Filed Pursuant to Rule 424(b)(4)
Registration No. 333-109700

5,400,000 Shares

Common Stock

This is an initial public offering of shares of common stock of GTx, Inc. All of the 5,400,000 shares of common stock are being sold by the company.

Prior to this offering, there has been no public market for the common stock. The common stock has been approved for quotation on the Nasdaq National Market under the symbol “GTXI”.

See “Risk Factors” on page 7 to read about factors you should consider before buying shares of the common stock.

Neither the Securities and Exchange Commission nor any other regulatory body has approved or disapproved of these securities or passed upon the accuracy or adequacy of this prospectus. Any representation to the contrary is a criminal offense.

	Per Share	Total
Initial public offering price	$14.500	$78,300,000
Underwriting discount	$ 1.015	$ 5,481,000
Proceeds, before expenses, to GTx	$13.485	$72,819,000

To the extent that the underwriters sell more than 5,400,000 shares of common stock, the underwriters have the option to purchase up to an additional 810,000 shares from GTx at the initial public offering price less the underwriting discount.

The underwriters expect to deliver the shares against payment in New York, New York on February 6, 2004.

Goldman, Sachs & Co.

SG Cowen

Lazard

Prospectus dated February 2, 2004.

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PROSPECTUS SUMMARY

This summary highlights information contained elsewhere in this prospectus which we consider important to investors. You should read the entire prospectus carefully before making an investment in our common stock.

Our Business

GTx is a biopharmaceutical company dedicated to the discovery, development and commercialization of therapeutics primarily related to the treatment of serious men's health conditions. Our drug discovery and development programs are focused on small molecules that selectively modulate the effects of estrogens and androgens, two essential classes of hormones. We currently have two product candidates that are in human clinical trials. We are developing Acapodene, our most advanced product candidate, through clinical trials for two separate indications: (1) a Phase IIb clinical trial for the reduction in the incidence of prostate cancer in men with precancerous prostate lesions and (2) a pivotal Phase III clinical trial for the treatment of serious side effects of advanced prostate cancer therapy. Phase IIb clinical trials typically evaluate efficacy and safety and determine the optimal dosing regimen. Pivotal Phase III clinical trials typically further evaluate efficacy and safety in an expanded patient population. We are initially developing our second product candidate, Andarine, for the treatment of potentially life-threatening muscle wasting weight loss from various types of cancer. Andarine is the most advanced of our internally discovered portfolio of compounds designed to modulate the effects of hormones.

We plan to build a specialized sales and marketing capability to market our product candidates directly to the relatively small and concentrated community of urologists and medical oncologists in the United States and to seek collaborators to commercialize our product candidates outside the United States and to broader target physician markets.

Acapodene for the Reduction in the Incidence of Prostate Cancer in Men with Precancerous Prostate Lesions

Prostate cancer is one of the most commonly diagnosed cancers in men and the second leading cause of cancer-related deaths in the United States. We believe that treating the precancerous lesions of prostate cancer known as high grade PIN may be an effective approach to this disease. In the United States, there are over 115,000 new cases of precancerous prostate lesions diagnosed each year, and an estimated 9.4 million men unknowingly harbor this condition. As there is currently no therapy for the treatment of this condition, we believe this represents a significant unmet medical need.

A planned interim analysis of the first 120 patients in our Phase IIb clinical trial showed that treatment with Acapodene resulted in a 26% to 57% reduction in the incidence of prostate cancer compared to the placebo group 12 months after the diagnosis of precancerous prostate lesions. While these interim results do not necessarily predict favorable results from this trial or any future trial, we believe that the results of this interim analysis suggest that Acapodene may be effective in reducing the incidence of prostate cancer in men with precancerous prostate lesions. The last of the 515 enrolled patients is scheduled to complete this trial in May 2004, with final results expected in the third quarter of 2004. We believe that if the results of this Phase IIb clinical trial and an anticipated single Phase III clinical trial are positive, this trial and the anticipated Phase III clinical trial will be sufficient to support an application with the FDA for marketing approval of Acapodene for this indication. However, even if we file this application, it may not result in marketing approval from the FDA.

Acapodene for the Treatment of Side Effects of Advanced Prostate Cancer Therapy

The standard medical treatment for men who have advanced, recurrent or metastatic prostate cancer is androgen deprivation therapy, which reduces blood level of testosterone, the growth factor for prostate cancer. In the United States, more than 675,000 men are currently being treated by this therapy, with over 120,000 new patients started on this therapy each year. Advanced prostate cancer therapy has serious side effects, including: severe bone loss, or osteoporosis, leading to skeletal fractures; hot flashes; and breast pain and enlargement. We are developing Acapodene as a treatment for these side effects. Because there are no drugs approved by the FDA for the treatment of these side effects, we believe that there could be a substantial market for Acapodene for this indication.

We have completed two six-month Phase II clinical trials of Acapodene for the treatment of osteoporosis and hot flashes in men receiving advanced prostate cancer therapy. Phase II clinical trials are typically conducted in a limited population to evaluate dosage, safety and, preliminarily, efficacy for a specific indication. The first Phase II clinical trial evaluated the use of Acapodene shortly after initiation of therapy, and the second Phase II clinical trial evaluated Acapodene in patient who had been receiving therapy for more than 12 months. The analysis of the second trial showed that Acapodene at the highest tested dose produced an increase in bone mineral density, an indicator of bone strength, and a reduction in the frequency of hot flashes. Our pivotal Phase III clinical trial for this indication, which we commenced in November 2003, is principally based on the results of the second Phase II clinical trial. The Phase III trial will evaluate the effect of Acapodene on the incidence of skeletal fractures as well as on bone loss and the incidence of hot flashes and breast pain and enlargement. The increase in bone mineral density and reduction in the frequency of hot flashes observed in the second Phase II clinical trial are not necessarily indicative of the results that will be demonstrated in our pivotal Phase III trial.

Andarine for the Treatment of Muscle Wasting Weight Loss from Cancer

We believe that Andarine has the potential to treat a variety of men's health conditions, including testosterone deficiency in aging men and related diseases, including osteoporosis and muscle wasting. Our strategy is to develop Andarine initially for the treatment of muscle wasting weight loss from various types of cancer, which is known as cancer cachexia. We selected this indication because it represents a potentially large market and, we believe, has a relatively well-defined clinical and regulatory process. There are approximately 1.3 million patients diagnosed with cancer each year in the United States. Muscle wasting weight loss afflicts approximately one-third of newly-diagnosed cancer patients. There are no drugs that have been approved by the FDA for the treatment of muscle wasting weight loss from cancer.

We have completed three Phase I clinical trials of Andarine in which Andarine was well-tolerated by all participants with no serious adverse events. Phase I clinical trials are designed to confirm safety and tolerance. In one of these Phase I clinical trials, we measured increased levels of a growth factor in the blood of some men who received Andarine, which suggests that Andarine may promote growth activity and thus may be an effective treatment for muscle wasting weight loss from cancer. However, Phase I clinical trials are not designed to show efficacy, and these early observations are not necessarily indicative of the results that will be demonstrated in future clinical trials. We plan to commence a placebo-controlled, dose-finding Phase II clinical trial of Andarine for the treatment of muscle wasting weight loss from non-small cell lung cancer in the first half of 2004.

Pipeline

We have multiple product candidates that we are evaluating in preclinical and toxicology studies to support the possible commencement of clinical trials. We are developing our current preclinical product candidates for the treatment of major indications in men's health, including:

- Prostarine for the treatment of a benign prostate enlargement that results in obstruction of the urinary tract;
- Ostarine for the treatment of osteoporosis and testosterone deficiency in aging men; and
- Andromustine for the treatment of prostate cancer that is not responsive to androgen deprivation therapy.

We believe that our drug discovery capabilities position us well to design and develop nonsteroidal small molecule drugs that modulate the effects of hormones.

Early Stage Company

All of our product candidates are undergoing clinical trials or are in early stages of development, and failure is common and can occur at any stage of development. To date, we have not obtained regulatory approval for the commercial sale of any products, and we have not received any revenues from the commercial sale of products. Industry sources report that the preparation and submission of new drug applications, or NDAs, which are required for regulatory approval, generally take six months to one year to complete after completion of a pivotal clinical trial. Industry sources also report that approximately 75% of all NDAs are approved by the FDA, and the FDA reports that most NDAs are approved within 12 to 24 months of submission, although it may take longer if additional information is required by the FDA or for other reasons. The Pharmaceutical Research and Manufacturers of America reports that only one out of five product candidates that enter clinical trials will ultimately be approved by the FDA for commercial sale. We do not expect any of our product candidates, if successfully developed, to receive regulatory approval for commercial sale for at least several years. Any products that we sell may not become commercially successful.

For the nine months ended September 30, 2003, our net loss was $9.6 million, and as of that date, we had a deficit accumulated during the development stage of $144.9 million, of which $110.6 million related to non-cash dividends and adjustments to the preferred stock redemption value.

Company Information

We were originally incorporated under the name Genotherapeutics, Inc. in Tennessee in September 1997. We changed our name to GTx, Inc. in 2001, and we reincorporated in Delaware in 2003. Our principal executive office is located at 3 N. Dunlap Street, 3rd Floor, Van Vleet Building, Memphis, Tennessee, and our telephone number is (901) 523-9700. Our website address is www.gtxinc.com. The information contained in our website is not a part of this prospectus.

Service marks, trademarks and trade names referred to in this prospectus are the property of their respective owners.

The Offering

Common stock offered by GTx	5,400,000 shares
Common stock to be outstanding after the offering	24,592,753 shares
Nasdaq National Market symbol	GTXI
Use of proceeds	We expect to use the net proceeds from this offering to fund our clinical trials and other research and development activities and for general corporate purposes.

The number of shares of common stock to be outstanding after this offering is based on the number of shares outstanding as of December 31, 2003 and excludes:

- 828,750 shares of common stock issuable upon exercise of options issued under our current stock option plans, at a weighted average exercise price of $6.18 per share;
- 453,050 shares of common stock reserved for future issuance under our current stock option plans and 1,700,000 shares of common stock reserved for future issuance under our 2004 Equity Incentive Plan and 2004 Non-Employee Directors' Stock Option Plan, which will become effective upon the completion of this offering; and
- shares of common stock issuable upon conversion of outstanding preferred stock in satisfaction of dividends that will accrue on our preferred stock between January 1, 2004 and the closing of this offering.

Except as otherwise noted, all information in this prospectus:

- assumes no exercise of the underwriters' over-allotment option;
- gives effect to the conversion into common stock of all outstanding shares of preferred stock and dividends accrued thereon through December 31, 2003; and
- gives effect to an 8.5-for-1 stock split of our common stock, which was effected on January 14, 2004.

Summary Financial Information

You should read the summary financial information below in conjunction with "Management's Discussion and Analysis of Financial Condition and Results of Operations" and the financial statements, notes thereto and other financial information included elsewhere in this prospectus. We derived the information presented for the nine-month periods ended September 30, 2002 and September 30, 2003 from unaudited financial statements which include, in the opinion of management, all adjustments, consisting only of normal recurring accruals, necessary to present fairly the information for such periods. The results for the nine-month period ended September 30, 2003 are not necessarily indicative of the results to be expected for the full fiscal year.

We computed pro forma net loss per share for the year ended December 31, 2002 and the nine months ended September 30, 2003 by using the weighted average number of shares of common stock outstanding, including the pro forma effects of the automatic conversion of our preferred stock and dividends accrued thereon into shares of common stock effective upon the closing of the offering as if such conversion occurred on January 1, 2002 and January 1, 2003 or at the date of the original issuance, if later. The calculation of pro forma net loss per share attributable to common stockholders excludes incremental common stock issuable upon exercise of options, as its effect would be antidilutive.

	Year Ended December 31,			Nine Months Ended September 30,	
	2000	2001	2002	2002	2003
				(unaudited)	
	(in thousands, except share and per share data)				
Statement of Operations Data:					
Operating expenses:					
Research and development	$ 2,679	$ 5,744	$ 9,285	$ 6,408	$ 7,123
General and administrative	1,203	2,187	2,405	1,830	2,339
Depreciation	80	215	332	240	264
Total operating expenses	3,962	8,146	12,022	8,478	9,726
Interest income	150	83	156	105	79
Net loss	(3,812)	(8,063)	(11,866)	(8,373)	(9,647)
Accrued preferred stock dividends	(297)	(790)	(2,147)	(1,466)	(2,300)
Adjustment to preferred stock redemption value	(21,077)	(57)	(7,220)	(7,147)	(76,666)
Net loss attributable to common stockholders	$ (25,186)	$ (8,910)	$ (21,233)	$ (16,986)	$ (88,613)
Net loss per share attributable to common stockholders, basic and diluted:	$ (3.26)	$ (1.15)	$ (2.75)	$ (2.20)	$ (11.46)
Weighted average shares used in computing net loss per share attributable to common stockholders, basic and diluted:	7,735,000	7,735,000	7,735,000	7,735,000	7,735,000
Pro forma net loss per share attributable to common stockholders — basic and diluted			$ (0.80)		$ (0.59)
Shares used in computing pro forma net loss per share attributable to common stockholders — basic and diluted			14,811,786		16,455,728

The following table presents a summary of our balance sheet as of September 30, 2003:

- on an actual basis; and
- on an as adjusted basis to reflect the conversion into common stock of all outstanding shares of preferred stock and dividends accrued thereon through September 30, 2003 and the sale in this offering of 5,400,000 shares of common stock at the initial public offering price of $14.50 per share, after deducting the underwriting discounts and commissions and estimated offering expenses payable by us.

	As of September 30, 2003	
	Actual	As Adjusted
	(in thousands)	
Balance Sheet Data:		
Cash and cash equivalents	$ 19,788	$ 90,307
Working capital	18,280	88,799
Total assets	21,107	91,626
Cumulative redeemable convertible preferred stock	162,978	—
Deficit accumulated during development stage	(144,891)	(145,160)
Total stockholders' (deficit) equity	(143,838)	89,660

RISK FACTORS

Risks Related to Our Financial Results and Need for Additional Financing

We have incurred losses since inception and anticipate that we will incur continued losses for the foreseeable future.

We are a development stage company with a limited operating history. As of September 30, 2003, we had a deficit accumulated during the development stage of $144.9 million, of which $110.6 million related to non-cash dividends and adjustments to the preferred stock redemption value. We have incurred losses in each year since our inception in 1997. Net losses were $11.9 million in 2002, $8.1 million in 2001 and $3.8 million in 2000. For the nine months ended September 30, 2003, net losses were $9.6 million. We expect to continue to incur significant and increasing operating losses for the foreseeable future. These losses have had and will continue to have an adverse effect on our stockholders' equity and working capital.

