controls and a proposed clinical

trial protocol. Long term preclinical tests, such as animal tests of reproductive toxicity and carcinogenicity, may continue after the IND is submitted.

A 30-day waiting period after the submission of each IND is required prior to the commencement of clinical testing in humans. If the FDA has not commented on or questioned the IND within this 30-day period, the clinical trial proposed in the IND may begin.

Clinical trials involve the administration of the investigational new drug to healthy volunteers or patients under the supervision of a qualified investigator. Clinical trials must be conducted in compliance with federal regulations, good clinical practices or GCP, as well as under protocols detailing the objectives of the trial, the parameters to be used in monitoring safety and the effectiveness criteria to be evaluated. Each protocol involving testing on U.S. patients and subsequent protocol amendments must be submitted to the FDA as part of the IND.

The FDA may order the temporary or permanent discontinuation of a clinical trial at any time or impose other sanctions if it believes that the clinical trial is not being conducted in accordance with FDA requirements or presents an unacceptable risk to the clinical trial patients. The study protocol and informed consent information for patients in clinical trials must also be submitted to an institutional review board, or IRB, for approval. An IRB may also require the clinical trial at the site to be halted, either temporarily or permanently, for failure to comply with the IRB's requirements, or may impose other conditions.

Clinical trials to support NDAs for marketing approval are typically conducted in three sequential phases, but the phases may overlap. In Phase I, the initial introduction of the drug into healthy human subjects or patients, the drug is tested to assess metabolism, pharmacokinetics, pharmacological actions, side effects associated with increasing doses and, if possible, early evidence on effectiveness. Phase II usually involves trials in a limited patient population, to determine the effectiveness of the drug for a particular indication or indications, dosage tolerance and optimum dosage, and identify common adverse effects and safety risks. If a compound demonstrates evidence of effectiveness and an acceptable safety profile in Phase II evaluations, Phase III trials are undertaken to obtain the additional information about clinical efficacy and safety in a larger number of patients, typically at geographically dispersed clinical trial sites, to permit FDA to evaluate the overall benefit-risk relationship of the drug and to provide adequate information for the labeling of the drug.

After completion of the required clinical testing, an NDA is prepared and submitted to the FDA. FDA approval of the NDA is required before marketing of the product may begin in the U.S. The NDA must include the results of all preclinical, clinical and other testing and a compilation of data relating to the product's pharmacology, chemistry, manufacture, and controls. The cost of preparing and submitting an NDA is substantial. Under federal law, the submission of most NDAs is additionally subject to a substantial application user fee, and the manufacturer and/or sponsor under an approved new drug application are also subject to annual product and establishment user fees. These fees are typically increased annually.

The FDA has 60 days from its receipt of a NDA to determine whether the application will be accepted for filing based on the agency's threshold determination that it is sufficiently complete to permit substantive review. Once the submission is accepted for filing, the FDA begins an in-depth review. The FDA has agreed to certain performance goals in the review of new drug applications. Most such applications for non-priority drug products are reviewed within ten months. The review process may be extended by FDA for three additional months to consider certain information or clarification regarding information already provided in the submission. The FDA may also refer applications for novel drug products or drug products which present difficult questions of safety or efficacy to an advisory committee, typically a panel that includes clinicians and other experts, for review, evaluation and a recommendation as to whether the application should be approved. The FDA is not bound by the recommendation of an advisory committee, but it generally follows such recommendations. 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 drug is manufactured. FDA will not approve the product unless compliance with current good manufacturing practices is satisfactory and the NDA contains data that provide substantial evidence that the drug is safe and effective in the indication studied.

After FDA evaluates the NDA and the manufacturing facilities, it issues an approval letter, an approvable letter or a not-approvable letter. Both approvable and not-approvable letters generally outline the deficiencies in the submission and may require substantial additional testing or information in order for the FDA to reconsider the application. If and when those deficiencies have been addressed to the FDA's satisfaction in a resubmission of the NDA, the FDA will issue an approval letter. FDA has committed to reviewing such resubmissions in 2 or 6 months depending on the type of information included.

An approval letter authorizes commercial marketing of the drug with specific prescribing information for specific indications. As a condition of NDA approval, the FDA may require substantial post-approval testing and surveillance to monitor the drug's safety or efficacy and may impose other conditions, including labeling restrictions which can materially affect the potential market and profitability of the drug. Once granted, product approvals may be withdrawn if compliance with regulatory standards is not maintained or problems are identified following initial marketing.

The Hatch-Waxman Act

In seeking approval for a drug through an NDA, applicants are required to list with the FDA each patent with claims that cover the applicant's product. Upon approval of a drug, each of the patents listed in the application for the drug is then published in the FDA's Approved Drug Products with Therapeutic Equivalence Evaluations, commonly known as the Orange Book. Drugs listed in the Orange Book can, in turn, be cited by potential competitors in support of approval of an abbreviated new drug application, or ANDA. An ANDA provides for marketing of a drug product that has the same active ingredients in the same strengths and dosage form as the listed drug and has been shown through bioequivalence testing to be therapeutically equivalent to the listed drug. ANDA applicants are not required to conduct or submit results of pre-clinical or clinical tests to prove the safety or effectiveness of their drug product, other than the requirement for bioequivalence testing. Drugs approved in this way are commonly referred to as "generic equivalents" to the listed drug, and can often be substituted by pharmacists under prescriptions written for the original listed drug.

The ANDA applicant is required to certify to the FDA concerning any patents listed for the approved product in the FDA's Orange Book. Specifically, the applicant must certify that: (i) the required patent information has not been filed; (ii) the listed patent has expired; (iii) the listed patent has not expired, but will expire on a particular date and approval is sought after patent expiration; or (iv) the listed patent is invalid or will not be infringed by the new product. A certification that the new product will not infringe the already approved product's listed patents or that such patents are invalid is called a Paragraph 4 certification. If the applicant does not challenge the listed patents, the ANDA application will not be approved until all the listed patents claiming the referenced product have expired.

If the ANDA applicant has provided a Paragraph 4 certification to the FDA, the applicant must also send notice of the Paragraph 4 certification to the NDA and patent holders once the ANDA has been accepted for filing by the FDA. The NDA and patent holders may then initiate a patent infringement lawsuit in response to the notice of the Paragraph 4 certification. The filing of a patent infringement lawsuit within 45 days of the receipt of a Paragraph 4 certification automatically prevents the FDA from approving the ANDA until the earlier of 30 months, expiration of the patent, settlement of the lawsuit or a decision in the infringement case that is favorable to the ANDA applicant.

The ANDA application also will not be approved until any non-patent exclusivity, such as exclusivity for obtaining approval of a new chemical entity, listed in the Orange Book for the referenced product has expired. Federal law provides a period of five years following approval of a drug containing no previously approved active ingredients, during which ANDAs for generic versions of those drugs cannot be submitted unless the submission contains a Paragraph 4 challenge to a listed patent, in which case the submission may be made four years following the original product approval. Federal law provides for a period of three years of exclusivity following approval of a listed drug that contains previously approved active ingredients but is approved in a new dosage form, route of administration or combination, or for a new use, the approval of which was required to be supported by new clinical trials conducted by or for the sponsor, during which FDA

cannot grant effective approval of an ANDA based on that listed drug for the same new dosage form, route of administration or combination, or new use.

Other Regulatory Requirements

Once an NDA is approved, a product will be subject to certain post-approval requirements. For instance, FDA closely regulates the post-approval marketing and promotion of drugs, including standards and regulations for direct-to-consumer advertising, off-label promotion, industry-sponsored scientific and educational activities and promotional activities involving the internet.

Drugs may be marketed only for the approved indications and in accordance with the provisions of the approved labeling. Changes to some of the conditions established in an approved application, including changes in indications, labeling, or manufacturing processes or facilities, require submission and FDA approval of a new NDA or NDA supplement before the change can be implemented. An NDA supplement for a new indication typically requires clinical data similar to that in the original application, and the FDA uses the same procedures and actions in reviewing NDA supplements as it does in reviewing NDAs.

Adverse event reporting and submission of periodic reports is required following FDA approval of an NDA. The FDA also may require post-marketing testing, known as Phase 4 testing, risk minimization action plans, and surveillance to monitor the effects of an approved product or place conditions on an approval that could restrict the distribution or use of the product. In addition, quality control as well as drug manufacture, packaging, and labeling procedures must continue to conform to current good manufacturing practices, or cGMPs, after approval. Drug manufacturers and certain of their subcontractors are required to register their establishments with FDA and certain state agencies, and are subject to periodic unannounced inspections by the FDA during which the agency inspects manufacturing facilities to access compliance with cGMPs. Accordingly, manufacturers must continue to expend time, money and effort in the areas of production and quality control to maintain compliance with cGMPs. Regulatory authorities may withdraw product approvals or request product recalls if a company fails to comply with regulatory standards, if it encounters problems following initial marketing, or if previously unrecognized problems are subsequently discovered.

Orphan Drugs

Under the Orphan Drug Act, the FDA may grant orphan drug designation to drugs intended to treat a rare disease or condition, which is generally a disease or condition that affects fewer than 200,000 individuals in the U.S. Orphan drug designation must be requested before submitting an NDA. After the FDA grants orphan drug designation, the generic identity of the drug and its potential orphan use are disclosed publicly by the FDA. Orphan drug designation does not convey any advantage in or shorten the duration of the regulatory review and approval process. The first NDA applicant with FDA orphan drug designation for a particular active ingredient to receive FDA approval of the designated drug for the disease for which it has such designation, is entitled to a seven-year exclusive marketing period in the U.S. for that product, for that indication. During the seven-year period, the FDA may not approve any other applications to market the same drug for the same disease, except in limited circumstances, such as a showing of clinical superiority to the product with orphan drug exclusivity. Orphan drug exclusivity does not prevent FDA from approving a different drug for the same disease or condition, or the same drug for a different disease or condition. Among the other benefits of orphan drug designation are tax credits for certain research and a waiver of the NDA application user fee.

Pediatric Information

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.

Fast Track Designation

Under the fast track program, the sponsor of a new drug candidate may request FDA to designate the drug candidate as a fast track drug concurrent with or after the filing of the IND for the drug candidate. FDA must determine if the drug candidate qualifies for fast track designation within 60 days of receipt of the sponsor's request. Once FDA designates a drug as a fast track candidate, it is required to facilitate the development and expedite the review of that drug.

In addition to other benefits such as the ability to use surrogate endpoints and have greater interactions with FDA, FDA may initiate review of sections of a fast track drug's NDA before the application is complete. This rolling review is available if the applicant provides and FDA approves a schedule for the submission of the remaining information and the applicant pays applicable user fees. However, FDA's time period goal for reviewing an application does not begin until the last section of the NDA is submitted. Additionally, the fast track designation may be withdrawn by FDA if FDA believes that the designation is no longer supported by data emerging in the clinical trial process.

Priority Review

Under FDA policies, a drug candidate is eligible for priority review, or review within a six-month time frame from the time a complete NDA is accepted for filing, if the drug candidate provides a significant improvement compared to marketed drugs in the treatment, diagnosis or prevention of a disease. A fast track designated drug candidate would ordinarily meet FDA's criteria for priority review.

Accelerated Approval

Under FDA's accelerated approval regulations, FDA may approve a drug for a serious or life-threatening illness that provides meaningful therapeutic benefit to patients over existing treatments based upon a surrogate endpoint that is reasonably likely to predict clinical benefit. In clinical trials, a surrogate endpoint is a measurement of laboratory or clinical signs of a disease or condition that substitutes for a direct measurement of how a patient feels, functions, or survives. Surrogate endpoints can often be measured more easily or more rapidly than clinical endpoints. A drug candidate approved on this basis is subject to rigorous post-marketing compliance requirements, including the completion of Phase 4 or post-approval clinical trials to confirm the effect on the clinical endpoint. Failure to conduct required post-approval studies, or confirm a clinical benefit during post-marketing studies, will allow FDA to withdraw the drug from the market on an expedited basis. All promotional materials for drug candidates approved under accelerated regulations are subject to prior review by FDA.

Section 505(b)(2) New Drug Applications

Most drug products obtain FDA marketing approval pursuant to an NDA or an ANDA. A third alternative is a special type of NDA, commonly referred to as a Section 505(b)(2) NDA, which enables the applicant to rely, in part, on the safety and efficacy data of an existing product, or published literature, in support of its application.

505(b)(2) NDAs often provide an alternate path to FDA approval for new or improved formulations or new uses of previously approved products. Section 505(b)(2) permits the filing of an NDA where at least some of the information required for approval comes from studies not conducted by or for the applicant and for which the applicant has not obtained a right of reference. The applicant may rely upon certain preclinical or clinical studies conducted for an approved product. The FDA may also require companies to perform additional studies or measurements to support the change from the approved product. The FDA may then approve the new product candidate for all or some of the label indications for which the referenced product has been approved, as well as for any new indication sought by the Section 505(b)(2) applicant.

To the extent that the Section 505(b)(2) applicant is relying on studies conducted for an already approved product, the applicant is required to certify to the FDA concerning any patents listed for the approved product in the Orange Book to the same extent that an ANDA applicant would. Thus approval of a 505(b)(2) NDA can

be stalled until all the listed patents claiming the referenced product have expired, until any non-patent exclusivity, such as exclusivity for obtaining approval of a new chemical entity, listed in the Orange Book for the referenced product has expired, and, in the case of a Paragraph 4 certification and subsequent patent infringement suit, until the earlier of 30 months, settlement of the lawsuit or a decision in the infringement case that is favorable to the Section 505(b)(2) applicant.

Anti-Kickback, False Claims Laws & The Prescription Drug Marketing Act

In addition to FDA restrictions on marketing of pharmaceutical products, several other types of state and federal laws have been applied to restrict certain marketing practices in the pharmaceutical industry in recent years. These laws include anti-kickback statutes and false claims statutes. The federal healthcare program anti-kickback statute prohibits, among other things, knowingly and willfully offering, paying, soliciting or receiving remuneration to induce or in return for purchasing, leasing, ordering or arranging for the purchase, lease or order of any healthcare item or service reimbursable under Medicare, Medicaid or other federally financed healthcare programs. This statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on the one hand and prescribers, purchasers and formulary managers on the other. Violations of the anti-kickback statute are punishable by imprisonment, criminal fines, civil monetary penalties and exclusion from participation in federal healthcare programs. Although there are a number of statutory exemptions and regulatory safe harbors protecting certain common activities from prosecution or other regulatory sanctions, the exemptions and safe harbors are drawn narrowly, and practices that involve remuneration intended to induce prescribing, purchases or recommendations may be subject to scrutiny if they do not qualify for an exemption or safe harbor.

Federal false claims laws prohibit any person from knowingly presenting, or causing to be presented, a false claim for payment to the federal government, or knowingly making, or causing to be made, a false statement to have a false claim paid. Recently, several pharmaceutical and other healthcare companies have been prosecuted under these laws for allegedly inflating drug prices they report to pricing services, which in turn were used by the government to set Medicare and Medicaid reimbursement rates, and for allegedly providing free product to customers with the expectation that the customers would bill federal programs for the product. In addition, certain marketing practices, including off-label promotion, may also violate false claims laws. The majority of states also have statutes or regulations similar to the federal anti-kickback law and false claims laws, which apply to items and services reimbursed under Medicaid and other state programs, or, in several states, apply regardless of the payor.

Physician Drug Samples

As part of the sales and marketing process, pharmaceutical companies frequently provide samples of approved drugs to physicians. The Prescription Drug Marketing Act, or the PDMA, imposes requirements and limitations upon the provision of drug samples to physicians, as well as prohibits states from licensing distributors of prescription drugs unless the state licensing program meets certain federal guidelines that include minimum standards for storage, handling and record keeping. In addition, the PDMA sets forth civil and criminal penalties for violations.

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 Plicera 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 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 "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 Plicera in the European Union for the treatment of Gaucher disease because Plicera 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 advisors who are leading experts in the fields of lysosomal enzymes, protein folding and structures, protein trafficking, sugar and carbohydrate biochemistry, post-transcriptional regulation and the underlying pathology, clinical diagnosis and treatment of lysosomal storage disorders. Our scientific advisory board consults with us regularly on matters relating to:

- our research and development programs;
- the design, implementation of basic science and mechanistic studies;
- the design, implementation and interpretation of animal model studies;
- market opportunities from a clinical perspective;
- new ideas, science and technologies relevant to our research and development programs; and
- scientific, technical and medical issues relevant to our business.

Our current scientific advisory board members are:

Name	Professional Affiliation
Michel Bouvier, Ph.D.	Professor and Director, University Research Group on Drug Discovery, Department of Biochemistry, Institute for Research in Immunology and Cancer, Faculty of Medicine, Université de Montréal; Canada Research Chair in Signal Transduction and Molecular Pharmacology
Barry J. Byrne, M.D., Ph.D.	Director, UF Powell Gene Therapy Center; Professor, Molecular Genetics & Microbiology; Associate chair of Pediatrics, Department of Pediatrics/Powell Gene Therapy Center
Arthur L. Horwich, M.D.	Professor of Genetics and Pediatrics, Yale University School of Medicine; Investigator, Howard Hughes Medical Institute

Name	Professional Affiliation
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

Medical Advisory Board

Our medical advisory board consists of physician scientists who are leading experts in the diagnosis, understanding and treatment of Gaucher disease, Fabry disease and Pompe disease. The members of the board are well-published and perform clinical and basic science research in lysosomal storage disease; they are recognized as opinion-leaders in the field of genetic medicine and metabolic disorders. Our medical advisory board consults with us periodically on matters relating to:

- our research and clinical development programs;
- the design and implementation of our clinical studies;
- market opportunities from a medical perspective;
- leading medical understanding of lysosomal diseases; and
- current therapeutic paradigms in our target medical areas.