Because of the numerous risks and uncertainties associated with developing small molecule drugs, we are unable to predict the extent of any future losses or when we will become profitable, if at all. Currently, we have no products approved for commercial sale, and, to date, we have not generated any product revenue. We have financed our operations and internal growth almost exclusively through private placements of preferred stock. We have devoted substantially all of our efforts to research and development, including clinical trials.

We expect our research and development expenses to increase in connection with the conduct of clinical trials. In addition, subject to regulatory approval of any of our product candidates, we expect to incur sales and marketing and increased manufacturing expenses.

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 will need to raise additional capital to:

- fund our operations and clinical trials;
- continue our research and development; and
- commercialize our product candidates, if any such product candidates receive regulatory approval for commercial sale.

We believe that the net proceeds from this offering, our existing cash resources and interest on these funds will be sufficient to meet our projected operating requirements through the end of 2005. Our future funding requirements will depend on many factors, including:

- the scope, rate of progress and cost of our clinical trials and other research and development activities;
- future clinical trial results;
- the terms and timing of any collaborative, licensing and other arrangements that we may establish;
- the cost and timing of regulatory approvals;
- the cost and timing of establishing sales, marketing and distribution capabilities;
- the cost of establishing clinical and commercial supplies of our product candidates and any products that we may develop;
- the effect of competing technological and market developments;

- the cost of filing, prosecuting, defending and enforcing any patent claims and other intellectual property rights; and

- the extent to which we acquire or invest in businesses, products and technologies, although we currently have no commitments or agreements relating to any of these types of transactions.

Until we can generate a sufficient amount of product revenue, if ever, we expect to finance future cash needs through public or private equity offerings, debt financings or corporate collaboration and licensing arrangements, as well as through interest income earned on cash balances.

If we raise additional funds by issuing equity securities, our stockholders may experience dilution. Debt financing, if available, may involve restrictive covenants. Any debt financing or additional equity that we raise may contain terms that are not favorable to us or our stockholders. If we raise additional funds through collaboration and licensing arrangements with third parties, it will be necessary to relinquish some rights to our technologies or our product candidates, or grant licenses on terms that are not favorable to us.

Risks Related to Development of Product Candidates

We will not be able to commercialize our product candidates if our preclinical studies do not produce successful results or our clinical trials do not demonstrate safety and efficacy in humans.

Preclinical and clinical testing is expensive, can take many years and has an uncertain outcome. Success in preclinical testing and early clinical trials does not ensure that later clinical trials will be successful, and interim results of a clinical trial do not necessarily predict final results. 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 commercialize our product candidates, including:

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

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

- registration or enrollment in our clinical trials may be slower than we currently anticipate, resulting in significant delays;

- we might have to suspend or terminate our clinical trials if the participating patients are being exposed to unacceptable health risks;

- regulators or institutional review boards may suspend or terminate clinical research for various reasons, including noncompliance with regulatory requirements; and

- the effects of our product candidates may not be the desired effects or may include undesirable side effects.

If any of these events were to occur and, as a result, we have significant delays in or termination of clinical trials, our costs would increase and our ability to generate revenue could be impaired, which would adversely impact our financial results.

Risks Related to Our Dependence on Third Parties

If third parties do not manufacture our product candidates in sufficient quantities and at an acceptable cost, clinical development and commercialization of our product candidates would be delayed.

We do not currently own or operate manufacturing facilities and rely and expect to continue to rely on third parties for the production of clinical and commercial quantities of our product candidates. 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 product candidates on a timely and competitive basis.

We have agreed to purchase from Orion Corporation our worldwide requirements of Acapodene in finished tablet form at specified transfer prices under a license and supply agreement. We rely on Orion as a single source supplier for Acapodene. In the event that Orion terminates the agreement under specified circumstances, we would not be able to manufacture Acapodene until Orion's patents with respect to the composition of matter of toremifene, the active pharmaceutical ingredient in Acapodene, expire. This could delay the development of and impair our ability to commercialize this product candidate. In addition, Orion may terminate its obligation to supply us with toremifene under specified circumstances. Under some of these circumstances, we will have the right to manufacture Acapodene, but we would be required to make arrangements with a qualified alternative supplier to do so.

In addition, we currently rely on ChemSyn Laboratories, a department of EaglePicher Technologies, LLC, as our single supplier of Andarine. We do not have a contract with ChemSyn for the supply of Andarine for full-scale commercialization.

We may not be able to maintain or renew our existing or any other third-party manufacturing arrangements on acceptable terms, if at all. If we are unable to continue relationships with Orion for Acapodene and ChemSyn for Andarine, or to do so at an acceptable cost, or if these suppliers fail to meet our requirements for these product candidates for any reason, we would be required to obtain alternate suppliers, which we may not be permitted to do for Acapodene under our license agreement with Orion in some circumstances. Any inability to obtain alternate suppliers, including an inability to obtain approval of an alternate supplier from the Food and Drug Administration, or FDA, would delay or prevent the clinical development and commercialization of these product candidates.

Use of third-party manufacturers may increase the risk that we will not have adequate supplies of our product candidates.

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;
- 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.

If we are not able to obtain adequate supplies of our product candidates, it will be more difficult for us to develop our product candidates and compete effectively. Our product candidates and any products that we may develop may compete with other product candidates and products for access to manufacturing facilities. For example, the active pharmaceutical ingredient in Acapodene is also the active pharmaceutical ingredient in Fareston. Orion also manufactures Fareston for Shire Pharmaceuticals Group, which markets it in the United States for the treatment of advanced breast cancer in post-menopausal women.

Our present or future manufacturing partners may not be able to comply with FDA mandated current Good Manufacturing Practice regulations, other FDA regulatory requirements or similar regulatory requirements outside the United States. Failure of our third-party manufacturers or us 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 supplies of our product candidates.

If third parties on whom we rely do not perform as contractually required or expected, we may not be able to obtain regulatory approval for or commercialize our product candidates.

We do not have the ability to independently conduct clinical trials for our product candidates, and we must rely on third parties, such as contract research organizations, medical institutions, clinical investigators and contract laboratories to conduct our clinical trials. In addition, we rely on third parties to assist with our preclinical development of product candidates. If these third parties do not successfully carry out their contractual duties or regulatory obligations or meet expected deadlines, if the third parties need to be replaced, or if the quality or accuracy of the data they obtain is compromised due to the failure to adhere to our clinical protocols or regulatory requirements or for other reasons, our preclinical development activities or clinical trials may be extended, delayed, suspended or terminated, and we may not be able to obtain regulatory approval for or successfully commercialize our product candidates.

We expect to be dependent upon collaborative arrangements to complete the development and commercialization of some of our product candidates. These collaborative arrangements may place the development of our product candidates outside our control, may require us to relinquish important rights or may otherwise be on terms unfavorable to us.

We may not be successful in entering into collaborative arrangements with third parties. If we fail to enter into additional collaborative arrangements on favorable terms, it could delay or impair our ability to develop and commercialize our product candidates and could increase our costs of development and commercialization. Dependence on collaborative arrangements will subject us to a number of risks, including:

- we may not be able to control the amount and timing of resources that our collaborators may devote to the product candidates;
- our collaborators may experience financial difficulties;
- we may be required to relinquish important rights such as marketing and distribution rights;
- should a collaborator fail to develop or commercialize one of our compounds or product candidates, we may not receive any future milestone payments and will not receive any royalties for this compound or product candidate;
- business combinations or significant changes in a collaborator’s business strategy may also adversely affect a collaborator’s willingness or ability to complete its obligations under any arrangement;
- a collaborator could independently move forward with a competing product candidate developed either independently or in collaboration with others, including our competitors; and
- the collaborative arrangements are often terminated or allowed to expire, which would delay the development and may increase the cost of developing our product candidates.

Risks Related to Our Intellectual Property

Our license agreement with Orion Corporation is limited to specific fields of use of toremifene and will limit our ability to market Acapodene.

Our license from Orion is limited to the use of toremifene for the prevention and treatment of prostate cancer and the prevention and treatment of osteoporosis, hot flashes and breast pain and enlargement as side effects of advanced prostate cancer therapy. The license is exclusive in North America and Japan in these fields. Orion has licensed Shire Pharmaceuticals Group in the United States and other parties elsewhere in the world to market, sell and distribute toremifene for the treatment of advanced breast cancer and could license other parties to market, sell and distribute toremifene for other indications in the United States and elsewhere.

Under the terms of our license agreement with Orion, Orion may require us to modify our final Acapodene development plans for specified major markets if such development plans could adversely affect toremifene outside the fields that Orion has licensed to us. Although we do not believe that our development plans adversely affect toremifene outside the licensed fields, any future modifications to our plans may limit our ability to maximize the commercial potential of Acapodene.

Furthermore, we and our affiliates are prohibited from selling a product that competes with toremifene in the licensed field in major countries located outside the European Union during the term of the agreement and in major countries in the European Union through October 2006. While we are not currently developing any product candidate that would compete with toremifene in the licensed field, this noncompetition provision may limit our ability to commercialize any other compounds in the licensed field even if we believe that other compounds have more commercial potential than Acapodene. The binding effect of this noncompetition provision on our affiliates, as well as Orion's right to terminate the agreement if we are acquired by a direct competitor of Orion with respect to toremifene, may make it more difficult for us to be acquired by some potential buyers even if we determine that a sale of the company would be in the best interests of our stockholders.

If some or all of our patents expire or are invalidated or are unenforceable, or if some or all of our patent applications do not yield issued patents or yield patents with narrow claims, we may be subject to competition from third parties with products with the same active pharmaceutical ingredients as our product candidates.

Our commercial success will depend in part on obtaining and maintaining patent and trade secret protection for our product candidates, the methods used to manufacture these product candidates and the methods for treating patients using these product candidates. We will be able to protect our product candidates and our technologies from unauthorized use by third parties only to the extent that valid and enforceable patents or trade secrets cover them.

Even if our product candidates and technologies are covered by valid and enforceable patents, the patents will provide protection only for a limited amount of time. For example, the patents that we have licensed from Orion covering the composition of matter of toremifene expire in the United States in 2009 and have expired in countries outside the United States or are likely to expire in such countries before we commercialize Acapodene. As a result, outside the United States and in the United States after 2009, we will need to rely primarily on the protection afforded by method of use patents that have issued or may issue in respect of our owned or licensed patent applications relating to the use of Acapodene for the relevant indications. To date, most of these pending method of use patent applications have not yielded issued patents.

Our and our collaborators' ability to obtain patents is highly uncertain because, to date, some legal principles remain unresolved and there has not been a consistent policy regarding the breadth or interpretation of claims allowed in patents in the United States, and the specific content of patents and patent applications that are necessary to support and interpret patent claims is highly uncertain due to the complex nature of the relevant legal, scientific and factual issues. Furthermore, the

policies governing biotechnology patents outside the United States are even more uncertain. 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.

Even if patents are issued regarding our product candidates or methods of using them, those patents can be challenged by our competitors who can argue such patents are invalid. Patents also will not protect our product candidates if competitors devise ways of making these product candidates without legally infringing our patents. The Federal Food, Drug, and Cosmetic Act and FDA regulations and policies provide incentives to manufacturers to challenge patent validity or create modified, noninfringed versions of a drug in order to facilitate the approval of abbreviated new drug applications for generic substitutes. These same types of incentives encourage manufacturers to submit new drug applications that rely on literature and clinical data not prepared for or by the drug sponsor. See “Government Regulation” beginning on page 53 for additional information.

We also rely on trade secrets to protect our technology, especially where we do not believe that patent protection is appropriate or obtainable. However, trade secrets are difficult to protect. Our employees, consultants, contractors, outside scientific collaborators and other advisors may unintentionally or willfully disclose our confidential information to competitors, and confidentiality agreements may not provide an adequate remedy in the event of unauthorized disclosure of confidential information. Enforcing a claim that a third party illegally obtained and is using our trade secrets is expensive and time-consuming, and the outcome is unpredictable. Moreover, our competitors may independently develop equivalent knowledge, methods and know-how. Failure to obtain or maintain trade secret protection could adversely affect our competitive business position.

Off-label sale or use of generic toremifene products could decrease sales of Acapodene and could lead to pricing pressure if such products become available at competitive prices and in dosages that are appropriate for the indications for which we are developing Acapodene.

In all countries in which we hold or have licensed rights to patents or patent applications related to Acapodene, the composition of matter patents will expire before the method of use patents. Method of use patents may not protect Acapodene from the risk of off-label sale or use of the subject compounds. Physicians are permitted to prescribe legally available drugs for uses that are not described in the drug’s labeling and that differ from those uses tested and approved by the FDA. Such off-label uses are common across medical specialties. Off-label sales would adversely affect our ability to generate revenue from the sale of Acapodene, if approved for commercial sale.

In the event that patents issue in respect of our pending method of use patent applications, after the expiration of the patent covering the composition of matter of toremifene in a particular country, competitors could market and sell generic versions of toremifene at doses and in formulations that are equivalent to Acapodene for uses other than the indications for Acapodene covered by these pending method of use patent applications, and physicians would be permitted to prescribe these generic versions of toremifene for indications that are protected by these method of use patents and pending patent applications. Moreover, because Orion has licensed and could further license other parties to market, sell and distribute toremifene for other indications in the United States and elsewhere, physicians could prescribe these products sold pursuant to another Orion license off-label. This further increases the risk of off-label competition developing for Acapodene for the indications for which we are developing this product candidate. In addition, if no patents issue in respect of our pending method of use patent applications related to the use of Acapodene, after the expiration of the patent covering the composition of matter of toremifene in a particular country, competitors could market and sell generic versions of toremifene at doses and in formulations equivalent to Acapodene for the indications covered by our pending method of use patent applications.

If we infringe intellectual property rights of third parties, it may increase our costs or prevent us from being able to commercialize our product candidates.

There is a risk that we are infringing the proprietary rights of third parties because numerous United States and foreign issued patents and pending patent applications, which are owned by third parties, exist in the fields that are the focus of our drug discovery and development efforts. Others might have been the first to make the inventions covered by each of our or our licensors' pending patent applications and issued patents and might have been the first to file patent applications for these inventions. In addition, because patent applications can take many years to issue, there may be currently pending applications, unknown to us, which may later result in issued patents that cover the production, manufacture, commercialization or use of our product candidates. In addition, the production, manufacture, commercialization or use of our product candidates may infringe existing patents of which we are not aware. Defending ourselves against third-party claims, including litigation in particular, would be costly and time consuming and would divert management's attention from our business, which could lead to delays in our development or commercialization efforts. If third parties are successful in their claims, we might have to pay substantial damages or take other actions that are adverse to our business.

As a result of intellectual property infringement claims, or to avoid potential claims, we might:

- be prohibited from selling or licensing any product that we may develop unless the patent holder licenses the patent to us, which it is not required to do;
- be required to pay substantial royalties or grant a cross license to our patents to another patent holder; or
- be required to redesign the formulation of a product candidate so it does not infringe, which may not be possible or could require substantial funds and time.