Name	Professional Affiliation
Dominique Germain, M.D., Ph.D.	Assistant Professor, Department of Genetics; Director, “Centre de référence de la maladie de Fabry et des maladies héréditaires du tissu conjonctif,” Assistance Publique, Hopitaux de Paris, Paris, France
Pramod K. Mistry M.D., Ph.D., FRCP	Professor and Chief, Section of Pediatric Hepatology and Gastroenterology, Yale University School of Medicine; Director, National Gaucher Disease Program; Director, Inherited Metabolic Liver Disease Clinic, Yale University School of Medicine
Marc Patterson, M.D., FRACP	Professor of Clinical Neurology and Pediatrics and Director, Division of Pediatric Neurology, Departments of Neurology and Pediatrics, College of Physicians & Surgeons of Columbia University; Director of Pediatric Neurology and Child Neurology Training Program Director, Morgan Stanley Children’s Hospital of New York-Presbyterian Columbia University Medical Center
Thomas Voit, M.D., Ph.D.	Medical and Scientific Director, Institut de Myologic, Groupe Hospitalier Pitié-Salpétrière; Assistant Professor, University Pierre et Marie Curie Paris VI, Paris, France

Employees

As of April 25, 2007, we had 76 full-time employees, 53 of whom were primarily engaged in research and development activities and 23 of whom provide administrative services. A total of 30 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 sublease agreement for this office and laboratory space. This operating sublease will expire by its terms in February 2012. In July 2006, we entered into a 3-year non-cancellable operating sublease agreement for additional office and laboratory space at a second facility located in Cranbury, New Jersey, consisting of approximately 17,000 square feet. This operating sublease will expire by its terms in August 2009. As we have the ability to reasonably grow our operations for the foreseeable future within our existing 32,000 square feet of subleased space, we believe that our current office and laboratory facilities in Cranbury, New Jersey are adequate and suitable for our current and anticipated needs.

Legal Proceedings

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

MANAGEMENT

Our executive officers and directors and their respective ages and positions as of April 25, 2007 are as follows:

Name	Age	Position
John F. Crowley	40	President and Chief Executive Officer and Director
Matthew R. Patterson	35	Chief Operating Officer
James E. Dentzer	40	Chief Financial Officer
David J. Lockhart, Ph.D.	45	Chief Scientific Officer
David Palling, Ph.D.	53	Senior Vice President, Drug Development
Karin Ludwig, M.D.	45	Senior Vice President, Clinical Research
Mark Simon	45	Senior Vice President, Business Development
Douglas A. Branch	50	Vice President, General Counsel and Secretary
Gregory P. Licholai, M.D.	42	Vice President, Medical Affairs
S. Nicole Schaeffer	38	Vice President, Human Resources and Leadership Development
Bradley L. Campbell	31	Vice President, Business Planning
Donald J. Hayden(3)	51	Chairman and Director
Alexander E. Barkas, Ph.D.(3)	59	Director
Michael G. Raab(1)(3)	42	Director
Glenn P. Sblendorio(2)	50	Director
James N. Topper, M.D., Ph.D.(1)	45	Director
Stephen Bloch, M.D.(2)	44	Director
Gregory M. Weinhoff, M.D.(2)	36	Director
P. Sherrill Neff(1)	55	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 since January 2005, and has also served as a Director of Amicus since August 2004, with the exception of the period from September 2006 to March 2007 when he was not an officer or director of Amicus while he was in active duty service in the United States Navy (Reserve). He was President and Chief Executive Officer of Orexigen Therapeutics, Inc. from September 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 Operating Officer since September 2006. From December 2004 to September 2006 he served as Chief Business Officer. From 1998-2004, Mr. Patterson worked at BioMarin Pharmaceuticals Inc. where he was Vice President, Regulatory and Government Affairs from 2001-2003 and later Vice President, Commercial Planning from 2003-2004. From 1993-1998, Mr. Patterson worked at Genzyme Corporation in Regulatory Affairs and Manufacturing. Mr. Patterson received a B.A. in Biochemistry from Bowdoin College.

James E. Dentzer has served as Chief Financial Officer since October 2006. From November 2003 to October 2006, Mr. Dentzer was Corporate Controller at Biogen Idec Inc. From 2001 until the 2003 merger of

Biogen, Inc. and IDEC Pharmaceuticals Corporation, Mr. Dentzer served as Corporate Controller of Biogen, Inc. Prior to that, he served in a variety of financial positions at E. I. du Pont de Nemours and Company, most recently as Chief Financial Officer of DuPont Flooring Systems. Mr. Dentzer received his B.A. from Boston College and his M.B.A. from the University of Chicago.

David J. Lockhart, Ph.D., has served as 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 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, United States 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.

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.

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.

Bradley L. Campbell has served as Vice President, Business Planning since May 2007. From April 2006 until May 2007, he served as Senior Director, Business Development. From 2002 until 2006, Mr. Campbell served as Senior Product Manager and later Business Director of CV Gene Therapy at Genzyme Corporation. Mr. Campbell received his B.A. from Duke University and his M.B.A. from Harvard Business School.

Donald J. Hayden, Jr. has served as Chairman since March 2006 and from September 2006 until March 2007 he served as Interim President and Chief Executive Officer. From 1991 to 2005 he held several executive positions with Bristol-Myers Squibb Company, most recently serving as Executive Vice President and President, Americas. 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 1997, Dr. Barkas has been a co-founder and served as a managing member of the general partner of a series of Prospect Venture Partners' funds. Dr. Barkas serves as the chairman of the board of directors of two publicly-held biotechnology companies, Geron Corporation and Tercica, Inc., and as a director of several private biotechnology and medical device companies. 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, he was a Senior Vice President, Therapeutics and General Manager, Renagel® at Genzyme Corporation. Mr. Raab is a director of Novacea, Inc. Mr. Raab holds a B.A. from DePauw University.

Glenn P. Sblendorio has served as a member of our board of directors since June 2006. Mr. Sblendorio has served as Chief Financial Officer and Executive Vice President of The Medicines Company since March 2006. Prior to joining The Medicines Company, Mr. Sblendorio was Executive Vice President and Chief Financial Officer of Eyetech Pharmaceuticals, Inc. from February 2002 until it was acquired by OSI Pharmaceuticals, Inc. in November 2005. From July 2000 to February 2002, Mr. Sblendorio served as Senior Vice President of Business Development at The Medicines Company. Mr. Sblendorio received his B.B.A. from Pace University and his M.B.A. from Fairleigh Dickinson 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 Pharmaceutical 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 venture partner 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 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 Composition and Election of Directors

Our board of directors is currently authorized to have, and we currently have, nine 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 Drs. Barkas and Bloch, and Mr. Neff, and their term will expire at the annual meeting of stockholders to be held in 2008;
- the class II directors will be Drs. Topper and Weinhoff, and Mr. Hayden, and their term will expire at the annual meeting of stockholders to be held in 2009; and
- the class III directors will be Messrs. Crowley, Raab, and Sblendorio, and their term will expire at the annual meeting of stockholders to be held in 2010.

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.

Our board of directors has reviewed the materiality of any relationship that each of our directors has with us, either directly or indirectly. Based on this review, the board has determined that the following directors are “independent directors” as defined by the rules of The NASDAQ Global Market: Messrs. Hayden, Raab, Sblendorio and Neff and Drs. Barkas, Topper, Bloch and Weinhoff. Upon the listing of our common stock on the NASDAQ Global Market, 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 and corporate governance committee.

Audit Committee

The members of our audit committee are Mr. Sblendorio and Drs. Bloch and Weinhoff. Mr. Sblendorio chairs the audit committee and serves as our audit committee financial expert. 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.

Our audit committees responsibilities 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.

Nasdaq rules require that all members of the audit committee be independent directors, as defined by the rules of the Nasdaq and the SEC. The Nasdaq rules also permit a company, such as us, listing on The NASDAQ Global Market in connection with its initial public offering to have only one member of the audit committee comply with the independence requirements on the date of listing, provided that a majority of the members satisfy the requirements within 90 days after listing and that all of the members satisfy the requirements within one year after listing. Currently, our board of directors has determined that Mr. Sblendorio satisfies the independence requirements for service on the audit committee. Our board of directors also believes that both Dr. Weinhoff and Dr. Bloch will satisfy these independence requirements within 90 days after the date of listing of our common stock. In the event that either of Dr. Weinhoff or Dr. Bloch does not satisfy these independence requirements prior to the end of the 90 day period, our board of directors would modify the committee in order to comply with these requirements.

Compensation Committee

Messrs. Neff and Raab and Dr. Topper 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.

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;
- reviewing and approving potential executive and senior management succession plans; and
- reviewing and approving non-routine employment agreements, severance agreements and change in control agreements.

We believe that the composition of our compensation committee meets the requirements for independence under the current NASDAQ Global Market rules and regulations.

Nominating and Corporate Governance Committee

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

We believe that the composition of our nominating and corporate governance committee meets the requirements for independence under the current NASDAQ Global Market rules and regulations.

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.

COMPENSATION DISCUSSION AND ANALYSIS

Objectives and Philosophy of Executive Compensation

The primary objective of our compensation program, as established by the compensation committee of our board of directors, composed entirely of independent directors, is to attract, retain and motivate the best possible executive talent. Our overall philosophy is to tie both short and long-term cash and equity incentives to the achievement of our executives against measurable corporate and individual performance objectives, and to align their incentives with the creation of value for our stockholders. The role of the compensation committee is to oversee our compensation and benefit plans and policies, administer our equity incentive plans, and review and approve annually all compensation decisions relating to all executive officers. Specifically, our compensation programs are designed to:

- Attract and retain individuals of superior ability and managerial talent;
- Ensure senior officer compensation is aligned with our corporate strategies, business objectives and the long-term interests of our stockholders;
- Increase the incentive to achieve key strategic and financial performance measures by linking incentive award opportunities to the achievement of performance goals in these areas; and
- Enhance the officers' incentive to maximize stockholder value, as well as promote retention of key people, by providing a portion of total compensation opportunities for senior management in the form of direct ownership in our company.

To achieve these objectives, the compensation committee expects to implement and maintain compensation plans that tie a substantial portion of the executives' overall compensation to achievement of corporate and individual performance objectives. Base salary increases and performance bonus payments are primarily tied to these corporate and individual objectives, while the size of equity awards are primarily tied to promoting long-term employee retention.

Corporate Objectives. Corporate objectives are established at the beginning of each year and are the basis for determining corporate performance for the year. The key strategic corporate, financial and operational goals that are established by our board of directors include:

- clinical trial progress;
- pre-clinical drug development;
- continued intellectual property development; and
- implementation of appropriate financing or business development strategies.

Individual Objectives. Individual objectives are also established at the beginning of each year by the supervisor of each executive. These objectives represent significant milestones that must be met by each executive, along with dates for achieving the milestones. Factors are identified and specified that will be used to measure success in reaching the goal or objective. Objectives are established based on the executive's principal areas of responsibility. For example, our scientific executives will have measurable objectives established for areas such as key research or scientific milestones and our clinical executives will be measured by clinical trial progress.

Evaluations. After the completion of each fiscal year, we evaluate individual and corporate performance against stated goals for the year. Consistent with our overall compensation philosophy, each employee undergoes a performance evaluation process involving his or her direct supervisor and other senior executives to the extent appropriate. This process leads to a recommendation for annual salary increases, bonuses and equity awards, if any, which are then reviewed and approved by our compensation committee. The performance of our executive officers, after input from each of them as to their own performance, is generally assessed by our chief executive officer. In the case of our chief executive officer, his performance is assessed primarily by the chairman of our board of directors, with an

opportunity for input from each member of our board of directors. Any annual base salary increases, equity awards and bonuses, to the extent granted, are generally implemented during the first calendar quarter of the following year.

The compensation committee evaluates individual executive performance with the goal of setting compensation at levels the committee believes are in the upper half for executives in companies of similar size and stage of development operating in the biotechnology industry, taking into account our relative performance and our own strategic goals. In order to ensure that we continue to remunerate our executives appropriately and consistent with market information, we will participate in, and review data from, certain compensation surveys, and may confer with outside compensation consultants.

Elements of Executive Compensation

Executive compensation consists of the following elements:

Base Salary. Base salaries for our executives are generally established based on the scope of their responsibilities, taking into account competitive market compensation paid by other companies for similar positions and recognizing cost of living considerations. As with total executive compensation, we believe that our executive base salaries should be targeted in the upper half of the range of salaries for executives in similar positions and with similar responsibilities in comparable biotechnology companies. We have reviewed data from the Radford Biotechnology Survey and the Radford Biotech Pre-IPO Survey as primary reference points. These surveys are analyses of compensation which use private biotechnology companies for benchmarking purposes. In general, base salaries are reviewed annually, and adjusted to realign salaries with market levels and adjust for inflation. Base salaries may be adjusted from time to time during the year in connection with promotions that may occur.

Annual Performance Bonus. The compensation committee has the authority to award annual performance bonuses to our executives. Bonuses are determined by two factors: individual performance and company performance. Each of our executives is eligible to receive an annual performance bonus based upon a targeted percentage of base salary. The targeted bonus level for a particular executive is determined by the executive's rank, with each level differentiated as follows:

Position	Targeted Bonus % of Base Salary
• Chief Executive Officer	50%
• Other Chief Officers	30%
• Vice Presidents	25%

If the company and or the executive exceeds the objectives established at the beginning of the year, or if the performance of either the company or the executive is extraordinary, then the bonus payable to the executive could exceed the targeted percentages of base salary. If an executive's performance does not meet objectives established for the year, then the bonus payable to the executive will not meet the targeted percentages. In addition, if the company objectives are not met, the amount of bonus paid to our executives can be substantially reduced or not paid at all. Performance levels and bonuses are at the judgment of the compensation committee.

Long-Term Incentive Program. We believe that long-term performance will be enhanced through stock and equity awards that reward our executives for maximizing shareholder value over time and that align the interests of our employees and management with those of stockholders. The compensation committee believes that the use of stock and equity awards offers the best approach to achieving our compensation goals because equity ownership ties a significant portion of an executive's compensation to the performance of our company's stock. We have historically elected to use stock options as the primary long-term equity incentive vehicle.

Stock Options. Our 2007 equity incentive plan, or the 2007 plan, to be in effect upon the closing of this offering, and our 2002 equity incentive plan, or the 2002 plan, authorize or authorized us to grant

options to purchase shares of common stock to our employees, directors and consultants. Our compensation committee oversees the administration of our stock options. Stock option grants are made at the commencement of employment and, occasionally, following a significant change in job responsibilities or to meet other special retention objectives. We have also historically made option grants on a company-wide basis and may also make company-wide grants in the future. The compensation committee considers and approves stock option awards to executive officers based upon a review of competitive compensation data, its assessment of individual performance, a review of each executive's existing long-term incentives, and retention considerations. Periodic company-wide option grants and case-by-case option grants are made at the discretion of the compensation committee to eligible employees and, in appropriate circumstances, with the input of the chairman of our board of directors, as well as our chief executive officer and other members of management.

In 2006, certain named executive officers were awarded stock options in the amounts indicated in the section entitled "Grants of Plan-Based Awards." This includes stock options granted company-wide in February 2006, including all named executive officers (other than Mr. Dentzer who did not join us until the fall of 2006). These option grants were based on the performance of the employees, to encourage continued service with us and to recalibrate their ownership on a percentage basis, taking into account equity dilution resulting from stock issuance and grants made to recently hired executives. All of the stock option awards were subject to a standard vesting schedule.

In 2006 we made a grant of stock options to Mr. Crowley and this grant was determined by our compensation committee and approved by our board of directors. Options granted in 2006 to Mr. Hayden in connection with his election as chairman were determined by the board of directors, after obtaining information from discussions among Mr. Neff, acting on behalf of our board, and Mr. Crowley. Mr. Hayden was granted additional options in 2006 in connection with his service as Interim President and Chief Executive Officer. The amount of that grant was determined by our board of directors after obtaining information from discussions between Mr. Neff, acting on behalf of the compensation committee, and Mr. Hayden. The grant of stock options to Mr. Dentzer in 2006 in connection with his hiring was made after obtaining information from discussions among Mr. Neff, acting on behalf of the compensation committee, Mr. Crowley and Mr. Dentzer. Option grants in February 2006 for our executive officers were determined by the board on the recommendation of the compensation committee, based in part upon recommendations made by Mr. Crowley. Mr. Crowley and the compensation committee relied in part on the Radford Survey as a reference point to bring our executive compensation packages more in line with those prevailing in the market. The initial grant to Dr. Lockhart upon the commencement of his employment in January 2006 was made after obtaining information from discussions among Mr. Neff, acting on behalf of the compensation committee, Mr. Crowley and Dr. Lockhart.

The exercise price of options is the fair market value of our common stock as determined by our board of directors on the date of grant. Our stock options typically vest over a four-year period with 25% vesting 12 months after the vesting commencement date and the remainder vesting ratably each month thereafter in equal installments over a 3-year period subject to continued employment or association with us, and generally expire ten years after the date of grant. Incentive stock options also include certain other terms necessary to assure compliance with the applicable provision of the Internal Revenue Code.

We expect to continue to use stock options as a long-term incentive vehicle because we believe that:

- Stock options and the vesting period of stock options attract and retain executives.
- Stock options are inherently performance based. Because all the value received by the recipient of a stock option is based on the growth of the stock price, stock options enhance the executives' incentive to increase our stock price and maximize stockholder value.
- Stock options help to provide a balance to the overall executive compensation program as base salary and our annual performance bonus program focus on short-term compensation, while stock options reward executives for increases in shareholder value over the longer term.

Restricted Stock. Our 2007 plan and our 2002 plan authorize us to grant restricted stock. To date, we granted under our 2002 plan 13,333 shares of restricted stock to Mr. Sblendorio, our audit committee chairman, and 40,000 shares of restricted stock to Mr. Dentzer. While we have no current plans to grant restricted stock under our 2007 plan, we may choose to do so in order to implement the long-term incentive goals of the compensation committee.

Other Compensation. Consistent with our compensation philosophy, we intend to continue to maintain our current benefits for our executive officers, including medical, dental, vision and life insurance coverage; however, the compensation committee in its discretion may revise, amend or add to the officer's executive benefits if it deems it advisable. Due to the extraordinary medical needs of the family of our chief executive officer, we do maintain an executive medical reimbursement contract. Under this contract, Messrs. Crowley and Patterson, Drs. Licholai and Palling and Ms. Schaeffer are entitled to the reimbursement of medical expenses, subject to certain limitations. We have no current plans to change the levels of benefits currently provided to our executives.

Termination Based Change of Control Compensation. Upon termination of employment under certain circumstances, our executive officers are entitled to receive varying types of compensation. Elements of this compensation may include payments based upon a number of months of base salary, bonuses amounts, acceleration of vesting of equity, and health and other similar benefits. We believe that our termination-based compensation and acceleration of vesting of equity arrangements are in line with severance packages offered to executives of other similar companies, including our package for our chief executive officer, based upon the market information we have reviewed. We also have granted severance and acceleration of vesting of equity benefits to our executives in the event of a change of control if the executive is terminated within a certain period of time of the change of control. We believe this "double trigger" requirement maximizes shareholder value because it prevents an unintended windfall to management in the event of a friendly or non-hostile change of control. Under this structure, unvested equity awards would continue to incentivize our executives to remain with the company after a change of control, and more appropriate than a single trigger acceleration mechanism contingent only upon a change of control. The specifics of each executive officer's arrangements is described in further detail below.

Relationship of Elements of Compensation. Our compensation structure is primarily weighted toward three of the elements discussed: base salary, annual performance bonus, and stock options. We utilize stock options as a substantial component of compensation because we currently have no revenue or earnings and expect this to be the case for the foreseeable future. Our mix of cash and non-cash compensation balances our need to limit cash expenditures with the expectations of those we hope to recruit and retain as employees. In the future, we may adjust the mix of cash and non-cash compensation if required by competitive market conditions for attracting and retaining skilled personnel.

We manage the expected impact of salary increases and performance bonuses by requiring that the size of such salary increases and bonuses be tied to the attainment of corporate and individual objectives. For example, the size of each employee's bonus is determined not only by individual performance, but also by whether the company has met corporate objectives.

We view the award of stock options as a primary long-term retention benefit. We make the award of stock options a significant component of total compensation and also tie the earning of these awards to long-term vesting schedules, generally four years. If an employee leaves our employ before the completion of the vesting period, then that employee would not receive any benefit from the non-vested portion of his award. We believe this feature makes it more attractive to remain as our employee and these arrangements do not require substantial cash payments by us.

Executive Compensation

Summary Compensation Table

The following table provides information regarding the compensation that we paid to each person serving as our chief executive officer and our chief financial officer, during the fiscal year ended December 31, 2006 and each of our other three most highly paid executive officers serving as of December 31, 2006 as well as one additional individual who could have been one of the three most highly paid executive officers had he been employed as of December 31, 2006. We use the term "named executive officers" to refer to these people later in this prospectus.

Name and Principal Position	Year	Salary ($)	Bonus[1] ($)	Stock Awards ($)	Option Awards[2] ($)	All Other Compensation ($)	Total ($)
John F. Crowley President and Chief Executive Officer	2006	$400,000	$210,667	—	$ 876,041	$ 659,963[3]	$2,146,671
Donald J. Hayden, Jr.[4] Chairman and Interim President and Chief Executive Officer	2006	145,705[5]	30,000[6]	—	207,887[7]	—	383,592
James E. Dentzer Chief Financial Officer	2006	70,000[8]	84,000	22,875	13,822	299,461[9]	490,158
John M. McAdam[10] Principal Financial Officer	2006	110,000	40,450	—	19,824	—	170,274
Joseph Warusz[11] Vice President, Finance	2006	48,094	—	—	161,368	124,887	334,349
Matthew R. Patterson Chief Operating Officer	2006	280,673	65,267	—	85,430	—	431,370
David Lockhart, Ph.D. Chief Scientific Officer	2006	280,000	66,547	—	316,375	94,926[12]	757,848
David Palling, Ph.D. Senior Vice President, Drug Development	2006	236,250	40,163	—	35,495	—	311,908
Pedro Huertas, M.D., Ph.D.[13] Chief Strategic Officer	2006	281,875	70,469	—	289,043	191,255[14]	832,642

(1) Represents bonuses earned in 2006 and paid in 2007.
(2) The value of each of the option awards was computed in accordance with FAS 123(R) for 2006 without consideration of forfeitures. Valuation assumptions are described in the notes to financial statements appearing elsewhere in this prospectus. Options generally vest over a four year period.
(3) Includes $214,440 of payments made in connection with executive medical reimbursement, $256,620 for health insurance premiums for Mr. Crowley's family and $188,903 for reimbursement of taxes.
(4) Mr. Hayden served as interim president and chief executive officer from September 11, 2006, until March 5, 2007.
(5) This amount includes all compensation paid to Mr. Hayden in 2006 and consists of $61,538 for his service as interim president and chief executive officer from September 11, 2006 until March 5, 2007, $25,000 for consulting services provided to us by him from February 28, 2006 to June 27, 2006, and $59,167 for his service as the chairman of the board of directors.
(6) This bonus amount was awarded to Mr. Hayden solely for his service to us as our interim president and chief executive officer.
(7) This amount is the value of the 13,334 common stock options granted to Mr. Hayden for his service as our interim president and chief executive officer, as well as the 66,667 common stock options granted to him in February 2006 for his service to us as the chairman of the board of directors.
(8) Mr. Dentzer began serving as our chief financial officer in October 2006.
(9) Consists of $199,461 of relocation expenses and a $100,000 signing bonus.
(10) Mr. McAdam has served as our Controller since March 2006. He also served as our Interim Principal Accounting and Principal Financial Officer from March 2006 to September 2006.
(11) Mr. Warusz's employment with us ended in March 2006. Other compensation consists of severance and salary continuance payments made to him during 2006 in connection with his departure.
(12) Includes $20,000 of signing bonus, $31,579 of relocation expenses, $25,550 for commuting expenses, and $17,797 for reimbursement of taxes.
(13) Dr. Huertas' employment with us ended on December 31, 2006.
(14) Other compensation consists of $140,938 for accrued severance, $37,183 for relocation expenses, and $13,134 for commuting expenses relating to Dr. Huertas' service with the Company through the end of 2006.

Grants of Plan-Based Awards

The following table presents information concerning grants of plan-based awards to each of the named executive officers during 2006.

Name and Principal Position	Grant Date	Performance-Based Stock Incentive Plans: Number of Restricted Stock Awards (#)	All Other Option Awards: Number of Securities Underlying Options (#)	Exercise or Base Price of Option or Stock Awards ($/Sh)	Grant Date Fair Value of Stock and Option Awards[1] ($)
John F. Crowley President and Chief Executive Officer	2/28/2006	—	280,000[2]	$ 5.33	$3,189,063
Donald J. Hayden, Jr. Chairman and Interim President and Chief Executive Officer	2/28/2006 9/13/2006	—	66,667[3] 13,334[4]	5.33 8.18	759,301 74,549
James E. Dentzer Chief Financial Officer	10/2/2006 10/2/2006	 40,000[5]	33,334[2] —	9.15 9.15	221,157 366,000
John M. McAdam Principal Financial Officer	2/28/06 3/27/06 5/15/06	—	2,000[2] 6,667 1,334	5.33 5.33 8.18	22,763 75,947 7,892
Joseph Warusz[6] Vice President, Finance	—	—	—	—	—
Matthew R. Patterson Chief Operating Officer	2/28/2006	—	33,334[2]	5.33	379,650
David Lockhart, Ph.D. Chief Scientific Officer	2/28/2006 2/28/2006	 —	100,000[2] 33,334	5.33 5.33	1,138,951 379,650
David Palling, Ph.D. Senior Vice President, Drug Development	2/28/2006	—	2,667[2]	5.33	30,372
Pedro Huertas, M.D., Ph.D.[7] Chief Strategic Officer	2/28/2006	—	20,000[2]	5.33	227,790

(1) The value of restricted stock and option awards granted to our named executive officers was computed in accordance with FAS 123(R) without consideration of forfeitures. Valuation assumptions are described in the notes to financial statements appearing elsewhere in this prospectus.

(2) The option has a term of ten years and vests in accordance with the following schedule: 25% of the total number of shares vest on the first anniversary of the Grant Date and 1/48th of the total number of shares vest on the first day of each calendar month following the grant date.

(3) The option to purchase 66,667 shares of common stock granted to Mr. Hayden was for his service as a director of the company, has a term of ten years and vests in accordance with the following schedule: 25% of the total number of shares vest on the first anniversary of the Grant Date and 1/48th of the total number of shares vest on the first day of each calendar month following the grant date.

(4) The option to purchase 13,334 shares of common stock granted to Mr. Hayden was for his service as our interim president and chief executive officer and vested entirely on completion of his service under his Employment Agreement on March 5, 2007.

(5) The award of 40,000 shares of restricted stock granted to Mr. Dentzer vests in accordance with the following schedule: 25% of the total number of shares vest on the first anniversary of the grant date and 1/48th of the total number of shares vest on the first day of each calendar month following the grant date.

(6) Mr. Warusz's employment with us ended in March 2006.

(7) Mr. Huertas' employment with us ended on December 31, 2006.

Outstanding Equity Awards at Fiscal Year-End

The following table presents the outstanding equity awards held by each of the named executive officers as of December 31, 2006.

	Option Awards				Stock Awards	
Name and Principal Position	Number of Securities Underlying Unexercised Options (#) Exercisable	Number of Securities Underlying Unexercised Options (#) Unexercisable	Option Exercise Price ($)	Option Expiration Date	Number of Shares or Units of Stock That Have Not Vested (#)	Market Value of Shares or Units of Stock That Have Not Vested ($)
John F. Crowley	57,426	162,410(1)	$ 0.638	1/6/2015	—	—
President and Chief Executive	7,328	9,162(1)	0.638	8/17/2014	—	
Officer	29,166	70,834(1)	5.33	10/20/2015	—	—
	—	280,000(1)	5.33	2/28/2016	—	
Donald J. Hayden, Jr.	—	66,667(1)	5.33	2/28/2016	—	—
Interim President and Chief	—	—	—	—		
Executive Officer	—	13,334(2)	8.18	9/13/2016	—	—
	—	—	—	—	—	—
James E. Dentzer	—	33,334(1)	8.18	10/2/2016	40,000(5)	396,000
Chief Financial Officer	—	—	—	—	—	—
John M. McAdam	—	2,000(1)	5.33	2/28/2016	—	—
Principal Financial Officer	—	6,667(1)	5.33	3/27/2016	—	—
	—	1,334(1)	8.18	5/15/2016	—	—
Joseph Warusz(3)	—	—	—	—	—	—
Vice President, Finance	—	—	—	—	—	—
Matthew R. Patterson	16,275	48,272(1)	0.638	12/15/2014	—	—
Chief Operating Officer	10,695	25,972(1)	5.33	10/20/2015	—	—
	—	33,333(1)	5.33	2/28/2016	—	—
David Lockhart, Ph.D.	—	100,000(1)	5.33	2/28/2016	—	—
Chief Scientific Officer	—	33,334(1)	5.33	2/28/2016	—	—
David Palling, Ph.D.	1,334	— (1)	0.075	8/12/2012	—	—
Senior Vice President,	2,667	334(1)	0.563	1/20/2014	—	—
Drug Development	8,076	19,181(1)	0.638	12/15/2014	—	—
	8,750	21,250(1)	5.33	10/20/2015	—	—
	—	2,667	5.33	2/28/2016	—	—
Pedro Huertas, M.D., Ph.D.(4)	58,332	— (1)	0.638	6/19/2015	—	—
Chief Strategic Officer	10,834	— (1)	5.33	10/20/2015	—	—
	9,167	— (1)	5.33	2/28/2016	—	—

(1) 25% of the total number of shares subject to the option vest at the end of the first year, the remainder vest 1/36th per month thereafter.
(2) 100% vested on March 5, 2007 due to the termination of his service as our interim president and chief executive officer.
(3) Mr. Warusz's employment with us ended in March 2006.
(4) Mr. Huertas' employment with us ended on December 31, 2006.
(5) 25% of the total number of shares vest on the first anniversary of the grant date and 1/48th of the total number of shares vest on the first day of each calendar month following the grant date.

Option Exercises and Stock Vested at Fiscal Year End

The following table presents certain information concerning the exercise of options by each of the named executive officers during the fiscal year ended December 31, 2006.

	Option Awards		Stock Awards	
Name and Principal Position	**Number of Shares Acquired on Exercise (#)**	**Value Realized on Exercise(1) ($)**	**Number of Shares Acquired on Vesting (#)**	**Value Realized on Vesting ($)**
John F. Crowley President and Chief Executive Officer	80,000	$ 1,053,000	—	—
Donald J. Hayden, Jr. Chairman and Interim President and Chief Executive Officer	—	—	—	—
James E. Dentzer Chief Financial Officer	—	—	—	—
John M. McAdam Principal Financial Officer	—	—	—	—
Joseph Warusz(2) Vice President, Finance	9,722	73,238	—	—
Matthew R. Patterson Chief Operating Officer	32,000	241,200	—	—
David Lockhart, Ph.D. Chief Scientific Officer	—	—	—	—
David Palling, Ph.D. Senior Vice President, Drug Development	48,866	376,577	—	—
Pedro Huertas, M.D., Ph.D.(3) Chief Strategic Officer	—	—	—	—

(1) Value Realized on Exercise is the difference between the aggregate exercise price and the aggregate fair value or retrospectively determined fair value for financial reporting purposes at the date of exercise. Our methodology for determining fair value and retrospectively determined fair value for reporting purposes is described in Management's Discussion and Analysis of Financial Condition and Results of Operation.
(2) Mr. Warusz's employment with us ended in March 2006.
(3) Mr. Huertas' employment with us ended on December 31, 2006.

Pension Benefits

None of our named executive officers participates in or has account balances in qualified or non-qualified defined benefit plans sponsored by us.

Nonqualified Deferred Compensation

None of our named executive officers participate in or have account balances in non-qualified defined contribution plans or other deferred compensation plans maintained by us. The compensation committee, which is comprised solely of independent directors, may elect to provide our officers and other employees with non-qualified defined contribution or deferred compensation benefits if the compensation committee determines that doing so is in our best interests.

Severance Benefits and Change of Control Arrangements

We have agreed to provide severance benefits and change of control arrangements to our current executives, as described below.

John F. Crowley. We employ Mr. Crowley as our president and chief executive officer pursuant to an employment agreement. 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 of his employment 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. We believe that the severance package for our chief executive officer is in line with severance packages offered to chief executive officers of comparable companies as represented by compensation data we have reviewed.

Other Executive Officers. We have entered into severance agreements with the following executive officers: Matthew R. Patterson, James E. Dentzer, David Lockhart, Ph.D., Karin Ludwig, M.D., Mark Simon, David Palling, Ph.D., Gregory P. Licholai, M.D., S. Nicole Schaeffer, Bradley L. Campbell and Douglas A. Branch. If any of Drs. Lockhart and Ludwig or Messrs. Dentzer, 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 or restricted stock awards then held by them will automatically accelerate by six months. If any of Dr. Palling, Dr. Licholai, Ms. Schaeffer or Messrs. Branch or Campbell is terminated without cause, we will be obligated to pay that executive six months of base salary following termination. In addition, if any of our executive officers is terminated other than for cause within six months following certain corporate changes or if, following those changes, the executive resigns for good reason, then 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;
- payment of a bonus equal to the bonus earned in the preceding year; and
- any outstanding unvested stock options or other equity based compensation 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. We believe that the severance packages for our executive officers are consistent with severance packages offered to executive officers of comparable companies as represented by compensation data we have reviewed.

Joseph Warusz and Pedro Huertas, M.D., Ph.D., each of whom are former executive officers, had agreements with us that contained provisions relating to severance benefits. Upon his departure in March 2006, Mr. Warusz was paid cash severance in the form of continuing base salary for six months. We are required to make cash payments to Dr. Huertas in the form of continuing base salary until June 30, 2007. In addition, we paid Dr. Huertas $70,469 in connection with his departure. We also accelerated all unvested options held by Dr. Huertas that would have become vested on or prior to December 31, 2007.

Potential Payments Upon Termination Without Cause

The following table sets forth quantitative estimates of the benefits that would have accrued to each of our named executive officers if his employment had been terminated without cause or was terminated upon a change in control on December 31, 2006. Amounts below reflect potential payments pursuant to the employment agreements for such named executive officers.

Name and Principal Position	Salary Continuation ($)	Bonus ($)	Benefit Continuation ($)	Value of Accelerated Option Vesting ($)
John F. Crowley President and Chief Executive Officer	$ 600,000	$300,000	$ 940,230(1)	$ 1,446,724
Donald J. Hayden, Jr. Chairman and Interim President and Chief Executive Officer	33,333	—	—	23,000
James E. Dentzer Chief Financial Officer	140,000	—	—	—
John M. McAdam Principal Financial Officer	—	—	—	—
Joseph Warusz(2) Vice President, Finance	—	—	—	—
Matthew R. Patterson Chief Operating Officer	150,000	62,500	—	185,494
David Lockhart, Ph.D. Chief Scientific Officer	280,000	—	—	203,333
David Palling, Ph.D. Senior Vice President, Drug Development	236,250	—	—	—
Pedro Huertas, M.D., Ph.D.(3) Chief Strategic Officer	140,000	70,469	—	288,378

(1) Benefits to be continued consist of healthcare costs and health insurance premiums for Mr. Crowley's family.
(2) Mr. Warusz's employment with us ended in March 2006.
(3) Dr. Huertas' employment with us ended on December 31, 2006.

Potential Payments Upon Termination Due to Change in Control

The following table sets forth quantitative estimates of the benefits that would have accrued to each of our named executive officers if his employment had been terminated without cause or due to constructive termination upon a change in control on December 31, 2006, assuming that such termination occurred within the period beginning on the first day of the calendar month immediately preceding the calendar month in which the effective date of a change in control occurs and ending on the last day of the twelfth calendar month following the calendar month in which the effective date of a change in control occurs. Amounts below reflect potential payments pursuant to the amended employment agreements for such named executive officers.