Risk Related to Regulatory Approval of Our Product Candidates

If we are not able to obtain 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 and the activities associated with their development and commercialization 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. We have not received regulatory approval to market any of our product candidates in any jurisdiction and have only limited experience in preparing and filing the applications necessary to gain regulatory approvals. The process of obtaining regulatory approvals is expensive, often takes many years, if approval is obtained at all, and can vary substantially based upon the type, complexity and novelty of the product candidates involved.

Changes in the regulatory approval policy during the development period, changes in or the enactment of additional regulations or statutes, or changes in regulatory review for each submitted product application, may cause delays in the approval or rejection of an application. Even if the FDA approves a product candidate, the approval may impose significant restrictions on the indicated uses, conditions for use, labeling, advertising, promotion, marketing and/or production of such product, and may impose ongoing requirements for post-approval studies, including additional research and development and clinical trials. The FDA also may impose various civil or criminal sanctions for failure to comply with regulatory requirements, including withdrawal of product approval.

Furthermore, the approval procedure and the time required to obtain approval varies among countries and can involve additional testing beyond that required by the FDA. Approval by one regulatory authority does not ensure approval by regulatory authorities in other jurisdictions.

The FDA has 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. For example, we believe that if the results of our ongoing Phase IIb clinical trial and an anticipated Phase III clinical trial of Acapodene for the reduction in the incidence of prostate cancer in men with high grade PIN are positive, our Phase IIb and anticipated Phase III clinical trials will support a single pivotal Phase III clinical trial for this indication. However, the FDA may require more than one pivotal Phase III clinical trial in order to grant marketing approval of Acapodene for this indication, which could delay the approval process. In addition, varying interpretations of the data obtained from preclinical and clinical testing could delay, limit or prevent regulatory approval of a product candidate. Furthermore, even if we file an application with the FDA for marketing approval of a product candidate, it may not result in marketing approval from the FDA.

We do not expect to receive regulatory approval for the commercial sale of any of our product candidates for a number of years. The inability to obtain FDA approval or approval from comparable authorities in other countries would prevent us from commercializing our product candidates in the United States or other countries. See “Government Regulation” beginning on page 53 for additional information.

Risks Related to Commercialization

The commercial success of any products that we may develop will depend upon the degree of market acceptance among physicians, patients, health care payors and the medical community.

Any products that we may develop may not gain market acceptance among physicians, patients, health care payors and the medical community. If these products do not achieve an adequate level of acceptance, we may not generate material product revenues 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;
- potential advantages over alternative treatments;
- the ability to offer our product candidates for sale at competitive prices;
- relative convenience and ease of administration;
- the strength of marketing and distribution support; and
- sufficient third-party coverage or reimbursement.

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.

We do not have a sales organization and have no experience as a company in the sales, marketing and distribution of pharmaceutical products. There are risks involved with establishing our own sales and marketing capabilities, as well as entering into arrangements with third parties to perform these services. For example, developing a sales force is expensive and time-consuming and could delay any product launch. In addition, to the extent that we enter into arrangements with third parties to perform sales, marketing and distribution services, our product revenues are likely to be lower than if we market and sell any products that we develop ourselves.

If we are unable to obtain adequate coverage and reimbursement from third-party payors for any products that we may develop or acceptable prices, our revenues and prospects for profitability will suffer.

Many patients will not be capable of paying for any products that we may develop themselves and will rely on Medicare and Medicaid, private health insurers and other third-party payors to pay for their medical needs. If third-party payors do not provide coverage or reimbursement for any products that we may develop, our revenues and prospects for profitability will suffer. In December 2003, the President signed into law legislation creating a prescription drug benefit program for Medicare recipients. The prescription drug program established by the legislation may have the effect of reducing the prices that we are able to charge for products we develop and sell through these plans. This prescription drug legislation may also cause third-party payors other than the federal government, including the states under the Medicaid program, to discontinue coverage for products we develop or to lower the amount that they pay.

State Medicaid programs generally have outpatient prescription drug coverage, subject to state regulatory restrictions, for the population eligible for Medicaid. The availability of coverage or reimbursement for prescription drugs under private health insurance and managed care plans varies based on the type of contract or plan purchased.

A primary trend in the United States health care industry is toward cost containment. In addition, in some foreign countries, particularly the countries of the European Union, the pricing of prescription pharmaceuticals is subject to governmental control. In these countries, pricing negotiations with governmental authorities can take six to 12 months or longer after the receipt of regulatory 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 candidates or products to other available therapies. The conduct of such a clinical trial could be expensive and result in delays in our commercialization. Third-party payors are challenging the prices charged for medical products and services, and many third-party payors limit reimbursement for newly-approved health care products. In particular, third-party payors may limit the indications for which they will reimburse patients who use any products that we may develop. Cost-control initiatives could decrease the price we might establish for products that we may develop, which would result in lower product revenues to us.

Another development that may affect the pricing of drugs is proposed Congressional action regarding drug reimportation into the United States. The Medicare Prescription Drug Plan legislation gives additional discretion to the Secretary of Health and Human Services to allow drug reimportation from foreign countries into the United States under some circumstances, including countries where the drugs are sold at a lower price than in the United States. Proponents of drug reimportation may attempt to pass legislation which would directly allow reimportation under certain circumstances. If legislation or regulations were passed allowing the reimportation of drugs, they could decrease the price we receive for any products that we may develop, negatively affecting our revenues and prospects for profitability.

If product liability lawsuits are brought against us, we will incur substantial liabilities and may be required 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. If we cannot successfully defend ourselves against claims that our product candidates 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;
- injury to our reputation;

- withdrawal of clinical trial participants;
- costs to defend the related litigation;
- substantial monetary awards to trial participants or patients;
- loss of revenue; and
- the inability to commercialize any products that we may develop.

We have product liability insurance that covers our clinical trials up to a $5 million annual aggregate limit. We intend to expand our insurance coverage to include the sale of commercial products if marketing approval is obtained for any products that we may develop. 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.

If our competitors are better able to develop and market products that are more effective than any products that we may develop, our commercial opportunity will be reduced or eliminated.

We face competition from established pharmaceutical and biotechnology companies, as well as from academic institutions, government agencies and private and public research institutions. Our commercial opportunity will be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer side effects or are less expensive than any products that we may develop. In addition, significant delays in the development of our product candidates could allow our competitors to bring products to market before us and impair our ability to commercialize our product candidates.

Various products are currently marketed or sold and used off-label for some of the diseases and conditions that we are targeting, and a number of companies are or may be developing new treatments. The occurrence of such off-label uses could significantly reduce our ability to market and sell any products that we may develop. For example, although there are no products that have been approved by the FDA to treat multiple side effects of advanced prostate cancer therapy, we are aware of a number of drugs marketed by Eli Lilly, Merck, Aventis, Proctor & Gamble, Wyeth Pharmaceuticals, Boehringer and Bristol Myers Squibb that are prescribed off-label to treat single side effects of this therapy and that external beam radiation is used to treat breast pain and enlargement. Similarly, while there are no drugs that have been approved by the FDA for the treatment of muscle wasting weight loss from cancer, there are drugs marketed by Steris Laboratories and Savient Pharmaceuticals that are being prescribed off-label for the treatment of some types of muscle wasting weight loss from cancer. In addition, there may be product candidates of which we are not aware at an earlier stage of development that may compete with our product candidates. If any are successfully developed and approved, they could compete directly with our product candidates. This could result in reduced sales and pricing pressure on our product candidates, if approved, which in turn would reduce our ability to generate revenue and have a negative impact on our results of operations.

Many of our competitors have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals and marketing approved products than we do. Smaller or 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 and technology licenses complementary to our programs or advantageous to our business.

Risks Related to Employees and Growth

If we fail to attract and keep senior management and key scientific personnel, we may be unable to successfully develop or commercialize our product candidates.

Our success depends on our continued ability to attract, retain and motivate highly qualified management, clinical and scientific personnel and on our ability to develop and maintain important relationships with leading academic institutions, clinicians and scientists. If we are not able to attract and keep senior management and key scientific personnel, particularly Dr. Mitchell S. Steiner, we may not be able to successfully develop or commercialize our product candidates. All of our employees are at-will employees and can terminate their employment at any time. We do not carry "key person" insurance covering members of senior management, other than $15 million of insurance covering Dr. Steiner.

We will need to hire additional employees in order to continue our clinical trials and commercialize our product candidates. Any inability to manage future growth could harm our ability to commercialize our product candidates, increase our costs and adversely impact our ability to compete effectively.

In order to continue our clinical trials and commercialize our product candidates, we will need to expand the number of our managerial, operational, financial and other employees. We currently anticipate that we will need between 150 and 250 additional employees by the time that Acapodene or Andarine is initially commercialized, including 50 to 80 sales representatives. While to date we have not experienced difficulties in recruiting and hiring qualified individuals, the competition for qualified personnel in the biotechnology field is intense.

Future growth will impose significant added responsibilities on members of management, including the need to identify, recruit, maintain and integrate additional employees. Our future financial performance and our ability to commercialize our product candidates and to compete effectively will depend, in part, on our ability to manage any future growth effectively.

Risks Related to the Offering

If you purchase our common stock in this offering, you will incur immediate and substantial dilution in the book value of your shares.

The initial public offering price is substantially higher than the net tangible book value per share of our common stock. Investors purchasing common stock in this offering will pay a price per share that substantially exceeds the book value of our tangible assets after subtracting our liabilities. As a result, investors purchasing common stock in this offering will incur immediate dilution of $10.83 per share, based on the initial public offering price of $14.50 per share. Further, investors purchasing common stock in this offering will contribute approximately 57% of the total amount invested by stockholders since our inception, but will own only approximately 22% of the shares of common stock outstanding.

This dilution is due to our investors who purchased shares prior to this offering having paid substantially less than the price offered to the public in this offering when they purchased their shares and the exercise of stock options granted to our employees. As a result of this dilution, investors purchasing stock in this offering may receive significantly less than the purchase price paid in this offering in the event of a liquidation.

Market volatility may cause our stock price and the value of your investment to decline.

Our stock price is likely to be volatile. Investors purchasing common stock in this offering may not be able to resell their shares at or above the initial public offering price. The market prices for

securities of biopharmaceutical companies in general have been highly volatile and may continue to be highly volatile in the future. The following factors, in addition to other risk factors described in this section, may have a significant impact on the market price of our common stock:

- adverse results or delays in our clinical trials;
- the timing of achievement of our clinical, regulatory and other milestones, such as the commencement of clinical development, the completion of a clinical trial or the receipt of regulatory approval;
- announcement of FDA approval or non-approval of our product candidates or delays in the FDA review process;
- actions taken by regulatory agencies with respect to our product candidates, our clinical trials or our sales and marketing activities;
- the commercial success of any product approved by the FDA or its foreign counterparts;
- regulatory developments in the United States and foreign countries;
- changes in the structure of health care payment systems;
- any intellectual property infringement lawsuit involving us;
- announcements of technological innovations or new products by us or our competitors;
- market conditions for the biotechnology or pharmaceutical industries in general;
- actual or anticipated fluctuations in our results of operation;
- changes in financial estimates or recommendations by securities analysts;
- sales of large blocks of our common stock;
- sales of our common stock by our executive officers, directors and significant stockholders;
- changes in accounting principles; and
- the loss of any of our key scientific or management personnel.

The stock markets in general, and the markets for biotechnology stocks in particular, have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. These broad market fluctuations may adversely affect the trading price of our common stock. In the past, class action litigation has often been instituted against companies whose securities have experienced periods of volatility in market price. Any such litigation brought against us could result in substantial costs, which would hurt our financial condition and results of operations and divert management's attention and resources, which could result in delays of our clinical trials or commercialization efforts.

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

Based on our outstanding shares as of December 31, 2003, after this offering, our officers, directors and holders of 5% or more of our outstanding common stock will beneficially own approximately 78% of our common stock, after giving effect to the conversion into common stock of all outstanding shares of our preferred stock and dividends accrued thereon through December 31, 2003, but assuming no exercise of the underwriters' over-allotment option and no exercise of outstanding options. As a result, these stockholders, acting together, will be able to control all matters requiring approval by our stockholders, including the election of directors and the approval of mergers or other business combination transactions. The interests of this group of stockholders may not always coincide with our interests or the interests of other stockholders.

Anti-takeover provisions in our 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 certificate of incorporation and our bylaws that will become effective upon the completion of this offering may delay or prevent an acquisition of us or a change in our management. 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. These provisions include:

- a classified Board of Directors;
- a prohibition on actions by our stockholders by written consent;
- the ability of 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
- limitations on the removal of directors.

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. Finally, these provisions establish advance notice requirements for nominations for election to our Board of Directors or for proposing matters that can be acted upon at stockholder meetings. These provisions would apply even if the offer may be considered beneficial by some stockholders.

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 necessarily 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.

A significant portion of our total outstanding shares are 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 intend to sell shares, could reduce the market price of our common stock. After this offering, we will have outstanding 24,592,753 shares of common stock based on the number of shares outstanding as of December 31, 2003. This includes the shares that we are selling in this offering, which may be resold in the public market immediately. The remaining 19,192,753 shares, or 78% of our outstanding shares after this offering, are currently restricted as a result of securities laws or lock-up agreements but will be able to be sold in the near future as set forth below.

Number of Shares and % of Total Outstanding	Date Available for Sale Into Public Market
16,302,695 shares, or 66%	180 days after the date of this prospectus due to lock-up agreements between the holders of these shares and the underwriters. However, the underwriters can waive the provisions of these lock-up agreements and allow these stockholders to sell their shares at any time.
2,890,058 shares, or 12%	Between 180 and 365 days after the date of this prospectus, depending on the requirements of the federal securities laws.

Moreover, after this offering, J.R. Hyde, III, Oracle Partners, L.P. and Memphis Biomed Ventures I, L.P., three of our largest stockholders, and their affiliates, who held in the aggregate 11,344,619 shares of common stock as of December 31, 2003, will have rights, subject to some conditions, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders. We also intend to register all shares of common stock that we may issue under our employee benefit plans. Once we register these shares, they can be freely sold in the public market upon issuance, subject to the lock-up agreements described in “Underwriting.” For additional information, see “Shares Eligible for Future Sale” on page 79.

SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This prospectus contains forward-looking statements. The forward-looking statements are contained principally in the sections entitled “Prospectus Summary,” “Risk Factors,” “Use of Proceeds,” “Management’s Discussion and Analysis of Financial Condition and Results of Operations” and “Business.” These statements involve known and unknown risks, uncertainties and other factors which may cause our actual results, performance or achievements to be materially different from any future results, performances or achievements expressed or implied by the forward-looking statements. Forward-looking statements include statements about:

- the anticipated progress of our research, development and clinical programs;
- our ability to market, commercialize and achieve market acceptance for our product candidates or products that we may develop;
- our ability to generate additional product candidates for clinical testing;
- our ability to protect our intellectual property and operate our business without infringing upon the intellectual property rights of others; and
- our estimates regarding the sufficiency of our cash resources.