Name and Principal Position	Salary Continuation ($)	Bonus ($)	Benefit Continuation ($)	Value of Accelerated Equity Vesting ($)
John F. Crowley President and Chief Executive Officer	$ 800,000	$400,000	$ 1,253,640(1)	$ 3,136,391
Donald J. Hayden, Jr. Interim President and Chief Executive Officer	—	—	—	—
James E. Dentzer Chief Financial Officer	280,000	—	—	421,000
John M. McAdam Principal Financial Officer	—	—	—	—
Joseph Warusz(2) Vice President, Finance	—	—	—	—
Matthew R. Patterson Chief Operating Officer	300,000	62,500	—	722,896
David Lockhart, Ph.D. Chief Scientific Officer	20,000	—	—	610,000
David Palling, Ph.D. Senior Vice President, Drug Development	23,250	56,250	—	318,193
Pedro Huertas, M.D., Ph.D.(3) Chief Strategic Officer	—	—	—	—

(1) Benefits to be continued consist of healthcare costs and health insurance premiums for Mr. Crowley's family.
(2) Mr. Warusz's employment with us ended in March 2006.
(3) Mr. Huertas' employment with us ended on December 31, 2006.

Confidential Information and Inventions Agreement

Each of our named executive officers has also entered into a standard form agreement with respect to confidential information and inventions. Among other things, this agreement obligates each named executive officer to refrain from disclosing any of our proprietary information received during the course of employment and to assign to us any inventions conceived or developed during the course of employment.

Director Compensation

In June, 2006, our board of directors adopted a compensation program for our non-employee directors, or the Director Compensation Policy. Pursuant to the Director Compensation Policy, each member of our board of directors who is not our employee receives the following cash compensation for board services, as applicable:

- $45,000 per year for service as chairman;

- $20,000 per year for service as a board member;
- $30,000 per year for service as chairperson of the audit committee;
- $30,000 for service as a financial expert;
- $20,000 per year each for service as chairperson of the compensation committee or the nominating/corporate governance committee; and
- $10,000 per year for service as a member of the audit committee and $5,000 per year for service as a member of the compensation committee or the nominating/corporate governance committee.

In November 2006, all directors who represented holders of our preferred stock declined receiving compensation under the Director Compensation Policy. Upon completion of this offering, we anticipate that those directors will elect to resume their compensation.

Summary Director Compensation Table

The following table provides information regarding the compensation that we paid to each of our directors during the fiscal year ended December 31, 2006, other than those directors included in the Summary Compensation Table above.

Name	Total ($)	Fees Earned or Paid in Cash(1) ($)	Stock Awards(2) ($)	Option Awards ($)	Non-Incentive Plan Compensation ($)	All Other Compensation ($)
Glenn P. Sblendorio	$ 149,000	$ 40,000	$ 18,167	—	—	—
Alexander E. Barkas, Ph.D.(3)	6,250	6,250	—	—	—	—
Michael G. Raab(3)	8,750	8,750	—	—	—	—
James N. Topper, M.D., Ph.D(3)	6,250	6,250	—	—	—	—
Stephen Bloch, M.D.(3)	7,500	7,500	—	—	—	—
Gregory M. Weinhoff, M.D. (3)	6,250	6,250	—	—	—	—
P. Sherrill Neff(3)	10,000	10,000	—	—	—	—

(1) Represents fees paid pursuant to Director Compensation Policy.
(2) The restricted stock award vests in 36 equal monthly installments.
(3) Commencing in November 2006, declined to accept any fees until we completed an initial public offering.

The exercise price of each option granted to a non-employee director will be equal to 100% of the fair market value on the date of grant of the shares covered by the option. Options will have a maximum term of 10 years measured from the grant date, subject to termination in the event of the optionee's cessation of board service.

Following the completion of this offering, all of our directors will be eligible to participate in our 2007 equity incentive plan. For a more detailed description of these plans, see "Employee Benefit and Stock Plans" appearing elsewhere in this prospectus.

Employment Agreements

John F. Crowley. 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 also provides that Mr. Crowley's compensation and benefits, including health benefits for him and his family, continue in full

during the term of any active duty service, and Mr. Crowley received full compensation and benefits during his active duty service from September 2006 to March 2007. The agreement further provides that Mr. Crowley is eligible to participate in any executive bonus plans established by the board from time to time. 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.

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 also provides for severance benefits and change of control arrangements as previously described in detail.

Other Executive Officers. We have entered into employment agreements with the following executive officers: James E. Dentzer, Matthew R. Patterson, David Lockhart, Ph.D., Karin Ludwig, M.D., Mark Simon, David Palling, Ph.D., Gregory P. Licholai, M.D., S. Nicole Schaeffer, Bradley L. Campbell and Douglas A. Branch. These agreements set forth the officer's position, duties, base salary and benefits, and severance arrangements as previously described in detail. Our executive employment agreements with Drs. Lockhart and Ludwig and Messrs. Patterson, Simon and Dentzer 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. Campbell and Branch have no term and are "at will".

Employee Benefit and Stock Plans

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 April 25, 2007, there were options to purchase 2,549,950 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.

2007 Equity Incentive Plan

Our board of directors in April 2007 and our stockholders in May 2007 approved our 2007 equity incentive plan, to become effective on the closing of this offering. The 2007 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 are issuable upon stock options granted under the 2007 equity incentive plan is 966,667, which number will be increased annually by the lesser of (a) 26,667 shares of common stock and (b) one percent (1%) of our outstanding equity on a fully diluted basis as of the end of the immediately preceding fiscal year. The aggregate number of shares of common stock that

may be granted in any calendar year to any one person pursuant to the 2007 equity incentive plan may not exceed 50% of the aggregate number shares of our common stock that may be issued pursuant to the 2007 equity incentive plan.

The 2007 equity incentive plan will be administered by the compensation committee of our board of directors. Subject to the provisions of the 2007 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, restricted stock units 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 2007 equity incentive plan and adopt rules and regulations thereunder. Generally, options granted under the 2007 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 2007 equity incentive plan at any time, subject to applicable rules and law and the rights of holders of outstanding awards. Our 2007 equity incentive plan will automatically terminate in April 2017 unless our board of directors terminates it prior to that time.

2007 Director Option Plan

In May 2007, our board of directors and stockholders approved the 2007 director option plan, to become effective on the closing of this offering. The 2007 director option plan provides for the automatic annual grant of stock options to our directors who are not our employees in order to promote the retention of highly qualified directors. The aggregate number of shares of our common stock that are issuable upon stock options granted under the 2007 director option plan is 200,000, which number will be increased annually on January 1 of each year, from 2008 and until 2017, by the lesser of (a) 66,667 shares of common stock or (b) one fourth of one percent, or 0.25%, of our outstanding equity on a fully diluted basis as of the end of the immediately preceding fiscal year. The 2007 director option plan will be administered by a committee appointed by our board of directors.

The 2007 director option plan provides that each director shall automatically receive an annual grant of options to purchase 10,000 shares at our annual meeting of stockholders, which grant will generally vest in full at the next annual stockholders meeting. Notwithstanding the foregoing, our board of directors may be resolution prior to such annual meeting rescind, reduce or increase the size of these annual grants.

In the event of a merger in which our stockholders before the merger own less than 50% of our voting power after the merger or a person or group acquire more than 20% of our outstanding capital stock after this offering, all outstanding options that are not exercisable in full at that time shall accelerate and the committee shall have the right to provide for either: (1) the assumption of, or substitution of outstanding options with equivalent options, by the acquiring entity or (2) the termination of all options that remain outstanding at that time. In the event that outstanding options are terminated, the compensation committee may determine that we need to make payments to the holders of such terminated options.

No awards may be granted under our 2007 director option plan after May 2017, unless our board of directors terminates the plan prior to that time.

2007 Employee Stock Purchase Plan

In May 2007, both our board of directors and our stockholders approved our 2007 employee stock purchase plan, to become effective upon the closing of this offering. The 2007 employee stock purchase plan authorizes the issuance of up to an aggregate of 200,000 shares of our common stock to eligible employees. The 2007 employee stock purchase plan will automatically terminate ten years after the effective date of this offering, unless our board of directors terminates it prior to that time. The 2007 employee stock purchase plan will be administered by the board of directors or by a committee appointed by the board.

The 2007 employee stock purchase plan, which is intended to qualify under Section 423 of the Internal Revenue Code, will be implemented through a series of offering periods. The duration of each offering period

(not to exceed 24 months) will be determined by our board of directors or the committee appointed by the board. Each offering period will consist of consecutive (or one) three-month purchase periods commencing on the first business day of July, October, January or April each year. At the end of each three-month purchase period an automatic purchase will be made for participants. 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 must be at least 1% but may not exceed 15% of an employee's compensation. Such purchases will be made at a price equal to the lower of 85% of the fair market value of the common stock at the end of each three-month purchase period or 85% of the higher of the fair market value of the common stock at the beginning of such three-month purchase period or at the beginning of the offering period.

Under the 2007 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 2007 employee stock purchase plan provides that no employee shall be granted an option 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. The board of directors may, at its discretion, prior to the beginning of a purchase period, subject the shares acquired (or to be acquired) by employees for such purchase period to transfer restrictions.

In the event of a merger or consolidation of us with and into another person or entity or the sale of transfer of all or substantially all of our assets, each right to purchase stock under the 2007 employee stock purchase plan will be assumed, or an equivalent right will be substituted by, the successor corporation. In the event that the successor corporation refuses to assume each purchase right or to substitute an equivalent right, any ongoing offering period will be shortened so that employees' rights to purchase stock under the 2007 employee stock purchase plan are exercised prior to the transaction, unless the employee has withdrawn. The board of directors has the power to amend or terminate the 2007 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; provided, however, that our board of directors may amend or terminate the 2007 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 or if the board determines that termination of the plan and/or offering period is in our best interest and the best interest of our stockholders. The board has not set an initial offering period under the plan but has the discretion to do so in the future.

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.

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.

PRINCIPAL STOCKHOLDERS

The following table sets forth information with respect to the beneficial ownership of our common stock, as of April 25, 2007, 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 17,234,426 shares of our common stock outstanding on April 25, 2007, assuming the conversion of all outstanding shares of our redeemable convertible preferred stock into 16,071,924 shares of our common stock upon the closing of this offering. The column entitled "Percentage of Shares Beneficially Owned — After Offering" is based on 22,234,426 shares of common stock to be outstanding after this offering, including the 5,000,000 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 April 25, 2007 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 New Enterprise Associates[1] 1119 St. Paul Street Baltimore, MD 21202	4,483,582	26.2%	20.3%
Entities affiliated with Frazier Healthcare Ventures[2] 601 Union, Two Union Square, Suite 3200 Seattle, WA 98101	2,600,014	15.2%	11.8%
Entities affiliated with Prospect Venture Partners II, L.P.[3] 435 Tasso Street, Suite 200 Palo Alto, CA 94301	2,240,752	13.1%	10.1%
Entities affiliated with CHL Medical Partners[4] 1055 Washington Boulevard, 6th Floor Stamford, CT 06901	2,108,554	12.3%	9.5%
Entities affiliated with Canaan Partners[5] 285 Riverside Avenue, Suite 250 Westport, CT 06880	2,050,790	12.0%	9.3%
Entities affiliated with Quaker BioVentures[6] Cira Center 2929 Arch Street Philadelphia, PA 19104-2868	1,419,762	8.3%	6.4%

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)	331,727	1.9%	1.5%
David Palling, Ph.D.(8)	80,778	*	*
Matthew R. Patterson(9)	86,733	*	*
Gregory P. Licholai, M.D.(10)	66,390	*	*
James E. Dentzer	-0-	*	*
S. Nicole Schaeffer(11)	25,317	*	*
David Lockhart, Ph.D.(12)	46,528	*	*
Karin Ludwig, M.D.(13)	22,223	*	*
Mark Simon	-0-	*	*
Bradley L. Campbell	-0-	*	*
Douglas A. Branch(14)	13,333	*	*
Pedro Huertas, M.D., Ph.D.	80,997	*	*
Joseph Warusz	9,722	*	*
John McAdam(15)	3,113	*	*
Donald J. Hayden, Jr.(16)	35,556	*	*
Alexander E. Barkas, Ph.D.(3)	2,240,752	13.1%	10.1%
Michael G. Raab(1)	4,483,582	26.2%	20.3%
James N. Topper, M.D., Ph.D.(2)	2,600,014	15.2%	11.8%
Glenn P. Sblendorio(17)	4,445	*	*
Stephen Bloch, M.D.(18)	2,050,790	12.0%	9.3%
Gregory M Weinhoff, M.D.(19)	2,108,554	12.3%	9.5%
P. Sherrill Neff(6)	1,419,762	8.3%	6.4%
All directors and executive officers as a group (22 persons)(20)	15,710,316	88.7%	69.2%

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

(1) Consists of 3,659,157 shares held of record by New Enterprise Associates 11, Limited Partnership (including 8,669 shares to be acquired immediately prior to the closing of this offering as a result of the net exercise of outstanding warrants at the initial public offering price of $15.00 per share), 2,689 shares held of record by NEA Ventures 2004, Limited Partnership (including 23 shares to be acquired immediately prior to the closing of this offering as a result of the net exercise of outstanding warrants at the initial public offering price of $15.00 per share), and 821,736 shares held of record by New Enterprise Associates 9, Limited Partnership. Voting and investment power over the shares held by NEA Ventures 2004, Limited Partnership is exercised by J. Daniel Moore, its general partner. Voting and investment power over the shares held by New Enterprise Associates 9, Limited Partnership is exercised by NEA Partners 9, Limited Partnership, its general partner. The individual general partners of NEA Partners 9, Limited Partnership are C. Richard Kramlich, Peter J. Barris, Charles W. Newhall, III, Mark W. Perry and John M. Nehra. Voting and investment power over the shares held by New Enterprise Associates 11, Limited Partnership is exercised by NEA Partners 11, Limited Partnership, its general partner. The general partner of NEA Partners 11, Limited Partnership is NEA 11 GP, LLC. The individual managers of NEA 11 GP, LLC are C. Richard Kramlich, Peter J. Barris, Forest Baskett, Charles W. Newhall, III, Mark W. Perry, Scott D. Sandell, Eugene A. Trainor, III, Charles M. Linehan, Ryan D. Drant, Krishna “Kittu” Kolluri and M. James Barrett. 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 9, Limited Partnership, New Enterprise Associates 11, Limited Partnership or NEA Ventures 2004, Limited Partnership and he disclaims beneficial ownership of shares held by New Enterprise Associates 9, Limited Partnership, New Enterprise Associates 11, Limited Partnership and NEA Ventures 2004, Limited Partnership, except to the extent of his pecuniary interest therein.

(2) Consists of 2,586,886 shares held of record by Frazier Healthcare IV, L.P. (including 15,042 shares to be acquired prior to the closing of this offering as a result of the exercise for cash of outstanding warrants) and 13,128 shares held of record by Frazier Affiliates IV, L.P. (including 76 shares to be acquired prior to the closing of this offering as a result of the exercise for cash of outstanding warrants). 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.

(3) Consists of 2,207,144 shares held of record by Prospect Venture Partners II, L.P. (including 8,562 shares to be acquired immediately prior to the closing of this offering as a result of the net exercise of outstanding warrants at the initial public offering price of $15.00 per share), and 33,608 shares held of record by Prospect Associates II, L.P. (including 130 shares to be acquired immediately prior to the closing of this offering as a result of the net exercise of outstanding warrants at the initial public offering price of $15.00 per share). 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.

(4) Consists of 1,975,455 shares held of record by CHL Medical Partners II, L.P. and 133,099 shares held of record by CHL Medical Partners II Side Fund, L.P. Voting and investment power over the shares held by each of the partnerships constituting CHL Medical Partners is exercised by Collinson Howe & Lennox II, L.L.C. in its role as general partner and investment advisor to the partnerships. The members of Collinson Howe & Lennox II, L.L.C. are Jeffrey J. Collinson, Myles D. Greenberg, Timothy F. Howe, Ronald W. Lennox, and Gregory M. Weinhoff, a member of our board of directors. Each of these members disclaims beneficial ownership of these shares except to the extent of his proportionate pecuniary interest therein.

(5) Consists of 1,976,967 shares held of record by Canaan Equity III, L.P. (including 7,859 shares to be acquired immediately prior to the closing of this offering as a result of the net exercise of outstanding warrants at the initial public offering price of $15.00 per share), and 73,823 shares held of record by Canaan Equity III Entrepreneurs, LLC (including 293 shares to be acquired immediately prior to the closing of this offering as a result of the net exercise of outstanding warrants at the initial public offering price of $15.00 per share). 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, LLC are John V. Balen, 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 1,064,822 shares held of record by Quaker BioVentures, L.P. and 354,940 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 Venture 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 185,061 shares issuable upon the exercise of stock options exercisable within 60 days of April 25, 2007, and 146,666 shares held of record. Includes 13,333 shares held of record by MPAJ, LLC, for which Mr. Crowley has sole voting and dispositive power, 60,000 shares held of record by Aileen A. Crowley 2007 Grantor Retained Annuity Trust, and 73,333 shares held of record by John F. Crowley 2007 Grantor Retained Annuity Trust. Mr. Crowley is the sole trustee of the John F. Crowley 2007 Grantor Retained Annuity Trust and exercises voting and investment power over its shares. Mr. Crowley disclaims beneficial ownership of the shares held by the Aileen A. Crowley 2007 Grantor Retained Annuity Trust.

(8) Consists of 25,246 shares issuable upon the exercise of stock options exercisable within 60 days of April 25, 2007, and 55,532 shares held of record.

(9) Consists of 54,733 shares issuable upon the exercise of stock options exercisable within 60 days of April 25, 2007, and 32,000 shares held of record.

(10) Consists of 39,572 shares issuable upon the exercise of stock options exercisable within 60 days of April 25, 2007, and 26,818 shares held of record. Includes 6,666 shares held of record by the Gregory P. Licholai 2006 Grantor Retained Annuity Trust, for which Mr. Licholai has sole voting and dispositive power.

(11) Consists of 12,522 shares issuable upon the exercise of stock options exercisable within 60 days of April 25, 2007, and 12,795 shares held of record.

(12) Consists of 46,528 shares issuable upon the exercise of stock options exercisable within 60 days of April 25, 2007.

(13) Consists of 22,223 shares issuable upon the exercise of stock options exercisable within 60 days of April 25, 2007.

(14) Consists of 13,333 shares issuable upon the exercise of stock options exercisable within 60 days of April 25, 2007.

(15) Consists of 3,113 shares issuable upon the exercise of stock options exercisable within 60 days of April 25, 2007.

(16) Consists of 35,556 shares issuable upon the exercise of stock options exercisable within 60 days of April 25, 2007.

(17) Consists of 4,445 shares of restricted stock which vest within 60 days of April 25, 2007.

(18) Consists of shares beneficially owned by entities affiliated with Canaan Partners, as described in footnote (5) above. 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.

(19) Consists of shares beneficially owned by entities affiliated with CHL Medical Partners, as described in footnote (4) above. 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.

(20) Consists of 437,887 total shares issuable upon the exercise of stock options exercisable within 60 days of April 25, 2007, warrants to purchase 40,654 shares of Series B redeemable convertible preferred stock and 15,231,775 total shares held of record.

CERTAIN RELATIONSHIPS AND RELATED PARTY TRANSACTIONS

Since January 1, 2004, 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.

On August 24, 2006, our board of directors adopted a formal policy such that all transactions between us and our officers, directors, principal stockholders and their affiliates must be approved by a majority of the members of the board of directors, including a majority of the independent and disinterested members of the board of directors, and that such transactions must be on terms no less favorable to us than those that could be obtained from unaffiliated third parties. We do not intend at this time to adopt specific standards for the approval of these transactions, but instead intend to have our board of directors review all such transactions on a case by case basis. Prior to August 24, 2006, although there was no formal policy, approval of the board of directors was obtained for all related party transactions.

Private Placement of Securities

In May 2004 and April 2005, we issued an aggregate of 4,862,734 shares of our series B redeemable convertible preferred stock at a price of $6.38 per share, along with warrants entitling the holders to purchase an aggregate of 73,996 shares of our series B redeemable convertible preferred stock at a price of $6.38 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 5,820,020 shares of our series C redeemable convertible preferred stock at a price of approximately $9.45 per share for total cash proceeds to us of approximately $55.0 million before transaction expenses.

In September 2006 and March 2007, we issued an aggregate of 4,930,405 shares of our series D redeemable convertible preferred stock at a price of approximately $12.17 per share for total cash proceeds to us of approximately $60.0 million before transaction expenses.

The following table sets forth the number of shares of series B redeemable convertible preferred stock, series C redeemable convertible preferred stock and Series D 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, series C redeemable convertible preferred stock and Series D 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	Number of Shares of Series D Redeemable Convertible Preferred Stock
Entities affiliated with Prospect Venture Partners(1)	993,464	1,016,220	222,376
Entities affiliated with New Enterprise Associates(2)	993,462	1,016,220	2,465,208
Entities affiliated with Frazier Healthcare Ventures(3)	993,462	1,016,220	575,214
Entities affiliated with Canaan Partners(4)	931,762	907,498	203,378
Entities affiliated with CHL Medical Partners(5)	796,247	529,098	205,434
Entities affiliated with Quaker BioVentures(6)	—	1,058,200	361,562
Total	4,708,397	5,543,456	4,033,172

(1) Includes 15,032 shares of series B redeemable convertible preferred stock (including the net exercise immediately prior to the closing of outstanding warrants to purchase 130 shares of series B redeemable convertible preferred stock at the initial public offering price of $15.00 per share), 15,242 shares of series C redeemable convertible preferred stock and 3,334 shares of series D redeemable convertible preferred stock, in each case issued to Prospect Associates II, L.P., and 987,124 shares of series B redeemable convertible preferred stock (including the net exercise immediately prior to the closing of outstanding warrants to purchase 8,562 shares of series B redeemable convertible preferred stock at the initial public offering price of $15.00 per share), 1,000,978 shares of series C redeemable convertible preferred stock and 219,042 shares of series D 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 2,689 shares of series B redeemable convertible preferred stock issued to NEA Ventures 2004, Limited Partnership (including the net exercise immediately prior to the closing of outstanding warrants to purchase 23 shares of series B redeemable convertible preferred stock at the initial public offering price of $15.00 per share), 999,465 shares of series B redeemable convertible preferred stock (including the net exercise immediately prior to the closing of outstanding warrants to purchase 8,669 shares of series B redeemable convertible preferred stock at the initial public offering price of $15.00 per share), 1,016,220 shares of series C redeemable convertible preferred stock and 1,643,472 shares of series D redeemable convertible preferred stock issued to New Enterprise Associates 11, L.P., and 821,736 shares of series D redeemable convertible preferred stock issued to New Enterprise Associates 9, Limited Partnership. Mr. Raab, one of our directors, is a partner of New Enterprise Associates.

(3) Includes 5,092 shares of series B redeemable convertible preferred stock (including the exercise for cash immediately prior to the closing of outstanding warrants to purchase 76 shares of series B redeemable convertible preferred stock), 5,132 shares of series C redeemable convertible preferred stock and 2,904 shares of series D redeemable convertible preferred stock issued to Frazier Affiliates IV, L.P., and 1,003,488 shares of series B redeemable convertible preferred stock (including the exercise for cash immediately prior to the closing of outstanding warrants to purchase 15,042 shares of series B redeemable convertible preferred stock), 1,011,088 shares of series C redeemable convertible preferred stock and 572,310 shares of series D 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.

(4) Includes 906,079 shares of series B redeemable convertible preferred stock (including the net exercise immediately prior to the closing of outstanding warrants to purchase 7,859 shares of series B redeemable convertible preferred stock at the initial public offering price of $15.00 per share), 874,830 shares of series C redeemable convertible preferred stock and 196,058 shares of series D redeemable convertible preferred stock issued to Canaan Equity III, L.P., and 33,835 shares of series B redeemable convertible preferred stock (including the net exercise immediately prior to the closing of outstanding warrants to purchase 293 shares of series B redeemable convertible preferred stock at the initial public offering price of $15.00 per share), 32,668 shares of series C redeemable convertible preferred stock and 7,320 shares of series D 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 51,015 shares of series B redeemable convertible preferred stock (including 753 shares issued pursuant to the exercise for cash immediately prior to the closing of warrants to purchase series B redeemable convertible preferred stock), 33,398 shares of series C redeemable convertible preferred stock and 12,968 shares of series D redeemable convertible preferred stock issued to CHL Medical Partners II Side Fund, L.P., and 757,167 shares of series B redeemable convertible preferred stock (including 11,182 shares issued pursuant to the exercise for cash immediately prior to the closing of warrants to purchase series B redeemable convertible preferred stock), 495,700 shares of series C redeemable convertible preferred stock and 192,466 shares of series D redeemable convertible preferred stock issued to CHL Medical Partners II, L.P.

(6) Includes 793,650 shares of series C redeemable convertible preferred stock and 271,172 shares of series D redeemable convertible preferred stock issued to Quaker BioVentures, L.P. and 264,550 shares of series C redeemable convertible preferred stock and 90,390 shares of series D 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 us 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
Entities affiliated with CHL Medical Partners	$ 5,500,000	784,313

In connection with these bridge financings, we also issued warrants to the investors that were exercisable in the aggregate for 133,332 shares of our common stock at an exercise price of fifty-six cents ($0.56) per share. The investors exercised all of these common stock warrants in August 2005.

Certain Relationships

Registration Rights

Pursuant to a third amended and restated investor rights agreement among holders of our redeemable convertible preferred stock and us, we granted registration rights to all such holders, to Mount Sinai School of Medicine of New York University and to the holder of a warrant to purchase 5,333 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 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.

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 50,000,000 shares of common stock, par value $0.01 per share, and 10,000,000 shares of preferred stock, par value $0.01 per share, all of which preferred stock will be undesignated.

As of April 25, 2007, we had issued and outstanding:

- 1,162,502 shares of common stock outstanding held by 40 stockholders of record;
- 444,443 shares of series A redeemable convertible preferred stock that are convertible into 444,443 shares of common stock;
- 4,877,056 shares of series B redeemable convertible preferred stock that are convertible into 4,877,056 shares of common stock;
- 5,820,020 shares of series C redeemable convertible preferred stock that are convertible into 5,820,020 shares of common stock; and
- 4,930,405 shares of series D redeemable convertible preferred stock that are convertible into 4,930,405 shares of common stock.

As of April 25, 2007, we also had outstanding:

- options to purchase 2,549,950 shares of common stock at a weighted average exercise price of $7.56 per share;
- warrants to purchase an aggregate of 59,674 shares of series B redeemable convertible preferred stock at an exercise price of $6.38 per share, which, upon the closing of this offering, will be automatically exercised for 40,797 shares of Series B redeemable convertible preferred stock which will then be converted into shares of common stock on a one for one basis; and
- a warrant to purchase 5,333 shares of common stock at an exercise price of $5.63 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 16,071,924 shares of our common 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 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 or warrants to purchase 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 5,333 shares of common stock at an exercise price of $5.63.

Options

As of April 25, 2007, options to purchase 2,549,950 shares of common stock at a weighted average exercise price of $7.56 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 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 Global 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 a majority of our outstanding voting stock, provided that provisions concerning certain stockholder actions, proposals and director nominations, our staggered board, the manner in which our by-laws may be amended and certain provisions relating to indemnification may be amended only by the affirmative vote of the holders of at least 67% of our outstanding voting stock.

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 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 16,570,855 shares of our common stock 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 reasonable best efforts to register all or part of their securities for sale under the Securities Act.

Form S-3 Registration Rights

If we are eligible to register any of our common stock on Form S-3, these stockholders may require that we use reasonable best efforts to register all or part of their securities for sale under the Securities Act. This right is subject to specified limitations, including but not limited to (i) if we have 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 American Stock Transfer and Trust Company following the closing of this offering.

The NASDAQ Global Market

Shares of our common stock have been approved for quotation on The NASDAQ Global Market under the symbol "FOLD."

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.

Upon the closing of this offering, we will have outstanding 22,234,426 shares of common stock, after giving effect to the issuance of 5,000,000 shares of common stock in this offering and the automatic conversion of all outstanding shares of our convertible preferred stock, into an aggregate of 16,071,924 shares of our common stock, assuming no exercise of the underwriters' over-allotment option and no exercise of options or other warrants outstanding as of April 25, 2007. As of April 25, 2007, we had outstanding options to purchase 2,549,950 shares of common stock, a warrant to purchase 5,333 shares of common stock and warrants to purchase an aggregate of 59,674 shares of series B redeemable convertible preferred stock. Upon the closing of this offering, these warrants to purchase series B redeemable convertible preferred stock will be automatically exercised and the shares of series B redeemable convertible preferred stock automatically converted to common stock, resulting in the issuance of 40,797 shares of common stock.

Of the shares to be outstanding immediately after the closing of this offering, the 5,000,000 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 17,234,426 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 222,344 shares immediately after this offering, and
- the average weekly trading volume in our common stock on The NASDAQ Global 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, 6,344,018 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 8,749,716 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 Rule 144, but without compliance with the various restrictions, including the holding period, contained in Rule 144. Subject to vesting and to the 180-day lock-up period described below, approximately 1,845,940 shares of our common stock (including shares of common stock that may be issued upon exercise of stock options) 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 and Merrill Lynch, 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 16,570,855 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 April 25, 2007, we had outstanding options to purchase 2,549,950 shares of common stock, of which options to purchase 678,538 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 and our 2007 equity incentive 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 5,333 shares of our common stock at an exercise price of $5.63 per share. Any shares purchased pursuant to this warrant will be freely tradable under Rule 144(k), subject to the 180-day lock-up period described above.

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 and Merrill Lynch & Co. 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:

Underwriter	Number of Shares
Morgan Stanley & Co. Incorporated	1,750,000
Merrill Lynch, Pierce, Fenner & Smith Incorporated	1,750,000
J.P. Morgan Securities Inc.	750,000
Lazard Capital Markets LLC	375,000
Pacific Growth Equities, LLC	375,000
Total	5,000,000

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

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.

Discount and Commissions

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 $0.63 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.

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	$ 1.05	$ 1.05
Total	$ 5,250,000	$ 6,037,500

In addition, we estimate that the expenses of this offering payable by us, other than the underwriting discount and commissions, will be approximately $1.9 million.

Over-allotment Option

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 750,000 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 $86.3 million, and the total proceeds to us would be $78.3 million after deducting the underwriting discount and commissions and estimated offering expenses.

No Sales of Similar Securities

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 and Merrill Lynch & Co. 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.

Price Stabilization and Short Positions

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.

Quotation on The NASDAQ Global Market

Our common stock has been approved for listing on The NASDAQ Global Market under the symbol “FOLD.”

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.

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.

Other Relationships

Certain of the underwriters or their affiliates may provide investment and commercial banking and financial advisory services to us in the ordinary course of business, for which they may receive customary fees and commissions.

Lazard Frères & Co. LLC referred this transaction to Lazard Capital Markets LLC and will receive a referral fee from Lazard Capital Markets LLC in connection therewith.

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, 2006 and 2005, and for each of the three years in the period ended December 31, 2006, and the period from February 4, 2002 (inception) to December 31, 2006 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.

Amicus Therapeutics, Inc.
(a development stage company)

Index to Consolidated Financial Statements

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Report of Independent Registered Public Accounting Firm

Board of Directors
Amicus Therapeutics, Inc.

We have audited the consolidated balance sheets of Amicus Therapeutics, Inc. and subsidiary (a development stage company) as of December 31, 2005 and 2006 and the related consolidated statements of operations, changes in stockholders' deficiency and cash flows for each of the three years in the period ended December 31, 2006 and the period February 4, 2002 (inception) to December 31, 2006. 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 consolidated financial position of Amicus Therapeutics, Inc. and subsidiary as of December 31, 2005 and 2006, and the results of their operations and their cash flows for each of the three years in the period ended December 31, 2006, and the period February 4, 2002 (inception) to December 31, 2006, in conformity with U.S. generally accepted accounting principles.

As discussed in Note 2 to the consolidated financial statements, effective January 1, 2006, the Company adopted Statement of Financial Accounting Standards No. 123(R), "Share-Based Payments" applying the prospective method.

/s/ Ernst & Young LLP

Metro Park, New Jersey
March 16, 2007, except for Note 1
as to which the date is May 24, 2007

Amicus Therapeutics, Inc.
(a development stage company)

Consolidated Balance Sheets

	December 31, 2005	December 31, 2006
Current assets:		
Cash and cash equivalents	$ 6,449,151	$ 12,126,581
Investments in marketable securities	17,969,096	42,572,468
Prepaid expenses and other current assets	441,081	321,275
Total current assets	24,859,328	55,020,324
Property and equipment, less accumulated depreciation and amortization of $604,864 and $1,557,316 at December 31, 2005 and 2006, respectively	3,278,887	4,357,912
Other non-current assets	531,739	267,338
Total Assets	$ 28,669,954	$ 59,645,574
Current liabilities:		
Accounts payable	906,226	1,195,318
Accrued expenses	1,407,025	7,703,775
Current portion of capital lease obligations	279,265	1,307,451
Total current liabilities	2,592,516	10,206,544
Warrant liability	704,187	608,767
Capital lease obligations, less current portion	734,370	2,256,092
Commitments and contingencies		
Series A redeemable convertible preferred stock, $.01 par value, 444,443 shares authorized, issued and outstanding at December 31, 2005 and 2006 (aggregate liquidation preference $2,500,000 at December 31, 2005 and 2006), zero pro forma shares outstanding (unaudited)	2,466,214	2,475,689
Series B redeemable convertible preferred stock, $.01 par value, 4,936,730 shares authorized, 4,862,734 and 4,877,056 shares issued and outstanding at December 31, 2005 and 2006 respectively (aggregate liquidation preference $31,000,000 at December 31, 2005 and 2006)	30,668,842	30,868,501
Series C redeemable convertible preferred stock, $.01 par value, 5,820,020 shares authorized, 2,910,010 and 5,820,020 shares issued and outstanding at December 31, 2005 and 2006 respectively (aggregate liquidation preference $27,499,665 and $55,999,331 at December 31, 2005 and 2006)	27,333,758	54,868,868
Series D redeemable convertible preferred stock, $.01 par value, 4,930,405 shares authorized, 2,953,878 issued and outstanding at December 31, 2006 (aggregate liquidation preference $35,946,897 at December 31, 2006)	—	35,876,547
Stockholders' (deficiency) equity:		
Common stock, $.01 par value, 21,333,333 shares authorized, 538,025 and 990,492 shares issued and outstanding at December 31, 2005 and 2006, respectively	40,352	70,288
Additional paid-in capital	4,015,140	6,066,876
Accumulated other comprehensive (loss)/income	(16,139)	14,752
Deferred compensation	(2,546,846)	—
Deficit accumulated during the development stage	(37,322,440)	(83,667,350)
Total stockholders' (deficiency) equity	(35,829,933)	(77,515,434)
	$ 28,669,954	$ 59,645,574

See accompanying notes to consolidated financial statements

Amicus Therapeutics, Inc.
(a development stage company)