In some cases, you can identify forward-looking statements by terms such as “anticipates,” “believes,” “could,” “estimates,” “expects,” “intends,” “may,” “plans,” “potential,” “predicts,” “projects,” “should,” “will,” “would,” and similar expressions intended to identify forward-looking statements. Forward-looking statements reflect our current views with respect to future events, are based on assumptions and subject to risks and uncertainties. We discuss many of these risks in this prospectus in greater detail under the heading “Risk Factors.” Given these uncertainties, you should not place undue reliance on these forward-looking statements. Also, forward-looking statements represent our estimates and assumptions only as of the date of this prospectus. 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.

Except as required by law, we assume no obligation to update any forward-looking statements publicly, or to update the reasons actual results could differ materially from those anticipated in any forward-looking statements, even if new information becomes available in the future.

USE OF PROCEEDS

We estimate that our net proceeds from the sale of 5,400,000 shares of common stock in this offering will be approximately $70.5 million and $81.4 million if the underwriters exercise their over-allotment option in full, based upon the initial public offering price of $14.50 per share and after deducting underwriting discounts and commissions and estimated offering expenses payable by us. The principal purposes of this offering are to obtain additional capital and to create a public market for our common stock.

We expect to use approximately $52.2 million of the net proceeds from this offering to fund our clinical trials and other research and development activities and the remainder for general corporate purposes. In addition, we may use a portion of the net proceeds from this offering to acquire equipment, products, technologies or businesses, although we currently have no commitments or agreements relating to any of these types of transactions. We believe that the net proceeds from this offering, our existing cash resources and interest on these funds will be sufficient to meet our projected operating requirements through the end of 2005.

While we have estimated the particular uses for the net proceeds to be received upon the completion of this offering, we cannot specify these uses with certainty. Accordingly, our management will have broad discretion in the application of the net proceeds, and investors will be relying on the judgment of our management regarding the application of the proceeds of this offering. Pending these uses, we plan to invest the net proceeds in short-term, interest bearing obligations, investment grade instruments, certificates of deposit or direct or guaranteed obligations of the United States. The goal with respect to the investment of these net proceeds is capital preservation and liquidity so that such funds are readily available to fund our research and development operations.

DIVIDEND POLICY

The payment of accrued dividends on our outstanding preferred stock will result in the issuance of additional shares of our common stock upon completion of this offering. As of December 31, 2003, approximately $6.8 million of dividends had accrued on our preferred stock. If our preferred stock had been converted into common stock on December 31, 2003, we would have issued 966,757 shares of our common stock in satisfaction of our accrued dividend obligations. We expect to issue approximately 53,500 shares of common stock in satisfaction of our accrued dividend obligations for the month of January 2004.

We have never declared or paid any cash dividends on our capital stock. We currently intend to retain any future earnings to fund the development and expansion of our business, and therefore we do not anticipate paying cash dividends on our common stock in the foreseeable future. Any future determination to pay dividends will be at the discretion of our Board of Directors.

CAPITALIZATION

The following table sets forth our capitalization as of September 30, 2003:

- on an actual basis; and
- on an as adjusted basis to reflect the conversion into common stock of all outstanding shares of preferred stock and dividends accrued thereon through September 30, 2003 and the sale in this offering of 5,400,000 shares of common stock at the initial public offering price of $14.50 per share, after deducting the underwriting discounts and commissions and estimated offering expenses payable by us.

The information in this table is based on shares outstanding as of September 30, 2003 and excludes:

- 799,000 shares of common stock issuable upon exercise of options issued under our current stock option plans, at a weighted average exercise price of $5.97 per share; and
- 483,650 shares of common stock reserved for future issuance under our current stock option plans and 1,700,000 shares of common stock reserved for future issuance under our 2004 Equity Incentive Plan and 2004 Non-Employee Directors' Stock Option Plan, which will become effective upon the completion of this offering.

You should read the information below in conjunction with the financial statements and the related notes thereto and "Management's Discussion and Analysis of Financial Condition and Results of Operations" included elsewhere in this prospectus.

	As of September 30, 2003	
	Actual	As Adjusted
	(unaudited) (in thousands, except share data)	
Cash and cash equivalents	$ 19,788	$ 90,307
Cumulative redeemable convertible preferred stock, $0.001 par value:		
1,975,000 shares authorized and 1,231,955 shares issued and outstanding, actual; and no shares authorized, issued or outstanding, as adjusted	162,978	—
Stockholders' (deficit) equity:		
Common stock, par value $0.001 per share:		
25,000,000 shares authorized and 7,735,000 shares issued and outstanding, actual; and 60,000,000 shares authorized and 24,431,780 shares issued and outstanding, as adjusted	8	24
Deferred stock compensation	(3,408)	(3,408)
Additional paid-in capital	4,453	238,204
Deficit accumulated during the development stage	(144,891)	(145,160)
Total stockholders' (deficit) equity	(143,838)	89,660
Total capitalization	$ 19,140	$ 89,660

DILUTION

Our net tangible book value as of September 30, 2003 was $19.1 million, or $1.01 per share of common stock, assuming the conversion of all of our preferred stock and dividends accrued thereon through September 30, 2003 into common stock and giving effect to the 8.5-for-1 stock split of our common stock effected on January 14, 2004. Net tangible book value per share represents the amount of our total tangible assets less total liabilities, divided by the total number of shares of common stock outstanding after giving effect to the conversion of all of our preferred stock and dividends accrued thereon through September 30, 2003 into common stock and the 8.5-for-1 stock split effected on January 14, 2004.

After giving effect to the sale of 5,400,000 shares of common stock offered in this offering at the initial public offering price of $14.50 per share and after deducting the underwriting discounts and commissions and estimated offering expenses payable by us, our as adjusted net tangible book value as of September 30, 2003 would have been $89.7 million, or $3.67 per share of common stock. This represents an immediate increase in net tangible book value of $2.66 per share to existing stockholders and an immediate dilution of $10.83 per share to new investors purchasing our common stock in this offering. The following table illustrates this per share dilution to the new investors:

Initial public offering price		$14.50
Net tangible book value per share as of September 30, 2003	$1.01	
Increase in net tangible book value per share attributable to this offering	2.66	
As adjusted net tangible book value per share after this offering		3.67
Dilution per share to new investors in this offering		$10.83

If the underwriters exercise their option to purchase additional shares of our common stock in full in this offering, the as adjusted net tangible book value per share after this offering would be $3.98 per share, the increase in net tangible book value per share to existing stockholders would be $2.97 per share and the dilution to new investors purchasing shares in this offering would be $10.52 per share.

The following table summarizes, on an as adjusted basis as of September 30, 2003, the differences between the number of shares of common stock purchased from us (assuming conversion of all of our preferred stock and dividends accrued thereon through September 30, 2003 into common stock and giving effect to the 8.5-for-1 stock split effected on January 14, 2004), the total consideration and the average price per share paid by existing stockholders and by the new investors, based on the initial public offering price of $14.50 per share, before deducting underwriting discounts and commissions and estimated offering expenses payable by us:

	Shares Purchased		Total Consideration		Average Price Per Share
	Number	Percent	Amount	Percent	
Existing stockholders	19,031,780	78%	$ 59,042,536	43%	$ 3.10
New investors	5,400,000	22	78,300,000	57	14.50
Total	24,431,780	100	$137,342,536	100	

If the underwriters exercise their option to purchase additional shares of our common stock in full in this offering, the number of shares held by new investors will increase to 6,210,000, or 25% of the total number of shares of our common stock outstanding after this offering.

The existing stockholders amounts in the table above have been calculated based on shares outstanding as of September 30, 2003 and exclude:

- 799,000 shares of common stock issuable upon exercise of options issued under our current stock option plans, at a weighted average exercise price of $5.97 per share; and

- 483,650 shares of common stock reserved for future issuance under our current stock option plans and 1,700,000 shares of common stock reserved for future issuance under our 2004 Equity Incentive Plan and 2004 Non-Employee Directors' Stock Option Plan, which will become effective upon the completion of this offering.

After this offering and assuming the exercise in full of all outstanding options, our as adjusted net tangible book value per share as of September 30, 2003 would be $3.74 per share, representing an immediate increase in net tangible book value of $2.73 per share to existing stockholders and an immediate dilution in net tangible book value of $10.76 per share to new investors.

SELECTED FINANCIAL DATA

You should read the selected financial data below in conjunction with "Management's Discussion and Analysis of Financial Condition and Results of Operations" and the financial statements, notes thereto and other financial information included elsewhere in this prospectus. We derived the selected financial data for each of the five fiscal years in the period ended December 31, 2002 from our financial statements which have been examined and reported upon by Ernst & Young LLP, independent public accountants. See "Experts." We derived the data presented for the nine-month periods ended September 30, 2002 and September 30, 2003 from unaudited financial statements which include, in the opinion of management, all adjustments, consisting only of normal recurring accruals, necessary to present fairly the data for such periods. The results for the nine-month period ended September 30, 2003 are not necessarily indicative of the results to be expected for the full fiscal year.

We computed pro forma net loss per share for the year ended December 31, 2002 and the nine months ended September 30, 2003 using the weighted average number of shares of common stock outstanding, including the pro forma effects of the automatic conversion of our preferred stock and dividends accrued thereon into shares of common stock effective upon the closing of the offering as if such conversion occurred on January 1, 2002 and January 1, 2003 or at the date of the original issuance, if later. The calculation of pro forma net loss per share attributable to common stockholders excludes incremental common stock issuable upon exercise of options, as its effect would be antidilutive.

	Year Ended December 31,					Nine Months Ended September 30,	
	1998	1999	2000	2001	2002	2002	2003
	(in thousands, except share and per share data)					(unaudited)	
Statement of Operations Data:							
Operating expenses:							
Research and development	$ 185	$ 518	$ 2,679	$ 5,744	$ 9,285	$ 6,408	$ 7,123
General and administrative	179	256	1,203	2,187	2,405	1,830	2,339
Depreciation	19	45	80	215	332	240	264
Total operating expenses	383	819	3,962	8,146	12,022	8,478	9,726
Other income:							
Research and development income	225	—	—	—	—	—	—
Interest income	42	69	150	83	156	105	79
Total other income	267	69	150	83	156	105	79
Net loss	(116)	(750)	(3,812)	(8,063)	(11,866)	(8,373)	(9,647)
Accrued preferred stock dividends	—	(83)	(297)	(790)	(2,147)	(1,466)	(2,300)
Adjustment to preferred stock redemption value	—	—	(21,077)	(57)	(7,220)	(7,147)	(76,666)
Net loss attributable to common stockholders	$ (116)	$ (833)	$ (25,186)	$ (8,910)	$ (21,233)	$ (16,986)	$ (88,613)
Net loss per share attributable to common stockholders, basic and diluted:	$ (.01)	$ (.11)	$ (3.26)	$ (1.15)	$ (2.75)	$ (2.20)	$ (11.46)
Weighted average shares used in computing net loss per share attributable to common stockholders, basic and diluted:	7,735,000	7,735,000	7,735,000	7,735,000	7,735,000	7,735,000	7,735,000
Pro forma net loss per share attributable to common stockholders — basic and diluted					$ (0.80)		$ (0.59)
Shares used in computing pro forma net loss per share attributable to common					14,811,786		16,455,728

stockholders — basic and diluted		

	As of December 31,					As of September 30, 2003
	1998	1999	2000	2001	2002	(unaudited)
				(in thousands)		
Balance Sheet Data:						
Cash and cash equivalents	$ 748	$1,542	$ 2,667	$ 8,834	$ 8,925	$ 19,788
Working capital	743	1,435	2,241	8,544	7,654	18,280
Total assets	870	1,678	3,201	10,117	10,030	21,107
Cumulative redeemable convertible preferred stock	—	1,538	27,912	43,702	64,026	162,978
Deficit accumulated during development stage	(116)	(949)	(26,135)	(35,045)	(56,278)	(144,891)
Total stockholders' (deficit) equity	854	21	(25,165)	(34,075)	(55,308)	(143,838)

MANAGEMENT'S DISCUSSION AND ANALYSIS OF

FINANCIAL CONDITION AND RESULTS OF OPERATIONS

The following discussion and analysis should be read in conjunction with our financial statements and related notes included elsewhere in this prospectus. This discussion may contain forward-looking statements based upon current expectations that involve risks and uncertainties. Our actual results and the timing of selected events could differ materially from those anticipated in these forward-looking statements as a result of several factors, including those set forth under "Risk Factors" and elsewhere in this prospectus.

GTx is a biopharmaceutical company dedicated to the discovery, development and commercialization of therapeutics primarily related to the treatment of serious men's health conditions. Our drug discovery and development programs are focused on small molecules that selectively modulate the effects of estrogens and androgens, two essential classes of hormones. We currently have two product candidates that are in human clinical trials. We are developing Acapodene, our most advanced product candidate, through clinical trials for two separate indications: (1) a Phase IIb clinical trial for the reduction in the incidence of prostate cancer in men with precancerous prostate lesions known as high grade prostatic intraepithelial neoplasia, or high grade PIN, and (2) a pivotal Phase III clinical trial for the treatment of serious side effects of advanced prostate cancer therapy known as androgen deprivation therapy. We are initially developing our second product candidate, Andarine, for the treatment of cachexia from various types of cancer. Cancer cachexia is a muscle wasting condition that is a potentially life-threatening complication of many cancers. Andarine is the most advanced of our internally discovered portfolio of compounds designed to modulate the effects of hormones. We plan to build a specialized sales and marketing capability to market our product candidates directly to the relatively small and concentrated community of urologists and medical oncologists in the United States and seek collaborators to commercialize our product candidates where the target physician market is broader than urologists and medical oncologists and outside the United States.

To date, we have not generated any product revenue, and we have financed our operations and internal growth almost exclusively through private placements of preferred stock. We are a development stage company and have incurred significant losses since our inception in 1997 as we have devoted substantially all of our resources to research and development, including our clinical trials. As of September 30, 2003, we had a deficit accumulated during the development stage of $144.9 million. Our accumulated deficit resulted primarily from:

- our research and development activities associated with Acapodene for the reduction in the incidence of prostate cancer in men with high grade PIN, including our Phase IIb clinical trial; Acapodene for the treatment of side effects of androgen deprivation therapy, including two Phase II clinical trials; Andarine for the treatment of cachexia from various forms of cancer, including three Phase I clinical trials; and other product candidates;

- general and administrative expenses; and

- non-cash dividends and adjustments to the preferred stock redemption value of $110.6 million related to our cumulative redeemable convertible preferred stock. See "Critical Accounting Policies — Adjustment to Preferred Stock Redemption Value."

We expect to continue to incur net losses over the next several years as we continue our clinical development and research and development activities, apply for regulatory approvals, establish sales and marketing capabilities and expand our operations.

Research and Development

Since our inception, we have been focused on drug discovery and development programs. Research and development expenses represented approximately 73% of our total operating expenses for the nine-month period ended September 30, 2003 and 76% of our total operating expenses for the nine-month period ended September 30, 2002. Research and development expenses include our expenses for:

- personnel associated with our research activities;
- screening and identification of product candidates;
- formulation and synthesis activities;
- manufacturing;
- preclinical studies, including toxicology studies;
- clinical trials;
- regulatory affairs; and
- quality assurance activities.

The following table identifies for each of our major drug discovery and development programs our lead product candidates, the development phase of each lead product candidate, the status of each lead product candidate and research and development spending for each lead product candidate for each of the periods presented. Research and development spending for past periods is not indicative of spending in future periods.

Research & Development Spending

Program/ Product Candidate/ Indication	Development Phase	Status	Year Ended December 31,			Nine Months Ended September 30,		Inception Through September 30,
			2000	2001	2002	2002	2003	2003
			(in thousands)					
SERM Program								
Acapodene								
• Reduction in the incidence of prostate cancer in men with high grade PIN	Phase IIb clinical trial	Enrollment complete; last patient scheduled to complete trial in May 2004; final results expected in the third quarter of 2004	$1,984	$2,436	$3,168	$2,015	$2,271	$10,562
• Side effects of androgen deprivation therapy	Pivotal Phase III clinical trial	Pivotal Phase III clinical trial initiated in November 2003	$ —	$ —	$ 807	$ 606	$ 661	$ 1,468

Program/ Product Candidate/ Indication	Development Phase	Status	Year Ended December 31, 2000	Year Ended December 31, 2001	Year Ended December 31, 2002	Nine Months Ended September 30, 2002	Nine Months Ended September 30, 2003	Inception Through September 30, 2003
						(in thousands)		
SARM Program								
Andarine								
Cachexia from various types of cancer	Three Phase I clinical trials completed	Phase II clinical trials for treatment of cachexia from non-small cell lung cancer scheduled to begin in the first half of 2004	$ 141	$2,430	$4,134	$2,811	$3,489	$10,194
Other product candidates	Preclinical		$ 554	$ 878	$1,176	$ 976	$ 702	$ 3,310
Total research and development spending			$2,679	$5,744	$9,285	$6,408	$7,123	$25,534

There is a risk that any drug discovery and development program may not produce revenue. Moreover, because of uncertainties inherent in drug discovery and development, including those factors described in the “Risk Factors” section of this prospectus, we may not be able to successfully develop and commercialize any of the product candidates included in the table above.

Drug development in the United States is a process that includes several steps defined by the FDA. The FDA approval process for a new drug involves completion of preclinical studies and the submission of the results of these studies to the FDA, together with proposed clinical protocols, manufacturing information, analytical data and other information in an Investigational New Drug application, or IND, which must become effective before human clinical trials may begin. Clinical development typically involves three phases of study: Phase I, II and III. The most significant costs associated with clinical development are the Phase III clinical trials as they tend to be the longest and largest studies conducted during the drug development process. After completion of clinical trials, a New Drug application, or NDA, may be submitted to the FDA. In responding to an NDA, the FDA may refuse to file the application, or if accepted for filing, the FDA may grant marketing approval, request additional information or deny the application if it determines that the application does not provide an adequate basis for approval.

The successful development of our product candidates is highly uncertain. We cannot reasonably estimate or know the nature, timing and estimated costs of the efforts necessary to complete the development of, or the period in which material net cash inflows are expected to commence from, any of our product candidates due to the numerous risks and uncertainties associated with developing drugs, including the uncertainty of:

- the scope, rate of progress and cost of our clinical trials and other research and development activities;
- future clinical trial results;
- the terms and timing of any collaborative, licensing and other arrangements that we may establish;
- the cost and timing of regulatory approvals;
- the cost and timing of establishing sales, marketing and distribution capabilities;

- the cost of establishing clinical and commercial supplies of our product candidates and any products that we may develop;
- the effect of competing technological and market developments; and
- the cost of filing, prosecuting, defending and enforcing any patent claims and other intellectual property rights.

Any failure to complete the development of our product candidates in a timely manner could have a material adverse effect on our operations, financial position and liquidity. A discussion of the risks and uncertainties associated with completing our projects on schedule, or at all, and some consequences of failing to do so, are set forth in the "Risk Factors" section of this prospectus.

Results of Operations

Comparison of Nine Months Ended September 30, 2003 and September 30, 2002

Research and Development. Research and development expenses increased 11.2% to $7.1 million for the nine months ended September 30, 2003 from $6.4 million for the nine months ended September 30, 2002. The increase was due primarily to an increase in research and development expenses for Andarine of approximately $677,000 of Phase I clinical trial expenses. Research and development expenses also increased due to an increase in clinical trial expenses for the Phase IIb clinical trial of Acapodene for the reduction in the incidence of prostate cancer in men with high grade PIN of approximately $256,000, as enrollment in the clinical trial was completed in May 2003. These increases were offset in part by a reduction in research and development spending on other product candidates of approximately $274,000.

We expect that research and development expenditures will continue to increase substantially during 2003 and subsequent years due to (1) the commencement in November 2003 of a pivotal Phase III clinical trial of Acapodene for the treatment of side effects of androgen deprivation therapy, (2) the completion of the current Phase IIb clinical trial in 2004 and planned commencement of a pivotal Phase III clinical trial of Acapodene for the reduction in the incidence of prostate cancer in men with high grade PIN and (3) the continued development of Andarine, including a Phase II clinical trial scheduled to begin in the first half of 2004. We expect to expand the scope of our drug discovery and development programs in future periods, which may result in substantial increases in research and development expenses.

General and Administrative. General and administrative expenses consist primarily of the costs of administrative personnel and related facilities and legal, accounting, human resources, information technology, public relations and other professional services. In the future, general and administrative expenses will also include the costs of sales and marketing. General and administrative costs increased 27.8% to $2.3 million for the nine months ended September 30, 2003 from $1.8 million for the nine months ended September 30, 2002. The increase was primarily due to an increase in salary and benefits expense of approximately $241,000 resulting from increases in staffing levels, annual salary increases and increased health insurance costs and an approximate $98,000 increase in professional fees. The increase in general and administrative expenses for the nine months ended September 30, 2003 included amortization of stock-based compensation expense of $83,000. In September 2003, in anticipation of this offering, we recorded deferred stock-based compensation expense of $3.5 million. The expense will be amortized over the service period, which is generally five years.

We expect that general and administrative expenditures will increase during 2003 and subsequent years due to increasing payroll, public company expenses, our initial commercialization expenses, business development costs and expanded operational infrastructure.

Interest Income. Interest income for the nine months ended September 30, 2003 was $79,000 and decreased from the corresponding period in 2002 as a result of a decrease in the average cash and cash equivalents balance and overall interest rates.

Adjustment to Preferred Stock Redemption Value. The adjustment to preferred stock redemption value consists of the amount of the change in the redemption value, which is the greater of the liquidation value or fair value, of the preferred stock. The adjustment for the nine months ended September 30, 2003 was an increase of $76.7 million, or $56.07 per share, as compared to an increase of $7.1 million, or $9.10 per share, for the nine months ended September 30, 2002. The per share redemption value was $57.66 as of December 31, 2001, $66.76 as of September 30, 2002 and December 31, 2002 and $122.83 as of September 30, 2003. The increases in the redemption value for the nine months ended September 30, 2003 and 2002 were the result of the achievement of significant milestones in clinical trials and general market conditions and, for the nine months ended September 30, 2003, was made in connection with this offering. See “Critical Accounting Policies Adjustment to Preferred Stock Redemption Value.”

Comparison of Years Ended December 31, 2002 and December 31, 2001

Research and Development. Research and development expenses increased 61.6% to $9.3 million for the year ended December 31, 2002 from $5.7 million for the year ended December 31, 2001. This increase was primarily due to an increase in clinical trial expenses for the Phase IIb clinical trial of Acapodene for the reduction in the incidence of prostate cancer in men with high grade PIN of approximately $732,000 and an increase in clinical trial expenses for two Phase II clinical trials of Acapodene for the treatment of side effects of androgen deprivation therapy of approximately $807,000. In addition, preclinical toxicology studies, formulation and synthesis activities, manufacturing activities and clinical development activities for Andarine increased research and development expenses by approximately $1.7 million. Research and development expenses related to other product candidates increased by approximately $297,000 for the year ended December 31, 2002 as compared to the prior year.

General and Administrative. General and administrative expenses increased 10% to $2.4 million for the year ended December 31, 2002 from $2.2 million for the year ended December 31, 2001. This increase was primarily due to an increase in salary and benefits expense by approximately $419,000 associated with increases in staffing levels, offset by a reduction in legal fees of approximately $72,000 and travel expenses of approximately $40,000. In addition, general and administrative expenses for the year ended December 31, 2001 included interest expense on notes payable of approximately $71,000. There were no notes outstanding in the year ended December 31, 2002.

Interest Income. Interest income increased 88% to approximately $156,000 for the year ended December 31, 2002 from approximately $83,000 for the year ended December 31, 2001. The increase was principally attributable to higher average cash and cash equivalents balances during the year ended December 31, 2002 as compared to the prior year.

Adjustment to Preferred Stock Redemption Value. The adjustment for the year ended December 31, 2002 was an increase of $7.2 million, or $9.10 per share, as compared to an increase of $57,000 for the year ended December 31, 2001. The per share redemption value was $57.66 as of December 31, 2000 and 2001 and $66.76 as of December 31, 2002. The increase in the redemption value for the year ended December 31, 2002 was the result of the achievement of significant milestones in clinical trials and general market conditions.

Comparison of Years Ended December 31, 2001 and December 31, 2000

Research and Development. Research and development expenses increased 114.4% to $5.7 million for the year ended December 31, 2001 from $2.7 million for the year ended December 31, 2000. This increase was primarily due to an increase in research and development

expenses for Andarine of approximately $2.3 million, which included preclinical toxicology studies, formulation and synthesis activities, manufacturing activities and clinical development activities, and an increase in clinical trial expenses for the Phase IIb clinical trial of Acapodene for the reduction in the incidence of prostate cancer in men with high grade PIN of approximately $450,000. Research and development expenses on other product candidates increased by approximately $325,000 for the year ended December 31, 2001 compared to the prior year.

General and Administrative. General and administrative expenses increased 81.8% to $2.2 million for the year ended December 31, 2001 from $1.2 million for the year ended December 31, 2000. This increase was primarily due to an increase in salary and benefits expense by approximately $460,000 associated with increases in staffing levels, an increase of occupancy expense of approximately $105,000, an increase in legal fees of approximately $112,000, as well as increases in other general and administrative expenses. In addition, general and administrative expenses for the year ended December 31, 2001 included interest expense on notes payable of approximately $71,000. There were no notes outstanding in the year ended December 31, 2000.

Interest Income. Interest income decreased 44.7% to $83,000 for the year ended December 31, 2001 from $150,000 for the year ended December 31, 2000. The decrease was principally attributable to lower average cash and cash equivalents balances during the year ended December 31, 2001 as compared to the prior year.

Adjustment to Preferred Stock Redemption Value. The adjustment for the year ended December 31, 2001 was an increase of $57,000, as compared to an increase of $21.1 million, or $50.38 per share, for the year ended December 31, 2000. The per share redemption value was $7.28 as of December 31, 1999 and $57.66 as of December 31, 2000 and 2001. The increase in the redemption value for the year ended December 31, 2000 was the result of the achievement of significant milestones in clinical trials and general market conditions.

Liquidity and Capital Resources

We have not generated any product revenue, and we have financed our operations and internal growth almost exclusively through private placements of preferred stock. We have incurred significant losses since our inception in 1997. As of September 30, 2003, we had a deficit accumulated during the development stage of $144.9 million, of which $110.6 million related to non-cash dividends and adjustments to the preferred stock redemption value.

The following table summarizes our issuances of preferred stock through September 30, 2003:

Series	Date	Number of Shares	Approximate Gross Proceeds
			(in thousands)
A	May 1999	200,000	$ 1,455
B	July 2000	277,500	5,000
C	October 2001	260,154	15,000
D	July 2002	164,765	11,000
E	August 2003	329,536	20,000

At September 30, 2003, we had cash and cash equivalents of $19.8 million, compared to $8.9 million at December 31, 2002, $8.8 million at December 31, 2001 and $2.7 million at December 31, 2000.

Net cash used in operating activities was $10.6 million for the year ended December 31, 2002 and $9.1 million for the nine months ended September 30, 2003. The use of cash in both periods resulted primarily from funding our net losses.

Net cash used in investing activities was $313,000 for the year ended December 31, 2002 and $60,000 for the nine months ended September 30, 2003, primarily for the purchase of research and development equipment.

Net cash provided by financing activities, which resulted from the sale of preferred stock, was $11.0 million for the year ended December 31, 2002 and $20.0 million for the nine months ended September 30, 2003.

We believe that the net proceeds from this offering, our current cash resources and interest on these funds will be sufficient to meet our projected operating requirements through the end of 2005. In addition to the net proceeds of this offering, we estimate that we will need to raise additional funds in the amount of approximately $70 million, assuming that we do not enter into collaborative arrangements for any of our product candidates, in order to complete development of Acapodene and Andarine for the indications currently being tested.

Our forecast of the period of time through which our financial resources will be adequate to support our projected operating requirements is a forward-looking statement and involves risks and uncertainties, and actual results could vary as a result of a number of factors, including the factors discussed in the “Risk Factors” section of this prospectus. We have based this estimate on assumptions that may prove to be wrong, and we could utilize our available capital resources sooner than we currently expect. Because of the numerous risks and uncertainties associated with the development of our product candidates and other research and development activities, including risks and uncertainties that could impact the rate of progress of our development activities, we are unable to estimate with certainty the amounts of increased capital outlays and operating expenditures associated with our current and anticipated clinical trials and other research and development activities. Our future funding requirements will depend on many factors, including:

- the scope, rate of progress and cost of our clinical trials and other research and development activities;
- future clinical trial results;
- the terms and timing of any collaborative, licensing and other arrangements that we may establish;
- the cost and timing of regulatory approvals;
- the cost and timing of establishing sales, marketing and distribution capabilities;
- the cost of establishing clinical and commercial supplies of our product candidates and any products that we may develop;
- the effect of competing technological and market developments;
- the cost of filing, prosecuting, defending and enforcing any patent claims and other intellectual property rights; and
- the extent to which we acquire or invest in businesses, products and technologies, although we currently have no commitments or agreements relating to any of these types of transactions.