Consolidated Statements of Operations

	Years Ended December 31,			Period from February 4, 2002 (Inception) to December 31, 2006
	2004	2005	2006	
Operating Expenses:				
Research and development	$ 6,300,885	$ 13,651,640	$ 33,630,262	$ 58,803,948
General and administrative	2,081,203	6,876,883	12,276,559	22,791,915
Impairment of leasehold improvements	—	—	—	1,029,696
Depreciation and amortization	145,961	302,832	952,452	1,557,316
In-process research and development	—	—	—	418,080
Total operating expenses	8,528,049	20,831,355	46,859,273	84,600,955
Loss from operations	(8,528,049)	(20,831,355)	(46,859,273)	(84,600,955)
Other income (expenses):				
Interest income	189,847	609,519	1,990,722	2,807,580
Interest expense	(550,004)	(81,776)	(272,890)	(1,082,933)
Change in fair value of warrant liability	(1,911)	(280,474)	(21,963)	(304,348)
Other expense	—	—	(1,181,506)	(1,181,506)
Loss before income tax benefit	(8,890,117)	(20,584,086)	(46,344,910)	(84,362,162)
Income tax benefit	83,015	611,797	—	694,812
Net loss	(8,807,102)	(19,972,289)	(46,344,910)	(83,667,350)
Deemed dividend	—	—	(19,424,367)	(19,424,367)
Preferred stock accretion	(125,733)	(138,743)	(158,802)	(450,890)
Net loss attributable to common stockholders	$ (8,932,835)	$ (20,111,032)	$ (65,928,079)	$ (103,542,607)
Net loss attributable to common stockholders per common share — basic and diluted	$ (29.05)	$ (49.02)	$ (89.58)	
Weighted-average common shares outstanding — basic and diluted	307,539	410,220	735,967	
Unaudited pro forma net loss			$ (46,344,910)	
Unaudited basic and diluted pro forma net loss per share			$ (2.76)	
Unaudited basic and diluted pro forma weighted-average shares outstanding			16,807,933	

See accompanying notes to consolidated financial statements

Amicus Therapeutics, Inc.
(a development stage company)

Consolidated Statements of Changes in Stockholders' Deficiency
Period from February 4, 2002 (inception) to December 31, 2002,
and the four year period ended December 31, 2006

	Common Stock		Additional Paid-In Capital	Other Comprehensive Gain/ (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	74,938	5,620	78,243	—	—	—	83,863
Stock issued for in-process research and development	232,266	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 arrangements	—	—	8,000	—	—	—	8,000
Accretion of redeemable convertible preferred stock	—	—	(10,720)	—	—	—	(10,720)
Net loss	—	—	—	—	—	(1,775,353)	(1,775,353)
Balance at December 31, 2002	307,204	23,040	685,049	—	(181,518)	(1,775,353)	(1,248,782)
Stock issued from exercise of stock options	333	25	—	—	—	—	25
Deferred compensation	—	—	14,138	—	(14,138)	—	—
Amortization of deferred compensation	—	—	—	—	70,340	—	70,340
Issuance of stock warrants with convertible notes	—	—	210,000	—	—	—	210,000
Issuance of stock options to consultants	—	—	4,434	—	—	—	4,434
Accretion of 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	307,537	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 redeemable convertible preferred stock	—	—	(125,732)	—	—	—	(125,732)
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	—	—	—	—	—	(8,807,102)	(8,807,102)
Net total comprehensive loss	—	—	—	—	—	—	(8,816,185)
Balance at December 31, 2004	307,537	23,065	1,182,548	(9,083)	(133,174)	(17,350,151)	(16,286,795)
Stock issued from exercise of stock options	97,156	7,287	16,641	—	—	—	23,928
Stock issued from exercise of warrants	133,332	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 redeemable convertible preferred stock	—	—	(138,743)	—	—	—	(138,743)
Comprehensive Loss:							
Unrealized holding loss on available-for-sale securities	—	—	—	(7,056)	—	—	(7,056)
Net loss	—	—	—	—	—	(19,972,289)	(19,972,289)
Net total comprehensive loss	—	—	—	—	—	—	(19,979,345)
Balance at December 31, 2005	538,025	40,352	4,015,140	(16,139)	(2,546,846)	(37,322,440)	(35,829,933)
Stock issued from exercise of options	265,801	19,936	138,345	—	—	—	158,281
Stock issued for license payment	133,333	10,000	1,210,000	—	—	—	1,220,000
Reversal of deferred compensation upon adoption of FAS 123(R)	—	—	(2,546,846)	—	2,546,846	—	—
Stock-based compensation	53,333	—	2,816,210	—	—	—	2,816,210
Issuance of stock options to consultants	—	—	475,446	—	—	—	475,446
Accretion of redeemable convertible preferred stock	—	—	(158,802)	—	—	—	(158,802)
Reclassification of Warrant liability upon exercise of Series B redeemable convertible preferred stock warrants	—	—	117,383	—	—	—	117,383
Beneficial conversion on issuance of Series C redeemable convertible preferred stock	—	—	19,424,367	—	—	—	19,424,367
Beneficial conversion charge (deemed dividend) on issuance of Series C redeemable convertible preferred stock	—	—	(19,424,367)	—	—	—	(19,424,367)
Comprehensive (Loss)/ Income:							
Unrealized holding gain on available-for-sale securities	—	—	—	30,891	—	—	30,891
Net loss	—	—	—	—	—	(46,344,910)	(46,344,910)
Net total comprehensive loss	—	—	—	—	—	—	(46,314,019)
Balance at December 31, 2006	990,492	$ 70,288	$ 6,066,876	$ 14,752	$ —	$ (83,667,350)	$ (77,515,434)

Amicus Therapeutics, Inc
(a development stage company)

Consolidated Statements of Cash Flows

	Years Ended December 31,			Period from February 4, 2002 (Inception) to December 31, 2006
	2004	2005	2006	
Operating activities				
Net loss	$ (8,807,102)	$ (19,972,289)	$ (46,344,910)	$ (83,667,350)
Adjustments to reconcile net loss to net cash used in operating activities:				
Non-cash interest expense	435,934	—	—	525,267
Depreciation and amortization	143,293	302,832	952,452	1,554,648
Amortization of non-cash compensation	59,842	364,551	—	522,081
Stock-based compensation	—	—	2,816,210	2,816,210
Stock-based license payments	—	—	1,220,000	1,220,000
Non-cash charge for stock based compensation issued to consultants	16,118	111,471	475,446	691,332
Change in fair value of warrant liability	1,911	280,474	21,963	304,348
Impairment of leasehold improvements	—	—	—	1,029,696
Non-cash charge for in process research and development	—	—	—	418,080
Beneficial conversion feature related to bridge financing	94,500	—	—	135,000
Changes in operating assets and liabilities:				
Prepaid expenses and other current assets	(147,664)	(285,698)	119,806	(321,275)
Other non-current assets	(19,936)	(491,202)	264,401	(288,505)
Accounts payable and accrued expenses	(1,008,299)	1,565,512	6,585,842	8,899,093
Net cash used in operating activities	(9,231,403)	(18,124,349)	(33,888,790)	(66,161,375)
Investing activities				
Sale and redemption of marketable securities	2,162,275	3,092,620	37,441,039	42,695,934
Purchases of marketable securities	(6,362,527)	(16,989,847)	(62,013,520)	(85,370,850)
Purchases of property and equipment	(227,317)	(3,040,442)	(2,031,477)	(6,942,256)
Net cash used in investing activities	(4,427,569)	(16,937,669)	(26,603,958)	(49,617,172)
Financing activities				
Proceeds from the issuance of preferred stock, net of issuance costs	12,877,598	40,316,115	63,370,682	118,969,210
Proceeds from the issuance of convertible notes	1,200,000	—	—	5,000,000
Payments of capital lease obligations	(171,914)	(272,697)	(880,747)	(1,477,661)
Payments from exercise of stock options	—	23,928	158,281	182,234
Proceeds from exercise of warrants (common and preferred)	—	75,000	91,307	166,307
Proceeds from capital asset financing arrangement	—	1,111,787	3,430,655	5,065,038
Net cash provided by financing activities	13,905,684	41,254,133	66,170,178	127,905,128
Net increase in cash and cash equivalents	246,712	6,192,115	5,677,430	12,126,581
Cash and cash equivalents at beginning of year/ period	10,324	257,036	6,449,151	—
Cash and cash equivalents at end of year/period	$ 257,036	$ 6,449,151	$ 12,126,581	$ 12,126,581
Supplemental disclosures of cash flow information				
Cash paid during the period for interest	$ 19,570	$ 481,577	$ 272,890	$ 788,014
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	$ 125,732	$ 138,743	$ 158,802	$ 450,890
Beneficial conversion feature related to issuance of the second tranche of Series C redeemable convertible preferred stock	$ —	$ —	$ 19,424,367	$ 19,424,367

See accompanying notes to consolidated financial statements

Amicus Therapeutics, Inc.
(a development stage company)

Notes To Consolidated 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 approximately $83.7 million at December 31, 2006 and anticipates incurring losses through the year 2007 and beyond. The Company has not yet generated revenues and has been able to fund its operating losses to date through 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 March 2007, the Company received cash amounting to approximately $24.1 million from the issuance of its second tranche series D redeemable convertible preferred stock. Management believes that the Company's current cash position and the additional funds received in March 2007 are sufficient to cover its cash flow requirements for 2007.

Reverse Stock Split

As a result of the 1:7.5 reverse stock split that became effective on May 24, 2007, every 7.5 shares of the Company's redeemable convertible preferred stock and common stock were combined into one share of the Company's redeemable convertible preferred stock and one share of common stock, respectively. All references to redeemable convertible preferred stock, redeemable convertible preferred stock outstanding, common stock, common shares outstanding, average number of common shares outstanding and per share amounts in these consolidated financial statements and notes to consolidated financial statements prior to the effective date of the reverse stock split have been restated to reflect the 1:7.5 reverse stock split on a retroactive basis.

2. Summary of Significant Accounting Policies

Unaudited Pro Forma Information

Pro forma net loss per share is computed using the weighted-average number of common shares outstanding and gives effect to the Company's issuance in March 2007, of 1,976,527 shares of series D redeemable convertible preferred stock and the automatic conversion of all outstanding shares of the Company's series A, B, C, and D redeemable convertible preferred stock into an aggregate of 16,071,924 shares of common stock upon completion of the Company's initial public offering, as if they had occurred at the beginning of the period. The pro forma information excludes shares of common stock issuable in connection with the exercise of outstanding warrants to purchase shares of series B redeemable convertible preferred stock. Upon the closing of this offering, these warrants will be automatically exercised for 40,797 shares of series B redeemable convertible preferred stock, which will then be automatically converted into shares of common stock on a one for one basis.

Basis of Presentation

The accompanying consolidated financial statements have been prepared in accordance with U.S. generally accepted accounting principles and include all adjustments necessary for the fair presentation of the Company's financial position for the periods presented.

Amicus Therapeutics, Inc.
(a development stage company)

Notes To Consolidated Financial Statements — (Continued)

Consolidation

The financial statements include the accounts of Amicus Therapeutics, Inc. and its wholly owned subsidiary. All significant intercompany transactions and balances are eliminated in consolidation.

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 and 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* ("SFAS No. 115"), these investments are classified as available-for-sale and are reported at fair value on the Company's balance sheet. Unrealized holding gains and losses are reported within accumulated other comprehensive income/(loss) as a separate component of stockholders' 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.

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* ("SFAS No. 107"), 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 values of the Company's redeemable convertible preferred stock at December 31, 2006 is approximately $171.3 million, based on the September 2006 series D redeemable convertible preferred stock price of $12.15 per share. The redeemable convertible preferred stock will be converted into common stock of the Company upon consummation of a qualified initial public offering. The

warrants to purchase shares of series B redeemable convertible preferred stock are recorded at fair value based on the Black-Scholes-Merton methodology and were valued at $0.6 million at December 31, 2006.

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 five 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.

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 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, 2005 and 2006.

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.

Interest Income and Interest Expense

Interest income consists of interest earned on the Company's cash and cash equivalents and marketable securities. Interest expense consists of interest incurred on the Company's capital lease facility.

Other Income and Expenses

During the second and third quarter of 2006 the Company deferred and capitalized $1.2 million of costs directly attributable to the planned offering of its securities as other non-current assets. These costs were recorded as other expenses when the planned offering was withdrawn during the third quarter of 2006.

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.

Other Comprehensive Income/(Loss)

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

Leases

In the ordinary course of business, the Company enters into lease agreements for office space as well as leases for certain property and equipment. The leases have varying terms and expirations and have provisions to extend or renew the lease agreement, among other terms and conditions, as negotiated. Once the agreement is executed, the lease is assessed to determine whether the lease qualifies as a capital or operating lease.

When a non-cancelable operating lease includes any fixed escalation clauses and lease incentives for rent holidays or build-out contributions, rent expense is recognized on a straight-line basis over the initial term of the lease. The excess between the average rental amount charged to expense and amounts payable under the lease is recorded in accrued expenses.

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.

Warrants to Purchase Redeemable Convertible Preferred Stock

The Company accounts for its warrants to purchase shares of its series B redeemable convertible preferred stock ("Series B Warrants") in accordance with FASB Staff Position 150-5: *Issuer's Accounting under FASB Statement No. 150 for Freestanding Warrants and Other Similar Instruments on Shares That Are Redeemable ("FSP150-5").* As the Series B Preferred shares underling the warrants have redemption rights, the warrants to purchase Series B shares are classified as a liability. The Company measures the fair value of its warrant liability using the Black-Scholes option pricing model with changes in fair value recognized as non-operating income or expense. The value of the warrant liability at issuance was $421,802.

Stock-Based Compensation

At December 31, 2005 and 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 this plan 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, 2004 and 2005 to the extent the options granted under the plan had an exercise price that was less than the "deemed" fair market value of the underlying common stock on the date of grant.

Amicus Therapeutics, Inc.
(a development stage company)

Notes To Consolidated Financial Statements — (Continued)

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 the prospective transition method, compensation expense is recognized in the financial statements on a prospective basis for all share-based payments granted subsequent to January 1, 2006, based upon the grant-date fair value estimated in accordance with the provisions of SFAS 123(R). For options granted prior to January 1, 2006, as a non-public company and accounted for using the intrinsic value method, the Company will continue to expense any intrinsic value recognized over the vesting period. The grant-date fair value of awards expected to vest is expensed on a straight-line basis over the vesting period of the related awards. Under the prospective transition method, results for prior periods are not restated and pro forma disclosures for outstanding awards accounted for under the intrinsic value method of APB No. 25 are not presented since the Company used the minimum value method for pro forma disclosure purposes prior to January 1, 2006.

As a result of the adoption of SFAS 123(R), both loss from operations and net loss for the year ended December 31, 2006 include incremental stock-based compensation expense of $2.2 million. For the year ended December 31, 2006, the impact of this incremental stock-based compensation expense on basic and diluted loss per share was $2.99. Results of operations for the year ended December 31, 2006 include $3.3 million of total stock-based compensation expense, including $2.2 million resulting from the adoption of SFAS 123(R), $0.5 million of expense on options granted to non employees, and $0.6 million amortization of the intrinsic value of options granted prior to the adoption of SFAS 123(R). Research and development expense and general and administrative expense include $1.7 million and $1.6 million of stock compensation expense, respectively. Stock-based compensation expense had not impact on the Company's cash flows from operations and financing activities.

SFAS 123(R) does not change the accounting guidance for how the Company accounts for options issued to non-employees. The Company accounts for options issued to non-employees in accordance with SFAS 123 and EITF Issue No 96-18, *Accounting for Equity Instruments That Are Issued to Other Than Employees for Acquiring, or in Conjunction with Selling, Goods or Services* ("EITF 96-18"). As such, the value of such options is periodically re-measured and income or expense is recognized during the vesting terms.

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.

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 volatility for future option grants. The average expected life was determined according to the Security and Exchange Commission ("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:

	Year Ended December 31, 2006
Expected stock price volatility	74.8%
Risk free interest rate	4.7%
Expected life of options (years)	6.25
Expected annual dividend per share	$ 0.00

Beneficial Conversion Charges

When the Company issues debt or equity securities which are convertible into common stock at a discount from the common stock fair value at the date the debt or equity financing is committed, a beneficial conversion charge is measured as the difference between the closing price and the conversion price at the commitment date. The beneficial conversion charge is presented as a discount or reduction to the related security, with an offsetting amount increasing additional paid-in capital. The Company recorded a beneficial conversion charge for its fiscal year 2003 bridge loan financing of $135,000 which was initially recorded as debt discount and amortized to interest expense through May 2004. The Company also recorded a beneficial conversion charge (also referred to as a deemed dividend) during April of 2006 of approximately $19.4 million related to the issuance of certain shares of series C redeemable convertible preferred stock. The beneficial conversion charge for equity instruments is recorded with offsetting charges and credits to additional paid in capital with no effect on total shareholder equity. The Series C investors committed to finance the second tranche of the Series C redeemable convertible preferred stock on March 31, 2006. The estimated fair value of the common stock was approximately $16.13 per share at the commitment date of the second tranche and the beneficial conversion charge was recognized upon issuance of the Series C redeemable convertible preferred stock as such stock could be converted upon issuance. The Company did not record a beneficial conversion charge for any other redeemable convertible preferred stock issuances as the common stock fair value was less than the conversion price of each offering on the respective commitment dates of those offerings.

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

The Company calculates net loss per share in accordance with SFAS No. 128, *Earnings Per Share*. The Company has determined that its series A, B, C, and D redeemable convertible preferred stock 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.

Amicus Therapeutics, Inc.
(a development stage company)

Notes To Consolidated Financial Statements — (Continued)

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,		
	2004	2005	2006
Historical			
Numerator:			
Net loss	$ (8,807,102)	$ (19,972,289)	$ (46,344,910)
Deemed dividend	—	—	(19,424,367)
Accretion of redeemable convertible preferred stock	(125,733)	(138,743)	(158,802)
Net loss attributable to common stockholders	$ (8,932,835)	$ (20,111,032)	$ (65,928,079)
Denominator:			
Weighted average common shares outstanding — basic and diluted	307,539	410,220	735,967

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 3,833,306, 9,459,737 and 16,530,450 for the years ended December 31, 2004, 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 July 2006, FASB issued FSAB Interpretation No. 48, *Accounting for Uncertainty in Income Taxes — an Interpretation of FASB Statement No. 109 ("FIN No. 48")*, which clarifies the accounting for uncertainty in tax positions. This Interpretation prescribes a recognition threshold and measurement attribute for the financial statement recognition and measurement of a tax position taken or expected to be taken in a tax return. It also provides guidance on derecognition, clarification, interest and penalties, accounting in interim periods, disclosures and transitions. The provision of FIN 48 are effective as of the beginning of the Company's 2007 fiscal year, with the cumulative effect, if any, of the change in accounting principle recorded as an adjustment to opening retained earnings. The Company is currently evaluating the impact of adopting FIN 48 on its financial statements. The Company does not expect that the adoption will have a material effect on the results of operations or financial condition.