We do not anticipate that we will generate product revenue for a number of years. Until we can generate a sufficient amount of product revenue, if ever, we expect to finance future cash needs through public or private equity offerings, debt financings or corporate collaboration and licensing arrangements, as well as through interest income earned on cash balances. We do not currently have any commitments for future external funding. We cannot be certain that additional funding will be available on acceptable terms, or at all. To the extent that we raise additional funds by issuing equity securities, our stockholders may experience dilution, and debt financing, if available, may involve restrictive covenants. To the extent that we raise additional funds through collaboration and licensing arrangements, it may be necessary to relinquish some rights to our technologies or product candidates, or grant licenses on terms that are not favorable to us. If adequate funds are not available, we may be required to delay, reduce the scope of or eliminate one or more of our research or development programs or to obtain funds through collaborations with others that are on

unfavorable terms or that may require us to relinquish rights to some of our technologies or product candidates that we would otherwise seek to develop on our own.

We have no long-term debt, and, as of September 30, 2003, we had contractual obligations related to a facilities lease as follows:

	Payments Due by Period (in thousands)				
	Total	Less than 1 year	1-3 years	4-5 years	After 5 years
Contractual obligations	$404	$202	$202	$ —	$ —

Our long-term commitments under the operating lease shown above consist of payments relating to a lease for laboratory and office space at 3 North Dunlap Street, Memphis, Tennessee. This lease expires on September 30, 2005. This lease is terminable by either party on 90 days' notice. The table above excludes contingent payments under the license agreements to which we are a party.

Critical Accounting Policies

The preparation of financial statements in conformity with accounting principles generally accepted in the United States requires management to make estimates and assumptions that affect the reported amounts of assets, liabilities, revenues, expenses and related disclosures. Actual results could differ from those estimates. We believe that the following critical accounting policies affect the more significant judgments and estimates used in the preparation of our financial statements.

Accounting for Income Taxes

Our income tax policy records the estimated future tax effects of temporary differences between the tax basis of assets and liabilities and amounts reported in the accompanying balance sheets, as well as operating loss and tax credit carryforwards. We have recorded a full valuation allowance to reduce our deferred tax assets as, based on available objective evidence, it is more likely than not that the deferred tax asset will not be realized. In the event that we determine that we will be able to realize our deferred tax assets in the future, an adjustment to the valuation allowance would increase net income in the period such determination is made.

Stock-Based Compensation

In accordance with Accounting Principles Board Opinion No. 25 and related interpretations, we do not recognize compensation expense when we issue stock options to employees and non-employee directors, unless the exercise price is below the fair market value of the stock on the date of grant. In anticipation of this offering, we determined that, for financial reporting purposes, the estimated value of our common stock was in excess of the exercise price for stock options issued to employees subsequent to June 30, 2003. Accordingly, we recorded deferred stock-based compensation and are amortizing the related expense over the service period, which is generally five years. Our compensation expense would have been approximately $115,000 higher and our diluted net loss per share attributable to common stockholders would have been approximately $0.01 higher in 2002 had we recognized an expense equal to the estimated fair market value of employee stock options granted through December 31, 2002 amortized over the vesting period of the options. For more information on this subject, you should refer to Note 11 to our financial statements included elsewhere in this prospectus.

Adjustment to Preferred Stock Redemption Value

We recognize changes in the redemption value of our preferred stock immediately as they occur and adjust the carrying value of the preferred stock to equal the redemption value at the end

of each reporting period. The preferred stock is subject to redemption on or after August 31, 2006 at a price per share equal to the greater of (1) the liquidation value, which includes accrued dividends or (2) the fair value calculated on an as-if converted to common stock basis. We determine fair value considering factors such as the share price of preferred stock issuances, achievement of significant milestones in the clinical trials and general market conditions. Although we consider these factors in determining fair value, this determination is, by its nature, subjective and subject to change in the future based upon a number of factors. The changes in redemption value affect the loss attributable to common stockholders, the preferred stock carrying values and the deficit accumulated during the development stage.

Quantitative and Qualitative Disclosures about Market Risk

Our exposure to market risk for changes in interest rates relates to our cash equivalents on deposit in highly liquid money market funds. The primary objective of our cash investment activities is to preserve principal while at the same time maximizing the income we receive from our invested cash without significantly increasing risk of loss. We do not use derivative financial instruments in our investment portfolio. Our cash and investments policy emphasizes liquidity and preservation of principal over other portfolio considerations.

We have operated primarily in the United States. Accordingly, we do not have any material exposure to foreign currency rate fluctuations. However, if we are successful in our efforts to commercialize Acapodene, our exposure to foreign currency rate fluctuations may increase because we are obligated to pay Orion in Euros.

Recent Accounting Pronouncements

In December 2002, the Financial Accounting Standards Board, or FASB, issued SFAS No. 148 “Accounting for Stock-Based Compensation — Transition and Disclosure,” which provides alternative methods of transition for a voluntary change to the fair value based method of accounting for stock-based employee compensation. SFAS No. 148 also requires that disclosures of the pro forma effect of using the fair value method of accounting for stock-based employee compensation be displayed more prominently and in a tabular format. Additionally, SFAS No. 148 requires disclosure of the pro forma effect in interim financial statements. The transition and annual disclosure requirements of SFAS No. 148 are effective for fiscal years ended after December 15, 2002. The interim disclosure requirements are effective for interim periods beginning after December 15, 2002. The adoption of this standard did not have a material impact on our financial statements.

In January 2003, the FASB issued FASB Interpretation No. 46, “Consolidation of Variable Interest Entities, an Interpretation of ARB No. 51.” FIN 46 requires certain variable interest entities to be consolidated by the primary beneficiary of the entity if the equity investors in the entity do not have the characteristics of a controlling financial interest or do not have sufficient equity at risk for the entity to finance its activities without additional subordinated financial support from other parties. FIN 46 is effective immediately for all new variable interest entities created or acquired after January 31, 2003. For variable interest entities created or acquired prior to February 1, 2003, the provisions of FIN 46 must be applied for the first interim or annual period ending after December 15, 2003. We did not have any ownership in any variable interest entities as of December 31, 2002. We will apply the consolidation requirement of FIN 46 in future periods if we own any interest in any variable interest entity.

In May 2003, the FASB issued SFAS No. 150, “Accounting for Certain Financial Instruments with Characteristics of both Liabilities and Equity.” SFAS No. 150 requires that certain financial instruments, which under previous guidance could be accounted for as equity, be classified as liabilities in the statement of financial position. SFAS No. 150 is effective for financial instruments entered into or modified after May 31, 2003. We do not expect the adoption of SFAS No. 150 to have a significant impact on our financial statements.

BUSINESS

Overview

GTx is a biopharmaceutical company dedicated to the discovery, development and commercialization of therapeutics primarily related to the treatment of serious men's health conditions. Our drug discovery and development programs are focused on small molecules that selectively modulate the effects of estrogens and androgens, two essential classes of hormones. We currently have two product candidates that are in human clinical trials. We are developing Acapodene, our most advanced product candidate, through clinical trials for two separate indications: (1) a Phase IIb clinical trial for the reduction in the incidence of prostate cancer in men with precancerous prostate lesions and (2) a pivotal Phase III clinical trial for the treatment of serious side effects of advanced prostate cancer therapy. We are initially developing our second product candidate, Andarine, for the treatment of cachexia from various types of cancer. Cancer cachexia is a muscle wasting condition that is a potentially life-threatening complication of many cancers. Andarine is the most advanced of our internally discovered portfolio of compounds designed to modulate the effects of hormones.

Our most advanced product candidate is Acapodene, which we are developing to reduce the incidence of prostate cancer in men with precancerous prostate lesions known as high grade prostatic intraepithelial neoplasia, or high grade PIN. We have licensed from Orion Corporation the right to develop, market and distribute toremifene, the active pharmaceutical ingredient in Acapodene, worldwide in the field of the prevention and treatment of prostate cancer and the treatment of the principal side effects of prostate cancer therapies. Scientific evidence has established that men who have high grade PIN are at high risk of developing prostate cancer. Currently, there is no therapy for the treatment of high grade PIN. We are conducting a Phase IIb clinical trial in which we have enrolled 515 patients to determine the efficacy and safety of Acapodene in reducing the incidence of prostate cancer in men with high grade PIN. The last patient is scheduled to complete this trial in May 2004, with final results expected in the third quarter of 2004.

We are also developing Acapodene for the treatment of side effects of androgen deprivation therapy, which reduces blood levels of testosterone, the growth factor for prostate cancer. Androgen deprivation therapy is the standard medical treatment for men who have advanced, recurrent or metastatic prostate cancer. Androgen deprivation therapy has serious side effects, including: severe bone loss, or osteoporosis, leading to skeletal fractures; hot flashes; and breast pain and enlargement, or gynecomastia. There are no drugs approved by the FDA for the treatment of these side effects of androgen deprivation therapy. We commenced a pivotal Phase III clinical trial of Acapodene for this indication in November 2003.

Our second product candidate is Andarine, which we are initially developing for the treatment of cachexia from various types of cancer, a potentially life-threatening complication of many cancers. There are no drugs that have been approved by the FDA for the treatment of cancer cachexia. We plan to commence a placebo-controlled, dose-finding Phase II clinical trial for the treatment of cachexia from non-small cell lung cancer in the first half of 2004.

We have multiple product candidates that we are evaluating in preclinical and toxicology studies to support the possible commencement of clinical trials. Our current preclinical product candidates focus on the treatment of major indications in men's health, including benign prostatic hyperplasia, or BPH, a benign prostate enlargement that results in obstruction of the urinary tract; osteoporosis; testosterone deficiency in aging men, or andropause; and prostate cancer.

We believe that our drug discovery capabilities position us well to sustain our clinical pipeline through the design and development of nonsteroidal small molecule drugs that modulate the effects of hormones.

Scientific Background on Estrogens and Androgens

Both estrogens and androgens are hormones that play critical roles in men's health, regulating not only the reproductive system, but also having important effects on the muscular, skeletal, cardiovascular and central nervous systems. In order for the body to function properly, a balance must exist between estrogens and androgens.

Estrogens prevent bone loss and osteoporosis and reduce the risk of skeletal fractures. In aging men, there is a gradual increase in estrogen levels in the blood, which may promote BPH, initiate prostate cancer and cause gynecomastia.

Testosterone is the predominant androgen in men. Testosterone is important for mental well-being and for masculine physical characteristics, such as muscle size and strength, bone strength and male pattern hair growth and loss. Testosterone also stimulates sebaceous glands, which can cause acne. Male reproductive health is also dependent on testosterone to maintain sexual interest, fertility, erectile function and normal prostate growth. In aging men, there is a gradual decrease in testosterone levels, leading to loss of muscle mass and strength, reduced bone mineralization resulting in osteoporosis and bone fractures, erectile dysfunction, decreased sexual interest, depression and mood changes.

In order for estrogens and androgens to perform their physiologic functions, they must interact with and activate their hormone receptors. Hormone receptors are sites located in tissues where hormones bind. Once a hormone binds with its receptor, a series of cellular events is activated, resulting in estrogenic or androgenic tissue effects.

Pharmaceuticals that target hormone receptors for estrogens or androgens have been prescribed for over 50 years. The drugs that have been used to stimulate androgen receptors are natural or synthetic hormones, known as steroids. Steroids activate hormone receptors in all tissue types in a non-selective manner. The absence of selectivity may result in unwanted side effects, such as the potential stimulation of latent prostate cancer, aggravation of existing BPH, acne, hair growth and gynecomastia. Testosterone products also have many pharmacologic limitations, such as an inability to administer them orally. Instead, they must be given by intramuscular injections, patches or gels. The delivery methods of testosterone products are inconvenient for patients and in some cases result in inconsistent levels of testosterone in the blood.

There are also classes of small molecules that are not steroids, but which bind to hormone receptors. These small molecules may either stimulate or block hormone receptors depending on the type of tissue in which the receptor is found. A drug that can either block or stimulate the same hormone receptor is called a receptor modulator. A drug that can either block or stimulate a receptor in a tissue-selective manner may be able to mimic the beneficial, and at the same time minimize the unwanted, effects of natural or synthetic hormones.

A selective estrogen receptor modulator, or SERM, is a small molecule that binds to and selectively modulates estrogen receptors. SERMs have the ability to either stimulate or block estrogen's activity in different tissue types. SERMs have been shown to stimulate estrogen's beneficial action in bone and block estrogen's harmful activity in the breast. In addition, we believe that SERMs have the potential to block estrogen's harmful activity in the prostate. Examples of SERMs currently on the market include tamoxifen, which has been prescribed to treat female and male breast cancer, and raloxifene, which is used to prevent and treat female post-menopausal osteoporosis.

Similarly, a selective androgen receptor modulator, or SARM, is a small molecule that binds to and selectively modulates androgen receptors. In men, we believe that SARMs will be able to stimulate testosterone's beneficial action in bone, muscle and brain, while blocking testosterone's harmful action in the prostate and skin. We further believe that SARMs will have the ability to either cross or not cross into the central nervous system and to selectively modulate receptors depending on tissue type. As a result, although no SARMs have been commercialized to date, we believe that

SARMs could be developed to treat a range of medical conditions and physiological functions, including: (1) low testosterone conditions, such as hypogonadism and andropause; (2) muscle wasting conditions of chronic diseases, such as cancer, AIDS, end stage renal disease, or ESRD, and neurodegenerative disorders, as well as muscle wasting from trauma and burns; (3) disorders of the central nervous system, such as low libido, depression and other mood disorders; (4) male reproductive functions, such as infertility, male contraception and erectile dysfunction; (5) prostate disorders, such as high grade PIN, BPH and prostate cancer; and (6) other conditions, such as anemia, hair loss and male osteoporosis.

Product Candidates

The following table summarizes key information about our product candidates:

Program	Product Candidate/Indication	Development Phase	Status
SERM	**Acapodene** - Reduction in the incidence of prostate cancer in men with high grade PIN	Phase IIb clinical trial	Enrollment complete; last patient scheduled to complete trial in May 2004; final results expected in the third quarter of 2004
	- Side effects of androgen deprivation therapy	Pivotal Phase III clinical trial	Pivotal Phase III clinical trial initiated in November 2003
SARM	**Andarine** - Cachexia from various types of cancer	Three Phase I clinical trials completed	Phase II clinical trials for treatment of cachexia from non- small cell lung cancer scheduled to begin in the first half of 2004
	Prostarine - BPH	Preclinical	Preclinical studies to support IND in progress
	Ostarine - Male osteoporosis and andropause	Preclinical	Preclinical studies to support IND in progress
	Andromustine - Prostate cancer that is not responsive to androgen deprivation therapy	Preclinical	Preclinical studies to support IND in progress

Acapodene

Our most advanced product candidate, Acapodene, is a selective estrogen receptor modulator, or SERM. Acapodene is taken orally and is being developed for a once-a-day dosing schedule. We have licensed from Orion the right to develop, market and distribute toremifene, the active pharmaceutical ingredient in Acapodene, worldwide in the field of the prevention and treatment of prostate cancer and the prevention and treatment of osteoporosis, hot flashes and gynecomastia as side effects of androgen deprivation therapy for prostate cancer. Our license rights are exclusive in North America and Japan. Toremifene is an FDA-approved SERM product for the treatment of advanced breast cancer in post-menopausal women that has been marketed in the United States as Fareston by Shire Pharmaceuticals Group since 1999 and by other companies in other countries for over 10 years. We licensed rights to toremifene based on our belief that a SERM potentially could reduce the incidence of prostate cancer in men with high grade PIN and the established safety and

efficacy record of toremifene in the treatment of post-menopausal women with advanced breast cancer. Orion manufactures commercial quantities of toremifene for Shire and is supplying us with Acapodene under a supply agreement.

The two indications for which we are developing Acapodene target different patient populations: (1) patients who have been diagnosed with high grade PIN, but do not yet have prostate cancer; and (2) patients who have been diagnosed with advanced, recurrent or metastatic prostate cancer and are being treated with androgen deprivation therapy.

Acapodene for the Reduction in the Incidence of Prostate Cancer in Men with High Grade PIN

Scientific Overview. Patients who have an abnormal result from a serum PSA test, a prostate cancer blood test that is commonly administered to men as part of physical examinations, or an abnormal digital rectal examination undergo a prostate biopsy to determine whether they have prostate cancer. Precancerous prostate lesions known as high grade prostatic intraepithelial neoplasia, or high grade PIN, rather than prostate cancer, are detected in approximately 10% of the patients who undergo prostate biopsies. Over the last 17 years, scientific evidence has established that men who have high grade PIN are at high risk of developing prostate cancer. Scientific studies have shown that prostate cancer is found in approximately 30% to 71% of high grade PIN patients within one year of a high grade PIN diagnosis and in 45% to 80% of high grade PIN patients within five years of a high grade PIN diagnosis. Because of this correlation between high grade PIN and prostate cancer, we believe that treating high grade PIN may reduce the incidence of prostate cancer.

Estrogens play an important role in the initiation of prostate cancer. One way estrogens may influence the initiation of prostate cancer is by stimulating high grade PIN and causing it to progress into prostate cancer. Estrogen receptors are found in the prostate and in high grade PIN lesions. In animal models of prostate cancer, blocking estrogens' action has been shown to regress high grade PIN and reduce the incidence of prostate cancer. Because Acapodene is designed to directly block estrogen receptors, we believe that it has the potential to reduce the incidence of prostate cancer in men with high grade PIN.

Potential Market. Prostate cancer is one of the most commonly diagnosed cancers and the second leading cause of cancer-related deaths in men in the United States. There are 400,000 new cases of prostate cancer diagnosed and 239,000 prostate cancer deaths annually worldwide. In the United States, there are over 115,000 new cases of high grade PIN diagnosed each year, and an estimated 9.4 million men unknowingly harbor high grade PIN.

Because there is currently no therapy for the treatment of high grade PIN, patients who are diagnosed with high grade PIN are subjected to repeat biopsies immediately after diagnosis and every three to six months thereafter in order to detect the progression of high grade PIN into prostate cancer. Prostate biopsies are performed through an ultrasound probe placed in the rectum. Hollow needles are then inserted into the prostate to obtain a core of tissue. Complications from this procedure include bleeding, pain, prostate infection and life-threatening blood infection. Because the prostate biopsy technique randomly samples the prostate gland with a relatively thin needle, both prostate cancer and high grade PIN may be missed by the biopsy. Patients with high grade PIN are exposed to the potential complications and the discomfort of invasive, repeat prostate biopsies and suffer the mental anguish of fearing that a diagnosis of prostate cancer may be imminent.

Clinical Trials. In 2000, we completed a Phase IIa clinical trial of Acapodene in 21 patients with high grade PIN. The trial was conducted at the University of Tennessee. Phase IIa clinical trials typically evaluate efficacy and safety and determine the optimal dosing regimen. The primary endpoint of the trial was the presence of high grade PIN. Each participant in the trial received a daily oral dose of Acapodene for four months. The trial was open label and not placebo-controlled, and we did not perform long-term follow-up on the patients in the trial. Each patient underwent a prostate biopsy to detect high grade PIN at the beginning and end of the four-month trial period. Results

showed that 72% of the trial participants had no detectable high grade PIN in the prostate biopsy performed at the end of the trial period. Based on studies reported in scientific literature, only approximately 18% of patients with untreated high grade PIN would be expected to have no high grade PIN detected in their repeat biopsy. There were no serious adverse events attributable to Acapodene in this trial.

Based on the results from our Phase IIa clinical trial, in 2001, we began a placebo-controlled, randomized Phase IIb clinical trial in men with recently diagnosed high grade PIN to determine the efficacy and safety of a daily dose of Acapodene at three dose levels for 12 months. The principal indication of efficacy that we are seeking to verify, or primary endpoint, of the trial is the incidence of prostate cancer, and the ancillary indication of efficacy that we are seeking to verify, or secondary endpoint, of the trial is the presence of high grade PIN. Study patients undergo a series of eight core prostate biopsies at six months and again at 12 months. In order to minimize the inclusion of patients who have, at the time of their enrollment in the trial, prostate cancer that was missed in their initial biopsy, patients in whom prostate cancer is detected six months after enrollment are removed from the trial. Therefore, the prostate cancer incidence will be determined based only on patients who receive Acapodene or the placebo for the entire 12 months. The trial is being conducted at 64 clinical sites across the United States and is fully enrolled with approximately 515 patients.

A planned interim analysis of the first 120 patients in this clinical trial who underwent prostate biopsies at six and again at 12 months was conducted in April 2003. Results of the interim analysis showed that patients who received Acapodene had a 10% to 17% incidence of prostate cancer 12 months after being diagnosed with high grade PIN, depending on the dose of Acapodene, compared to a 23% incidence in the placebo group. This represents an approximately 26% to 57% reduction in prostate cancer incidence in those patients who received Acapodene compared to the placebo group.

To date, four serious adverse events, including one death, have been reported in the 515 patients participating in this Phase IIb clinical trial. Because the safety results are blinded, we do not know whether these events were experienced by participants receiving Acapodene or the placebo. An autopsy was not performed on the 71-year old deceased patient. We have not observed any trend relating these four serious adverse events to Acapodene.

The last patient is scheduled to complete this Phase IIb clinical trial in May 2004, with final results expected in the third quarter of 2004. We believe that if the results of this Phase IIb clinical trial and an anticipated single Phase III clinical trial are positive, this trial and the anticipated Phase III clinical trial will be sufficient to support an application with the FDA for marketing approval of Acapodene for this indication. However, even if we file this application, it may not result in marketing approval from the FDA. We are evaluating the protocol of this pivotal Phase III trial and anticipate initiating the trial in the second half of 2004.

Acapodene for the Treatment of Side Effects of Androgen Deprivation Therapy

Scientific Overview. The standard medical treatment for patients who have advanced, recurrent or metastatic prostate cancer is androgen deprivation therapy, which reduces blood levels of testosterone, the growth factor for prostate cancer. Androgen deprivation therapy is accomplished either surgically by removal of the testes, or chemically by treatment with luteinizing hormone releasing hormone agonists, known as LHRH agonists. LHRH agonists work by shutting off luteinizing hormone secretion by the pituitary gland, which stops testosterone production by the testes. Examples of commercially marketed LHRH agonists are Lupron and Zoladex.

Side effects associated with LHRH agonists include bone loss leading to osteoporosis and skeletal fractures, muscle weakness, hot flashes, gynecomastia, depression, loss of libido and erectile dysfunction. In particular, of the patients treated with LHRH agonists, approximately 60% experience osteoporosis, 22% develop bone fractures, 55% to 80% experience hot flashes and 25% experience gynecomastia. Bone loss leading to osteoporosis and skeletal fractures is a significant

clinical problem because prostate cancer patients who develop skeletal fractures have shorter survival rates compared to patients who do not develop skeletal fractures, with the median survival time shortened by 39 months. Hot flashes occur because of the lack of testosterone in the brain. Hot flashes experienced by prostate cancer patients taking LHRH agonists tend to be severe, frequent and protracted.

Based on the results of our Phase II clinical trials and our preclinical testing of Acapodene, as well as information known about toremifene, we believe that Acapodene has estrogenic activity both in bone, which may prevent osteoporosis, and in the brain, which may reduce hot flashes. In addition, based on the same data and information, we believe that Acapodene can block estrogens' action in the male breast, which may prevent and treat gynecomastia. As a consequence, we believe that Acapodene has the potential to treat three serious side effects of LHRH agonists: osteoporosis, hot flashes and gynecomastia.

Potential Market. In the United States, more than 675,000 men are currently being treated with androgen deprivation therapy for advanced, recurrent or metastatic prostate cancer, with over 120,000 new patients started on this therapy each year. An increasing number of prostate cancer patients are being treated by androgen deprivation with LHRH agonists earlier than in the past because of two main factors. First, medical studies have shown that early androgen deprivation therapy prolongs the survival of prostate cancer patients. Second, the serum PSA test is detecting disease earlier than in the past. However, the effect of this trend is that the side effects of androgen deprivation therapy now contribute significantly to the morbidity, and in some cases the mortality, of men with prostate cancer. Physicians are prescribing some drugs on an off-label basis to help ameliorate some of the individual side effects of androgen deprivation therapy. These drugs include bisphosphonates for osteoporosis, Megace and antidepressants for hot flashes and tamoxifen for gynecomastia. Radiation is also used to treat gynecomastia. However, no single therapy is available to treat multiple side effects of androgen deprivation therapy.

Clinical Trials. We have completed two Phase II clinical trials of Acapodene for the treatment of osteoporosis and hot flashes in patients with advanced, recurrent or metastatic prostate cancer. The first Phase II trial was conducted at five clinical sites across the United States and treated 43 patients with advanced, recurrent or metastatic prostate cancer shortly after initiation of treatment with LHRH agonists. The second of these trials was conducted at three clinical sites across the United States and treated 46 patients with advanced, recurrent or metastatic prostate cancer who had been receiving LHRH agonists for more than 12 months. In each trial, participants were randomized to either a daily oral dose of Acapodene or a placebo for six months. The primary endpoint of both trials was bone mineral density. The secondary endpoint of both trials was the incidence of hot flashes. We measured bone mineral density and hot flash symptoms at entry into each of the clinical trials and at six months. We did not evaluate the effects of Acapodene on gynecomastia in either of these trials. There were no serious adverse events attributable to Acapodene in either of our Phase II clinical trials.

In our first Phase II clinical trial, which evaluated 43 patients shortly after initiation of treatment with LHRH agonists, patients who received Acapodene at the highest tested dose on average experienced an approximately 2% decrease in lumbar vertebral spine bone mineral density at six months, while the patients who received the placebo on average experienced an approximately 4% decrease in lumbar vertebral spine bone mineral density at six months. At the lower tested doses, Acapodene, as compared to the placebo, did not have a meaningfully different effect on lumbar vertebral spine bone mineral density. There was no significant difference between Acapodene and the placebo in the incidence of hot flashes at any tested dose.

In our second Phase II clinical trial, which evaluated 46 patients who had been receiving LHRH agonists for more than 12 months, patients who received Acapodene at the highest tested dose on average experienced a 3.5% increase in lumbar vertebral spine bone mineral density, while the patients who received the placebo on average experienced a 0.5% decrease in lumbar vertebral

spine bone mineral density. Only 12.5% of the patients in this trial who received Acapodene at the highest tested dose, compared to 50% of the patients who received the placebo, reported experiencing an increase in the frequency of hot flashes during the clinical trial. The magnitude of the bone changes seen in treated patients in this Phase II clinical trial were similar to those reported for each of raloxifene and bisphosphonates in post-menopausal women with osteoporosis and bisphosphonates being prescribed off-label to men with prostate cancer. However, bisphosphonates have not been shown to have any effect on hot flashes. At the lower tested doses, Acapodene, as compared to the placebo, did not have a meaningfully different effect on lumbar vertebral spine bone mineral density or frequency of hot flashes.

In November 2003, we initiated a pivotal Phase III clinical trial of Acapodene in patients undergoing androgen deprivation therapy for advanced, recurrent or metastatic prostate cancer. We designed this pivotal Phase III clinical trial principally based on the results of our Phase II clinical trial that evaluated patients who had been receiving LHRH agonists for more than 12 months. The primary endpoint of the trial is the incidence of skeletal fractures. The secondary endpoints of the trial include the measurement of bone loss and the incidence of hot flashes and gynecomastia. We expect that over 60 clinical sites across the United States will participate in this study. Approximately 1,200 patients with advanced, recurrent or metastatic prostate cancer who have been receiving androgen deprivation therapy for at least 24 months and who have significant existing bone loss, or osteopenia, will be randomized to receive either a placebo or a daily dose of Acapodene for 24 months. We are planning an interim analysis of the measurement of bone loss in the first 200 patients in this clinical trial in the first half of 2005.

Andarine

Our second product candidate, Andarine, a selective androgen receptor modulator, or SARM, is the most advanced of our internally discovered portfolio of compounds designed to target hormone receptors. Andarine is taken orally and is being developed for a once-a-day dosing schedule. We believe that Andarine has the potential to treat testosterone deficiency in aging men, or andropause, and related diseases, including male osteoporosis and muscle wasting. Our strategy is to develop Andarine initially for the treatment of a cachexia from various types of cancer. We selected this indication because it represents a potentially large market and, we believe, has a relatively well-defined clinical and regulatory process. Depending on the results of our initial development efforts, we may also develop Andarine for other andropause-related conditions. For cachexia from various types of cancer, we are developing Andarine for the treatment of both men and women.

Andarine for the Treatment of Cancer Cachexia

Scientific Overview. Cachexia is defined as the loss of over 5% of a patient's original body weight. Most of the weight loss attributable to cachexia comes from the loss of lean body weight, resulting from muscle wasting. Cancer causes the body to go into a starvation-like state that causes cachexia. Muscle wasting weight loss from cancer, or cancer cachexia, is diagnosed in approximately one-third of newly-diagnosed cancer patients and accounts for approximately 20% of cancer deaths. Weight loss is one of the most important indicators of how long a cancer patient will live since the survival of a patient with cancer is greatly impacted by the degree and rate of muscle wasting. A cancer patient's response to cancer chemotherapy is diminished by weight loss. Cachexia results in weakness, fatigue and immobility. A greater lean body weight may increase activity levels, quality of life, response to chemotherapy and, ultimately, survival time.