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

Amicus Therapeutics, Inc.
(a development stage company)

Notes To Consolidated Financial Statements — (Continued)

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
December 31, 2005				
Corporate Debt Securities	$ 17,985,235	$ —	$ (16,139)	$ 17,969,096
December 31, 2006				
Corporate Debt Securities	$ 42,557,716	$ 16,016	$ (1,264)	$ 42,572,468

All of the Company's available for sale investments as of December 31, 2005 and 2006 are due in one year or less.

Unrealized gains and losses are reported as a component of accumulated other comprehensive gain/loss in stockholders' deficiency. For the years ended December 31, 2004 and 2005, unrealized holding losses included in accumulated other comprehensive income/(loss) were $9,083 and $7,056. For the year ended December 31, 2006, unrealized holding gain included in accumulated other comprehensive income/(loss) was $30,891.

For the years ended December 31, 2004 and 2005, realized losses were $704 and $1,228. For the year ended December 31, 2006, there were no realized gains or losses. The cost of securities sold is based on specific identification method.

Unrealized loss positions in the available for sale securities as of December 31, 2005 and 2006 reflect temporary impairments that have not been recognized and have been in a loss position for less than twelve months. The fair value of these available for sale securities in unrealized loss positions was $17,969,096 and $4,819,983 as of December 31, 2005 and 2006, respectively.

Unrealized gains and 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. There are no changes in credit risk of the debt securities. 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.

Amicus Therapeutics, Inc.
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Notes To Consolidated Financial Statements — (Continued)

4. Property and Equipment

Property and equipment consist of the following:

	December 31, 2005	December 31, 2006
Property and equipment consist of the following:		
Computer equipment	$ 284,913	$ 563,729
Computer software	15,921	104,914
Research equipment	1,790,873	2,684,613
Furniture and fixtures	251,703	525,504
Leasehold improvements	109,345	2,036,468
Construction in progress	1,430,996	—
	3,883,751	5,915,228
Less accumulated depreciation and amortization	(604,864)	(1,557,316)
	$3,278,887	$ 4,357,912

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, the Company 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 are costs capitalized pursuant to capital lease obligations of $1,146,007 and $4,844,223 at December 31, 2005 and 2006. Depreciation and amortization expense relating to the capital lease obligations was $0, $137,504, $789,235,and $926,739 for the years ended December 31, 2004, 2005, and 2006, and for the Period February 4, 2002 (inception) to December 31, 2006, respectively.

5. Accrued Expenses

Accrued expenses consist of the following:

	December 31, 2005	December 31, 2006
Accrued construction costs	$ 592,594	$ —
Accrued professional fees	312,244	253,161
Accrued contract manufacturing & contract research costs	53,163	5,681,741
Accrued compensation and benefits	14,719	1,235,595
Accrued facility costs	182,303	482,482
Accrued other	252,002	50,796
	$ 1,407,025	$ 7,703,775

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Notes To Consolidated Financial Statements — (Continued)

6. Capital Structure

Redeemable Convertible Preferred Stock

At December 31, 2006 the Company is authorized to issue 444,443 shares of series A redeemable convertible preferred stock ("Series A"), 4,936,740 shares of series B redeemable convertible preferred stock ("Series B"), 5,820,020 shares of series C redeemable convertible preferred stock ("Series C") and 4,930,406 shares of series D redeemable convertible preferred stock ("Series D").

In September 2006, the Company commenced the sale of 4,930,405 shares of its Series D redeemable convertible preferred stock at $1.62 per share. The Company issued an aggregate of 2,953,878 shares in September 2007, resulting in gross proceeds to the Company of $35.9 million. The remaining shares of Series D redeemable convertible preferred stock were committed to be issued at the earlier of the date on which a majority of the members of the Board of Directors chose to close the second tranche or March of 2007. During March 2007, the Company issued the second tranche of 1,976,527 shares of Series D redeemable convertible preferred stock at $12.15 per share for gross proceeds to the Company of $24.1 million. The Company does not have any other commitment to issue preferred stock.

Voting

Series A, Series B, Series C, and Series D 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, Series C, and Series D 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, Series C and Series D 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, Series C, and Series D 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 $12.15 and the common stock is listed on a U.S. national securities exchange or admitted for quotation on the NASDAQ Global 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, Series C and Series D holders are entitled to receive, in preference to common stock, an amount equal to $5.63 per share, $6.38 per share, $9.45 per share, and $12.15 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, Series C and Series D stockholders shall participate with the holders of common stock on an as-converted basis.

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Notes To Consolidated Financial Statements — (Continued)

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 fourth anniversary of the Series D original issue date (September 13, 2006). The redeemable convertible preferred stock may be redeemed at an amount equal to the liquidation preference upon receipt by the Company of a request from the holders of at least a majority of the then outstanding shares of Series A, Series B, Series C, and Series D that the redeemable convertible preferred stock be redeemed.

As of December 31, 2005 and 2006, Series A, Series B, Series C, and Series D are recorded at its stated values (estimated fair value of $5.63 per share, $6.38 per share, $9.45 per share, and $12.15 per share, respectively, less issuance costs and accretion adjustments).

	Series A		Series B		Series C		Series D	
	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount
Balance at February 4, 2002 (inception)	—	$ —	—	$ —	—	$ —	—	$ —
Issuance of Series A at $5.63 per share	444,443	2,500,000	—	—	—	—	—	—
Issuance costs	—	(95,185)	—	—	—	—	—	—
Accretion	—	10,720	—	—	—	—	—	—
Balance at December 31, 2002	444,443	2,415,535	—	—	—	—	—	—
Accretion	—	16,893	—	—	—	—	—	—
Balance at December 31, 2003	444,443	2,432,428	—	—	—	—	—	—
Issuance of Series B at $6.38 per share	—	—	2,823,523	18,000,000	—	—	—	—
Issuance cost	—	—	—	(122,402)	—	—	—	—
Issuance of warrants with Series B	—	—	—	(421,802)	—	—	—	—
Accretion	—	16,893	—	108,840	—	—	—	—
Balance at December 31, 2004	444,443	2,449,321	2,823,523	17,564,636	—	—	—	—
Issuance of Series B at $6.38 per share	—	—	2,039,211	13,000,000	—	—	—	—
Issuance cost	—	—	—	(5,793)	—	—	—	—
Issuance of Series C at $9.45 per share	—	—	—	—	2,910,010	27,499,665	—	—
Issuance cost	—	—	—	—	—	(177,757)	—	—
Accretion	—	16,893	—	109,999	—	11,850	—	—
Balance at December 31, 2005	444,443	2,466,214	4,862,734	30,668,842	2,910,010	27,333,758	—	—
Exercise of warrants with Series B at $6.38	—	—	14,322	91,307	—	—	—	—
Issuance of Series C at $9.45 per share	—	—	—	—	2,910,010	27,499,667	—	—
Issuance of Series D at $12.15 per share	—	—	—	—	—	—	2,953,878	35,946,897
Issuance cost	—	—	—	—	—	—	—	(75,882)
Accretion to redemption value	—	9,475	—	108,352	—	35,443	—	5,532
Balance at December 31, 2006	444,443	$ 2,475,689	4,877,056	$ 30,868,501	5,820,020	$ 54,868,868	2,953,878	$ 35,876,547

Amicus Therapeutics, Inc.
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Notes To Consolidated Financial Statements — (Continued)

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 784,312 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.

In addition, the Company issued warrants for 133,332 shares of common stock in connection with some of the Bridge Loans (see warrants below).

Common Stock

As of December 31, 2006 the Company was authorized to issue 21,333,333 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.

In connection with the formation of the Company, the Company issued 232,266 shares of common stock to the Mount Sinai School of Medicine of New York University (MSSM) 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 October of 2006, the Company amended its license agreement MSSM to expand its exclusive worldwide patent rights to develop and commercialize pharmacological chaperones. In connection with the amendment, the Company paid $1.0 million and issued 133,333 shares of its common stock valued at $1,220,000 to MSSM.

In connection with an employment agreement and director compensation agreement, the Company issued 53,333 shares of common stock in return services. The shares will vest over three and four year periods. The Company recorded $41,000 as compensation expense during 2006 in connection with the issuance of these restricted shares and $0 in 2005 and 2004.

Warrants

During 2002, the Company issued 5,333 common stock warrants to a vendor as part of a capital lease agreement. These warrants were outstanding at December 31, 2005 and 2006. The warrants have an exercise price of $5.63 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.

In 2003, the Company issued 133,332 common stock warrants to certain investors in connection with its Bridge Loans. The warrants had an exercise price of $0.56 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 $126,000 for the year ended December 31, 2004.

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Notes To Consolidated Financial Statements — (Continued)

In 2004, the Company issued warrants to purchase 73,996 Series B shares to certain investors as part of the Series B financing. During 2006 there were 14,322 warrants exercised for Series B shares. As of December 31, 2006 there were 59,674 warrants still outstanding. The warrants have an exercise price of $6.38 per share (adjusted for stock splits, stock dividends, etc.) and can be exercised for cash or net shares at the option of the warrant holders. The warrants to purchase Series B Preferred shares will be automatically exercised upon a qualifying initial public offering. As the Series B Preferred shares underling the warrants have redemption rights, the warrants to purchase Series B shares are classified as a liability in accordance with FSP 150-5.

The Company measures the fair value of its warrant liability using the Black-Scholes option pricing model with changes in fair value recognized in earnings. The value of the warrant liability at issuance was $421,802. The Company recognized changes in the fair value of the warrant liability as non-operating income or (expense) of $(1,911), $(280,474), and $(21,963) in 2004, 2005, and 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 restricted stock and 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 25% on the first year anniversary date of grant plus an additional 1/48th for each month thereafter and may be exercised in whole or in part for 100% of the shares subject to vesting at any time after the date of grant.

As of December 31, 2006, the Company reserved up to 2,733,333 shares for issuance under the 2002 Plan.

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Notes To Consolidated Financial Statements — (Continued)

The following table summarizes information about stock options outstanding:

	Number of Shares (in thousands)	Weighted Average Exercise Price	Weighted Average Remaining Contractual Life	Aggregate Intrinsic Value (in millions)
Options outstanding, December 31, 2003	149.7	$ 0.15		
Granted	277.8	$ 0.60		
Forfeited	(0.9)	$ 0.60		
Options outstanding, December 31, 2004	426.6	$ 0.45		
Granted	1,010.2	$ 2.17		
Exercised	(97.2)	$ 0.22		
Forfeited	(102.5)	$ 0.45		
Options outstanding, December 31, 2005	1,237.1	$ 2.10		
Granted	1,005.1	$ 6.00		
Exercised	(265.8)	$ 0.60		
Forfeited	(108.0)	$ 2.20		
Options outstanding, December 31, 2006	1,868.4	$ 4.27	8.4 years	$ 78.7
Vested and unvested expected to vest, December 31, 2006	1,672.3	$ 4.12	8.3 years	$ 72.0
Exercisable at December 31, 2006	416.5	$ 2.17	7.4 years	$ 24.0

The weighted-average grant-date fair value per share of options granted during 2004, 2005 and 2006 were $5.40, $13.80 and $10.20, respectively. As of December 31, 2006, the total unrecognized compensation cost related to non-vested stock options granted was $8.1 million and is expected to be recognized over a weighted average period of 2.7 years.

The aggregate intrinsic value of options exercised during the years ended December 31, 2004, 2005, and 2006, was $0, $140,235, and $2,464,768. Cash proceeds from stock options exercised during the years ended December 31, 2004, 2005 and 2006 totaled $0, $23,928 and $158,281, respectively.

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

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Notes To Consolidated Financial Statements — (Continued)

The following table sets the Company's restricted stock activity as of and for the year ended December 31, 2006:

	Restricted Stock	
	Number of Shares (in thousands)	Weighted Average Grant Date Fair Value
Unvested at December 31, 2005	—	$ —
Granted	53.3	$ 8.92
Vested	(2.2)	$ 8.17
Forfeited	—	$ —
Unvested at December 31, 2006	51.1	$ 8.92

The weighted average grant-date fair value of restricted stock awards granted during the year ended December 31, 2006 was $8.92. There were no restricted stock grants prior to 2006. As of December 31, 2006, the total unrecognized compensation cost related to unvested restricted stock awards was $433,958. This cost is expected to be recognized over a weighted average period of 3.5 years. The total fair value of restricted stock awards which vested during 2006 was $18,166.

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 December 31, 2006 the Company has not made any match of 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. On August 14, 2006, the Company entered into another sublease agreement to expand office space in an adjacent building. This sublease term will expire on August 31, 2009 or on such earlier date upon mutual agreement of both parties. At December 31, 2006, aggregate annual future minimum lease payments under these leases are as follows:

Operating Leases	
Years ending December 31:	
2007	$ 1,629,181
2008	1,654,965
2009	1,527,021
2010	1,295,338
2011	1,306,790
2012 and thereafter	218,525
	$ 7,631,820

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Notes To Consolidated Financial Statements — (Continued)

Rent expense for the years ended December 31, 2004, 2005, and 2006 were $152,668, $971,688, and $1,572,843, respectively.

Capital Lease Facility

In August 2002, the Company entered into financing 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 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 leasehold improvements.

At December 31, 2005 and 2006, the total amount available to the Company under these agreements is $4.0 million and $1.4 million, respectively.

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

Capital Leases	
Years ending December 31:	
2007	$ 1,624,727
2008	1,558,565
2009	770,851
2010	159,282
2011	—
	4,113,425
Less payments for interest	(549,882)
Total principal obligation	3,563,543
Less short-term portion	(1,307,451)
Long-term portion	$ 2,256,092

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

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Notes To Consolidated 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:

	For Years Ended December 31,		
	2004	2005	2006
Current deferred tax asset			
Non — cash stock issue to consultants	$ —	$ 63,747	$ 246,307
Others	—	32,983	1,309,070
		96,730	1,555,377
Non — current deferred tax assets Amortization/Depreciation	198,941	132,097	1,288,355
Research tax credit	730,903	1,344,230	3,610,574
Net operating loss carry forwards	6,387,827	14,463,790	27,257,344
Others	75,165	28,829	121,398
Total deferred tax asset	7,392,836	16,065,676	34,833,048
Non — current deferred tax liability			
Depreciation	(29,865)	(57,027)	—
Total net deferred tax asset	7,362,971	16,008,649	34,833,048
Less valuation allowance	(7,362,971)	(16,008,649)	(34,833,048)
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, 2004, 2005, and 2006, the Company recorded valuation allowances of $7.4 million, $16.0 million and $33.8 million, respectively, representing a change in the valuation allowance of $8.6 million and $17.8 million for the two previous fiscal year-ends, due to the uncertainty regarding the realization of such deferred tax assets, to offset the benefits of net operating losses generated during those years.

As of December 31, 2006, the Company had federal and state net operating loss carryforwards of approximately $69.0 million and $64.0 million respectively. The federal carryforward will begin to expire in 2023 and will end in 2027. The state carryforward will begin to expire in 2011 and will end in 2014. 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 $0.1 million and $0.6 million in connection with the sale of net operating losses in the New Jersey Tax Transfer Program during the years ended December 31, 2004 and 2005, respectively.

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Notes To Consolidated Financial Statements — (Continued)

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

	Years Ended December 31,		
	2004	2005	2006
Statutory rate	(34)%	(34)%	(34)%
State taxes, net of federal benefit	(6)	(6)	(6)
Permanent adjustments	—	1	1
Non deductible interest	1	—	—
R&D credit	(5)	(3)	(4)
Other	(2)	(1)	2
Benefit from sale of net operating loss	(1)	(3)	—
Valuation allowance	44	43	41
Net	(1)%	(3)%	0%

Income tax benefit consisted of the following components:

	Years Ended December 31,		
	2004	2005	2006
Current 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 of New York University (MSSM) — The Company acquired exclusive worldwide patent rights to develop and commercialize Amigal, Plicera and AT2220 and other pharmacological chaperones for the treatment of diseases which can be achieved by enhancing lysosomal enzyme activity pursuant to a license agreement with MSSM. In connection with this agreement, the Company issued 232,266 shares of common stock to MSSM in April 2002. In 2006, the Company amended its license agreement with MSSM to expand its exclusive worldwide patent rights to develop and commercialize pharmacological chaperones. In connection with the amendment, the Company paid $1.0 million and issued 133,333 shares of its common stock with an estimated fair value of $1.2 million to MSSM. In total, the Company recorded $2.2 million of research and development expense in connection with the amendment in 2006. 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, subject to any patent term extension that may be granted.

University of Maryland, Baltimore County — The Company acquired exclusive U.S. patent rights to develop and commercialize Plicera 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 $29,500, which were expensed as research and development expense. The Company is required to make a milestone payment upon the demonstration of safety and efficacy of Plicera for the treatment of Gaucher disease in a Phase II study, and another payment upon receiving FDA approval for Plicera for the treatment of Gaucher disease. Upon satisfaction of both milestones, the Company could be required to make up to $175,000 in aggregate payments. The Company is 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 — The Company acquired exclusive patent rights to develop and commercialize Plicera 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. The Company is also required to make milestone payments based on clinical progress of Plicera, with a payment due after initiation of a Phase III clinical trial for Plicera for the treatment of Gaucher disease, and a payment due upon each filing for regulatory approval of Plicera for the treatment of Gaucher disease in any of the US, Europe or Japan. An additional payment is due upon approval of Plicera for the treatment of Gaucher disease in the U.S. and a payment is also due upon each approval of Plicera for the treatment of Gaucher disease in either Europe or Japan. Assuming successful development of Plicera for the treatment of Gaucher disease in the U.S., Europe and Japan, total milestone payments would be $7,750,000. 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 Plicera.

The Company's rights with respect to these agreements to develop and commercialize Amigal, Plicera 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 232,266 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 $418,080.

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Notes To Consolidated Financial Statements — (Continued)

13. Selected Quarterly Financial Data (Unaudited)

	Quarters Ended			
	March 31	**June 30**	**September 30**	**December 31**
2005				
Net loss	$ (3,391,294)	$ (5,345,461)	$ (5,425,901)	$ (5,809,634)
Net loss attributable to common stockholders	(3,423,017)	(5,377,184)	(5,463,549)	(5,847,282)
Basic and diluted net loss per common share(1)	(11.10)	(15.98)	(12.00)	(10.88)
2006				
Net loss	(8,287,253)	(8,623,668)	(11,642,604)	(17,791,385)
Net loss attributable to common stockholders	(8,327,864)	(28,088,646)	(11,683,215)	(17,828,354)
Basic and diluted net loss per common share(1)	(15.43)	(39.04)	(15.01)	(19.77)

(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 March 2007, the Company received approximately $24.1 million from the issuance of 1,976,527 shares of Series D redeemable convertible preferred stock at $12.15 per share.

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Consolidated Balance Sheets

	December 31, 2006	March 31, 2007 (unaudited)	March 31, Pro Forma (unaudited)
Assets:			
Current assets:			
Cash and cash equivalents	$ 12,126,581	$ 19,852,531	$ 19,852,531
Investments in marketable securities	42,572,468	47,853,240	47,853,240
Prepaid expenses and other current assets	321,275	387,577	387,577
Total current assets	55,020,324	68,093,348	68,093,348
Property and equipment, less accumulated depreciation and amortization of $1,557,316 and $1,852,061 at December 31, 2006 and March 31, 2007, respectively	4,357,912	4,264,661	4,264,661
Other non-current assets	267,338	689,823	689,823
Total Assets	$ 59,645,574	$ 73,047,832	$ 73,047,832
Liabilities			
Current liabilities:			
Accounts payable	1,195,318	1,737,650	1,737,650
Accrued expenses	7,703,775	5,486,732	5,486,732
Current portion of capital lease obligations	1,307,451	1,342,491	1,342,491
Total current liabilities	10,206,544	8,566,873	8,566,873
Warrant liability	608,767	672,418	—
Capital lease obligations, less current portion	2,256,092	1,907,039	1,907,039
Commitments and contingencies			
Series A redeemable convertible preferred stock, $.01 par value, 444,443 shares authorized, issued and outstanding at December 31, 2006 and March 31, 2007 (unaudited) (aggregate liquidation preference $2,500,000 at December 31, 2006 and March 31, 2007), zero pro forma shares outstanding (unaudited)	2,475,689	2,477,053	—
Series B redeemable convertible preferred stock, $.01 par value, 4,936,730 shares authorized, 4,877,056 shares issued and outstanding at December 31, 2006 and March 31, 2007 (unaudited), respectively (aggregate liquidation preference $31,000,000 at December 31, 2006 and March 31, 2007), zero pro forma shares outstanding (unaudited)	30,868,501	30,894,587	—
Series C redeemable convertible preferred stock, $.01 par value, 5,820,020 shares authorized, issued and outstanding at December 31, 2006 and March 31, 2007 (unaudited), respectively (aggregate liquidation preferences $55,999,331 at December 31, 2006 and March 31, 2007), zero pro forma shares outstanding (unaudited)	54,868,868	54,877,663	—
Series D redeemable convertible preferred stock, $.01 par value, 4,930,405 shares authorized, 2,953,878 and 4,930,405 issued and outstanding at December 31, 2006 and March 31, 2007 (unaudited), respectively (aggregate liquidation preference $36,000,000 and $60,000,000 at December 31, 2006 and March 31, 2007), zero pro forma shares outstanding (unaudited)	35,876,547	59,934,392	—
Stockholders' (deficiency) equity:			
Common stock, $.01 par value, 21,333,333 shares authorized, 990,492, 1,152,331 and 17,224,255 shares issued and outstanding at December 31, 2006, March 31, 2007 (unaudited) and March 31, 2007 pro forma (unaudited), respectively	70,288	82,427	1,287,821
Additional paid-in capital	6,066,876	6,981,092	153,959,393
Accumulated other comprehensive income	14,752	16,577	16,577
Deficit accumulated during the development stage	(83,667,350)	(93,362,289)	(92,689,871)
Total stockholders' (deficiency) equity	(77,515,434)	(86,282,193)	62,573,920
	$ 59,645,574	$ 73,047,832	$ 73,047,832

See accompanying notes to consolidated financial statements

Amicus Therapeutics, Inc.
(a development stage company)

Consolidated Statements of Operations

	Three Months Ended March 31,		Period from February 4, 2002 (Inception) to March 31, 2007
	2006 (unaudited)	2007 (unaudited)	(unaudited)
Operating Expenses:			
Research and development	$ 6,027,679	$ 7,084,763	$ 65,888,711
General and administrative	1,900,497	2,849,957	25,641,872
Impairment of leasehold improvements	—	—	1,029,696
Depreciation and amortization	199,224	297,414	1,854,730
In-process research and development	—	—	418,080
Total operating expenses	8,127,400	10,232,134	94,833,089
Loss from operations	(8,127,400)	(10,232,134)	(94,833,089)
Other income (expenses):			
Interest income	237,909	693,303	3,500,883
Interest expense	(51,774)	(92,169)	(1,175,102)
Change in fair value of warrant liability	(343,408)	(63,651)	(367,999)
Other expense	(2,580)	(288)	(1,181,794)
Loss before tax benefit	(8,287,253)	(9,694,939)	(94,057,101)
Income tax benefit	—	—	694,812
Net loss	(8,287,253)	(9,694,939)	(93,362,289)
Deemed dividend	—	—	(19,424,367)
Preferred stock accretion	(40,611)	(40,988)	(491,878)
Net loss attributable to common stockholders	$ (8,327,864)	$ (9,735,927)	$ (113,278,534)
Net loss attributable to common stockholders per common share — basic and diluted	$ (15.43)	$ (10.21)	
Weighted-average common shares outstanding — basic and diluted	539,789	953,959	
Unaudited pro forma net loss		$ (9,694,939)	
Unaudited basic and diluted pro forma net loss per share		$ (0.57)	
Unaudited basic and diluted pro forma weighted-average shares outstanding		17,025,885	

See accompanying notes to consolidated financial statements

Amicus Therapeutics, Inc.
(a development stage company)

Statements of Changes in Stockholders' Deficiency
(Unaudited)

	Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Gain/(Loss)	Deficit Accumulated During the Development State	Total Stockholders' Deficiency
	Shares	Amount				
Balance at December 31, 2006	990,492	$ 70,288	$ 6,066,876	$ 14,752	$ (83,667,350)	$ (77,515,434)
Stock issued from exercise of options	161,839	12,139	193,634	—	—	205,773
Stock-based compensation	—	—	704,549	—	—	704,549
Issuance of stock options to consultants	—	—	57,020	—	—	57,020
Accretion of redeemable convertible preferred stock	—	—	(40,988)	—	—	(40,988)
Unrealized holding gain on available-for-sale securities	—	—	—	1,826	—	1,826
Net loss	—	—	—	—	(9,694,939)	(9,694,939)
Total comprehensive loss	—	—	—	—	—	(9,693,113)
Balance at March 31, 2007	1,152,331	$ 82,427	$ 6,981,092	$ 16,577	$ (93,362,289)	$ (86,282,193)

See accompanying notes

Amicus Therapeutics, Inc.
(a development stage company)

Consolidated Statements of Cash Flows

	Three Months Ended March 31,		Period from February 4, 2002 (Inception) to March 31, 2007
	2006 (unaudited)	2007 (unaudited)	(unaudited)
Operating activities			
Net loss	$ (8,287,253)	$ (9,694,939)	$ (93,362,289)
Adjustments to reconcile net loss to net cash use in operating activities:			
Non-cash interest expense	—	—	525,267
Depreciation and amortization	199,224	297,414	1,852,062
Amortization of non-cash compensation	—	—	522,081
Stock-based compensation	218,965	704,549	3,520,759
Stock-based license payments	—	—	1,220,000
Non-cash charge for stock based compensation issued to consultants	359,019	57,020	748,352
Change in fair value of warrant liability	343,408	63,651	367,999
Impairment of leasehold improvements	—	—	1,029,696
Non-cash charge for in process research and development	—	—	418,080
Beneficial conversion feature related to bridge financing	—	—	135,000
Changes in operating assets and liabilities:			
Prepaid expenses and other current assets	162,543	(66,302)	(387,577)
Other non-current assets	64,401	(154,580)	(443,085)
Accounts payable and accrued expenses	796,078	(1,942,616)	6,956,477
Net cash used in operating activities	(6,143,615)	(10,735,803)	(76,897,178)
Investing activities			
Sale and redemption of marketable securities	10,989,643	21,564,695	64,260,629
Purchases of marketable securities	(2,263,220)	(26,843,642)	(112,214,492)
Purchases of property and equipment	(616,933)	(204,163)	(7,146,419)
Net cash provided by (used in) investing activities	8,109,490	(5,483,110)	(55,100,282)
Financing activities			
Proceeds from the issuance of preferred stock, net of issuance costs	—	24,053,102	143,022,312
Proceeds from the issuance of convertible notes	—	—	5,000,000
Payments of capital lease obligations	(175,114)	(314,013)	(1,791,674)
Proceeds from exercise of stock options	51,000	205,773	388,008
Proceeds from exercise of warrants (common and preferred)	—	—	166,307
Proceeds from capital asset financing arrangement	2,007,966	—	5,065,038
Net cash provided by financing activities	1,883,852	23,944,863	151,849,991
Net increase in cash and cash equivalents	3,849,727	7,725,950	19,852,531
Cash and cash equivalents at beginning of period	6,449,151	12,126,581	—
Cash and cash equivalents at end of period	$10,298,878	$ 19,852,531	$ 19,852,531
Supplemental disclosures of cash flow information			
Cash paid during the period for interest	$ 34,453	$ 92,169	$ 880,183
Non-cash activities			
Warrant issued with convertible notes	$ —	$ —	$ 8,000
Warrant issued with Series B redeemable convertible preferred stock	$ —	$ —	$ 49,950
Conversion of notes payable to Series B redeemable convertible preferred stock	$ —	$ —	$ 5,000,000
Accretion of redeemable convertible preferred stock	$ 40,611	$ 40,988	$ 491,878
Beneficial conversion feature related to issuance of the second tranche of Series C redeemable convertible preferred stock	$ —	$ —	$ 19,424,367

See accompanying notes to consolidated financial statements

Amicus Therapeutics, Inc.
(a development stage company)

Notes to Unaudited Financial Statements

1. Description of Business and Significant Accounting Policies

Corporate Information, Status of Operations and Management Plans

Amicus Therapeutics, Inc. (the "Company") was incorporated on February 4, 2002 in Delaware for the purpose of creating a premier drug development company at the forefront of therapy for human genetic diseases initially based on intellectual property in-licensed from Mount Sinai School of Medicine. The Company's activities since inception have consisted principally of raising capital, establishing facilities, and performing research and development. Accordingly, the Company is considered to be in the development stage.

The Company has an accumulated deficit of approximately $93.4 million at March 31, 2007 and anticipates incurring losses through the year 2007 and beyond. The Company has not yet generated revenues and has been able to fund its operating losses to date through 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.

Management believes that the Company's current cash position and the additional funds received are sufficient to cover its cash flow requirements until at least March 31, 2008.

Pro Forma Information

The unaudited pro forma balance sheet data as of March 31, 2007 gives effect to the elimination of the Company's warrant liability of $672,418, and the automatic conversion of all outstanding shares of the Company's series A, B, C, and D redeemable convertible preferred stock into an aggregate of 16,071,924 shares of common stock upon completion of the Company's initial public offering. The pro forma information excludes shares of common stock issuable in connection with the exercise of outstanding warrants to purchase shares of series B redeemable convertible preferred stock. Upon the closing of this offering, these warrants will be automatically exercised for 40,797 shares of series B redeemable convertible preferred stock, which will then be automatically converted into shares of common stock on a one for one basis.

Pro forma net loss per share for the three month period ended March 31, 2007 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 closing of the Company's initial public offering, as if they had occurred at the beginning of the period.

Basis of Presentation

The accompanying financial information as of March 31, 2007 and for the three months ended March 31, 2006 and 2007 has been prepared by the Company, without audit, pursuant to the rules and regulations of the Securities and Exchange Commission. Certain information and footnote disclosures normally included in financial statements prepared in accordance with U.S. generally accepted accounting principles have been condensed or omitted pursuant to such rules and regulations. The December 31, 2006 consolidated balance sheet was derived from audited financial statements, but does not include all disclosures required by U.S. generally accepted accounting principles in the United States. However, the Company believes that the disclosures are adequate to make the information presented not misleading. These consolidated financial statements should be read in conjunction with the annual consolidated financial statements and the notes thereto, included elsewhere in this prospectus.

Amicus Therapeutics, Inc.
(a development stage company)

Notes to Unaudited Financial Statements — (Continued)

In the opinion of management the unaudited financial information as of March 31, 2007 and for the three months ended March 31, 2006 and 2007 reflects all adjustments, which are normal recurring adjustments, necessary to present a fair statement of financial position, results of operations and cash flows. The results of operations for the three months ended March 31, 2007 are necessarily indicative of the operating results for the full fiscal year or any future periods.

Other Assets

Included in other assets at March 31, 2007 are capitalized offering costs, which are incremental costs directly attributable to the Company's proposed initial public offering of $422,000. Upon consummation of the Company's initial public offering, such costs will be applied to the offering proceeds; however, in the event the offering is not consummated, such costs will be expensed.

Income Taxes

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

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

2. Stock-Based Compensation

During the three months ended March 31, 2007, the Company recorded compensation expense of approximately $0.8 million. The compensation expense had no impact on the Company's cash flows from operations and financing activities. As of March 31, 2007, the total unrecognized compensation cost related to non-vested stock options granted was $7.5 million and is expected to be recognized over a weighted average period of 2.5 years.

Amicus Therapeutics, Inc.
(a development stage company)

Notes to Unaudited Financial Statements — (Continued)

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

	Three Months Ended March 31, 2006	Three Months Ended March 31, 2007
Expected stock price volatility	72.7%	78.8%
Risk free interest rate	4.6%	4.7%
Expected life of options (years)	6.25	6.25
Expected annual dividend per share	$ 0.00	$ 0.00

A summary of option activities related to the Company's stock options for the three months ended March 31, 2007 is as follows:

	Options Outstanding			
	Number of Shares (in thousands)	Weighted Average Exercise Price	Weighted Average Remaining Contractual Life	Weighted Average Grant Date Fair Value (in millions)
Balance at December 31, 2006	1,868.5	$ 4.27		
Options granted	17.9	$ 9.90		
Options exercised	(161.8)	$ 1.35		
Options forfeited	(10.5)	$ 8.17		
Balance at March 31, 2007	1,714.1	$ 4.57	8.1 years	$ 15.2
Vested and unvested expected to vest, March 31, 2007	1,568.2	$ 4.50	8.1 years	$ 13.9
Exercisable at March 31, 2007	529.4	$ 4.42	7.6 years	$ 4.8

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

The Company calculates net loss per share in accordance with SFAS No. 128, *Earnings Per Share*. The Company has determined that its series A, B, C, and D redeemable convertible preferred stock 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.

Amicus Therapeutics, Inc.
(a development stage company)

Notes to Unaudited Financial Statements — (Continued)

	Three Months Ended March 31,	
	2006	**2007**
Statement of Operations		
Net loss attributable to common stockholders	$ (8,327,864)	$(9,735,927)
Net loss attributable to common stockholders per common share — basic and diluted	$ (15.43)	$ (10.21)

4. Comprehensive Loss

The components of comprehensive loss are as follows:

	Three Months Ended March 31,	
	2006	**2007**
Net loss	$ (8,287,253)	$ (9,694,939)
Change in unrealized net gain on marketable securities	10,819	1,826
Comprehensive loss	$ (8,276,434)	$ (9,693,113)

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

5. Capital Structure

Redeemable Convertible Preferred Stock

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

At March 31, 2007 the Company is authorized to issue 444,443 shares of series A redeemable convertible preferred stock ("Series A"), 4,936,740 shares of series B redeemable convertible preferred stock ("Series B"), 5,820,020 shares of series C redeemable convertible preferred stock ("Series C") and 4,930,405 shares of series D redeemable convertible preferred stock ("Series D"). At March 31, 2007, the Company had outstanding 444,443 shares, 4,877,056 shares, 5,820,020 shares, and 4,930,405 shares of Series A, B, C, and D, respectively.

Amicus Therapeutics, Inc.
(a development stage company)

Notes to Unaudited Financial Statements — (Continued)

As of March 31, 2007 Series A, Series B, Series C, and Series D are recorded at its stated values (estimated fair value of $5.63 per share, $6.38 per share, $9.45 per share, and $12.15 per share, respectively, less issuance costs and accretion adjustments).

	Series A		Series B		Series C		Series D	
	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount
Balance at December 31, 2006	444,443	$ 2,475,689	4,877,056	$ 30,868,501	5,820,020	$ 54,868,868	2,953,878	$ 35,876,547
Issuance of Series D at $12.15 per share	—	—	—	—	—	—	1,976,527	24,053,102
Accretion to redemption value	—	1,364	—	26,086	—	8,795	—	4,743
Balance at March 31, 2007	444,443	$ 2,477,053	4,877,056	$ 30,894,587	5,820,020	$ 54,877,663	4,930,405	$ 59,934,392

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5,000,000 Shares

Common Stock

PROSPECTUS

May 30, 2007