Testosterone increases lean body weight in both men and women. One of the causes of cancer cachexia may be reduced levels of testosterone. Testosterone therapy, however, is not used for the treatment of cancer cachexia for two reasons. First, the delivery methods for testosterone are inconvenient for patients and in some cases result in inconsistent levels of testosterone in the blood. Testosterone cannot be given orally, but rather is given only by intramuscular injections, patches or gels. Second, testosterone has a number of undesirable side effects, such as the potential

stimulation of latent prostate cancer, aggravation of existing BPH and gynecomastia in men and masculinizing effects in women such as acne and facial hair.

We believe that Andarine is similar to testosterone in activating androgen receptors in muscle, thereby promoting lean body weight, but that it does not stimulate sebaceous glands, the cause of hair growth and acne, or the prostate, which exacerbates BPH. In addition, Andarine is taken orally, which makes it convenient to administer.

Potential Market. There are approximately 1.3 million patients diagnosed with cancer each year in the United States. Cancer cachexia afflicts approximately one-third of newly-diagnosed cancer patients. Over 30 clinical trials of supplemental nutritional support alone have reported little or no benefit in counteracting cachexia in cancer patients receiving chemotherapy or radiation. There are no drugs that have been approved by the FDA for the treatment of cancer cachexia. Although there are two commercially available drugs, both steroids, that are being prescribed off-label for the treatment of some types of cancer cachexia, chronic use of these drugs may result in bleeding liver cysts and liver cell tumors.

Clinical Trials. We have completed three Phase I clinical trials of Andarine in a total of 86 healthy male and female volunteers. We tested Andarine for safety and tolerance in single and multiple doses. Results from our Phase I trials support once-a-day oral dosing, and no serious adverse events were observed at any single or multiple dose tested. We observed early indications in the multiple-dose Phase I clinical trial in men that Andarine promoted growth activity, as measured by levels of a growth factor in the blood known as IGF-1, without affecting the sebaceous glands. We believe that these observations support the potential ability of Andarine to selectively modulate androgen receptors in a tissue-specific manner. However, Phase I clinical trials are not designed to show efficacy, and these early observations are not necessarily indicative of the results that will be demonstrated in future clinical trials.

We plan to commence a placebo-controlled dose-finding Phase II clinical trial of Andarine in the first half of 2004 for the treatment of cachexia from non-small cell lung cancer. Cancer cachexia occurs frequently with lung cancer, and the ensuing loss of lean body weight cannot be attributed solely to reduced dietary intake. There are a large number of patients, both male and female, with advanced lung cancer and cancer cachexia, and lung cancer is representative of several other types of cancer. As a result, we selected this patient population to determine the safety and efficacy of Andarine in the treatment of cachexia from non-small cell lung cancer. In our planned Phase II clinical trial, we anticipate that approximately 150 patients who have non-small cell lung cancer and cancer cachexia will be randomized to receive either a daily oral dose of Andarine or a placebo for 12 weeks. The primary endpoint of the trial will be muscle performance, and the secondary endpoints will be lean body weight and other body composition measurements.

Prostarine and Ostarine

We are also developing other SARM product candidates, including:

- Prostarine for the treatment of benign prostatic hyperplasia, or BPH, a benign prostate enlargement that results in obstruction of the urinary tract; and
- Ostarine for the treatment of osteoporosis and andropause.

In animal models, Prostarine shrinks the prostate gland, and Ostarine prevents bone loss and builds bone and muscle. We are conducting preclinical and toxicology studies to support the commencement of clinical trials.

Andromustine

Patients who have advanced, recurrent or metastatic prostate cancer are initially treated with androgen deprivation therapy. Since prostate cancer is dependent on androgens, including

testosterone, to grow, the reduction in testosterone forces prostate cancer into remission. Unfortunately, with time, prostate cancer circumvents the need for testosterone and comes out of remission. Once prostate cancer no longer responds to androgen deprivation, it is referred to as hormone refractory.

Building on the technology of our selective androgen receptor modulator, or SARM, discovery program, we have designed and are developing a small molecule, Andromustine, that is designed to specifically target androgen receptors and kill cancer cells. The Andromustine molecule has two components: (1) the SARM part of the molecule, which is designed to bind to the androgen receptor located on prostate cancer cells; and (2) the chemotherapeutic part of the molecule, which is designed to damage the DNA of prostate cancer cells. In cell culture, Andromustine selectively kills human metastatic prostate cancer cells. Because advanced prostate cancers, including hormone refractory prostate cancer, have more androgen receptors than the normal prostate, Andromustine is designed to bind to and selectively kill advanced prostate cancer cells.

There are over 675,000 men in the United States being treated with LHRH agonists and other hormonal therapies for prostate cancer. Hormone refractory prostate cancer will eventually occur in a majority of these patients. There is currently no effective chemotherapy for hormone refractory prostate cancer. Once a patient develops hormone refractory prostate cancer, his prognosis is poor.

We are in the process of conducting preclinical and animal toxicology studies to support the commencement of clinical trials of Andromustine.

Drug Discovery

Steroid hormone therapies, which include estrogen and testosterone therapies, have been used to treat humans for many years. Steroid hormones cannot, by their nature, have selective effects in various tissues. As a result, they have unintended side effects, which limit their clinical value.

SERM drugs, such as tamoxifen and raloxifene, have achieved commercial success in treating women as nonsteroidal small molecules that modulate hormone receptors in a tissue selective way and minimize some of the side effects of natural hormones. We believe that the success of SERMs indicates that it is possible to design and develop classes of nonsteroidal small molecule drugs to modulate hormone receptors in addition to estrogen receptors.

We believe that our drug discovery expertise positions us well to sustain our clinical pipeline through the design and development of nonsteroidal small molecule drugs that modulate hormone receptors. Our 19 in-house medicinal chemists and scientists provide us with significant discovery and development expertise. Using our capabilities in hormone receptor biology and medicinal chemistry, we are able to target many hormone receptors and generate compounds that are designed to address the shortcomings of natural hormone therapies. We augment our internal drug discovery capabilities through agreements with two universities that provide for our close collaboration with an additional 15 scientists, whose research is largely dedicated to our drug discovery program.

We design and synthesize new compounds based on computer, or *in silico*, models of a hormone receptor's binding sites. We continually modify and improve these *in silico* models to reflect our study of the activity of new compounds in the laboratory, in which we determine the link between chemical structures and biological activity, or structure-activity relationships.

We also have significant medicinal scale-up capabilities, which facilitate our rapid synthesis and evaluation of new compounds. Throughout our discovery process, we build diversity into our chemistry structures in order to improve our likelihood of success in developing novel compounds that have the potential to treat multiple indications. Through this approach, we have generated a clinical product candidate for the androgen receptor, Andarine, as well as additional preclinical compounds of the SARM class and other structurally diverse classes.

Our Strategy

Our objective is to develop and commercialize small molecule drugs to target serious men's health conditions. Key elements of our strategy to achieve this objective are to:

Maximize Commercial Potential of Acapodene

Obtain Regulatory Approval of Acapodene. We are focused on completing clinical trials, obtaining regulatory approval and preparing for the potential commercial launch of Acapodene.

Retain Commercial Rights to Acapodene and Establish Sales and Marketing Infrastructure. We intend to retain all commercial rights to Acapodene in the United States. We believe that we can effectively market Acapodene to the target physician audience of urologists and medical oncologists, principally urological oncologists, in the United States through a small, specialty sales force that we plan to build. We plan to collaborate with pharmaceutical companies to commercialize, market and sell Acapodene in Europe and Asia.

Extend Life Cycle of Acapodene. We intend to reformulate Acapodene with the goals of seeking longer intellectual property protection in the European and Asian markets and extending its life cycle in the United States.

Develop Noninvasive Diagnostic Test for High Grade PIN. We plan to collaborate with a large diagnostics company to develop a noninvasive, accurate blood test to detect high grade PIN. We believe that men would be more willing to be tested for high grade PIN if the diagnostic test were less invasive than a prostate biopsy. Given the large number of patients with undiagnosed high grade PIN, we believe that the development of a noninvasive test will increase the detection of high grade PIN and thereby expand the already large potential market for Acapodene.

Maximize Commercial Potential of Andarine

Pursue Clinical Development of Andarine. We intend to continue to aggressively pursue the clinical development of Andarine for the treatment of cachexia from various types of cancer. In addition, we may develop Andarine for the treatment of other causes of cachexia, including ESRD, which represents a large potential market with unmet medical needs. Andarine could also potentially be developed and commercialized for other men's health indications.

Strategically Seek Collaborators. Because it would require a large sales force to address the cancer cachexia market and because of the risks and costs of developing Andarine for cachexia from various types of cancer, we plan to seek one or more collaborators for the development and commercialization of Andarine for cancer cachexia resulting from all types of cancer other than urological cancers. We also plan to seek a collaborator for potential Andarine indications requiring a large sales force. For Andarine indications for which the target physician market is likely to overlap with that of Acapodene, including cancer cachexia resulting from urological cancers and indications related to andropause, our plan is to market and sell Andarine ourselves or to co-promote it with a collaborator in the United States, and, in the rest of the world, to seek a collaborator.

Build upon Our SARM and other Drug Discovery Capabilities to Sustain Our Small Molecule Product Candidate Pipeline

We intend to develop additional SARMs and other small molecule products to treat diseases that affect large numbers of patients and that are underserved by available alternatives. While our drug discovery efforts to date have focused on SERM and SARM technologies, we believe that we have the capability to discover additional drug candidates that target other hormone receptors. We plan to further strengthen our drug discovery, medicinal chemistry and preclinical pharmacology groups to sustain our pipeline of nonsteroidal small molecules designed to modulate a range of hormone receptors.

Licenses and Collaborative Relationships

We have established and intend to continue to pursue licenses from and collaborative relationships with pharmaceutical companies and academic institutions.

Orion Corporation

Under a license and supply agreement with Orion, we have a license from Orion to develop, use, market and distribute toremifene, the active pharmaceutical ingredient of Acapodene, under Orion's patents covering the composition of matter of toremifene. This license is limited to the fields of the prevention and treatment of prostate cancer and the prevention and treatment of osteoporosis, hot flashes and gynecomastia as side effects of androgen deprivation therapy in the treatment of prostate cancer. Our license rights are exclusive in North America and Japan. Without this license, we would not have the right to commercialize Acapodene for any indication prior to the expiration of the licensed patents. We have a right of first negotiation on a country-by-country basis to negotiate further agreements with Orion for the development, sale and distribution of specified products containing toremifene that are therapeutic equivalents of Acapodene for other indications excluding breast cancer.

Under the terms of the agreement, we paid Orion an initial license fee and have agreed to pay Orion a royalty based on net sales of Acapodene and a share of any consideration we receive for sublicensing our rights under the agreement. We also are required to pay Orion up to $1.0 million if we are acquired before we receive marketing approval for the use of Acapodene in the licensed field.

The agreement requires us to achieve specified minimum sales requirements of Acapodene in the United States or pay Orion royalties on the shortfall amount. Orion may require us to modify our final Acapodene development plans for specified major markets if such plans could adversely affect Fareston or toremifene outside of the licensed field. We have granted Orion a right of first negotiation for Scandinavian marketing rights to Acapodene and to European rights if we do not have a sublicensee in the United States to whom we have granted European marketing rights. We have also agreed to negotiate with Orion for a limited period of time the terms of an agreement granting Orion the exclusive right to distribute Acapodene in Japan, South Korea, China and Taiwan for use in the licensed field. We and our affiliates are prohibited from selling a product that competes with toremifene in the licensed field in major countries located outside the European Union during the term of the agreement and in major countries in the European Union through October 2006.

The term of our license from Orion continues on a country-by-country basis until the date of expiration or invalidation of the last to expire or be invalidated of patents and patent applications relating to Acapodene that we control. Each party has the right to terminate the license under specified circumstances, including in the event of a material breach by the other party that is not cured, bankruptcy of the other party or if the other party is acquired by a direct competitor with respect to toremifene. We also have the right to terminate the agreement in any country if we decide to discontinue the applications or withdraw the applications for regulatory approval of Acapodene due to adverse reactions or safety issues.

The license includes a right for us to use toremifene for research required to obtain regulatory approval. The results of such research are jointly owned by us and Orion, and may be exploited by Orion outside our licensed field.

University of Tennessee Research Foundation

We have exclusive, worldwide licenses from the University of Tennessee Research Foundation under its method of use patents relating to toremifene for the reduction in the incidence of prostate cancer in men with high grade PIN and its composition of matter and method of use patents and patent applications relating to Andarine to market, distribute and sell licensed products. We also have exclusive, worldwide licenses from the University of Tennessee Research Foundation under its

composition of matter and method of use patent applications relating to Prostarine and Ostarine to market, distribute and sell licensed products. Without these licenses, we would not have the right to commercialize these product candidates for any indication prior to the expiration of the licensed patents.

Under the terms of these license agreements, we have agreed to pay the University of Tennessee Research Foundation a royalty based on net sales of licensed products and sublicense income. We are also obligated to pay the University of Tennessee Research Foundation an annual license maintenance fee under each license agreement. The term of each of the license agreements is the longer of 20 years or the term of any licensed patent having a valid claim covering the licensed technology. After the term of each license agreement expires, we will have a perpetual, royalty-free license to the technology licensed under that agreement. The University of Tennessee Research Foundation has the right to terminate each of the agreements under specified circumstances, including in the event that we breach the agreement and do not cure the breach or in the case of our bankruptcy. We are obligated to use commercially reasonable efforts to develop and commercialize products based on the licensed patents and patent applications.

Pursuant to the license agreements, we assign to the University of Tennessee Research Foundation specified patentable inventions arising out of or related to the licensed patents. Upon our request, the University of Tennessee Research Foundation will amend the license agreements to confirm our exclusive licenses to such inventions assigned by us to the University of Tennessee Research Foundation.

National Cancer Institute

We are providing the National Cancer Institute with Acapodene for their use in an independent Phase II clinical trial of Acapodene at the University of Pittsburgh. The objective of the trial is to assess the biological effects of Acapodene on the prostate gland. In this trial, 80 patients who have been diagnosed with prostate cancer will be given a single oral daily dose of Acapodene for 12 weeks prior to surgical removal of their cancerous prostate.

Manufacturing

We do not currently own or operate manufacturing facilities for the production of clinical or commercial quantities of Acapodene or Andarine. We currently rely and expect to continue to rely on third parties for the manufacture of our product candidates or products that we may develop.

We purchase Acapodene from Orion under a license and supply agreement providing for clinical and commercial supply of Acapodene. Orion has agreed to supply us with, and we have agreed to purchase from Orion, our worldwide requirements of Acapodene in finished tablet form at specified transfer prices. Orion's manufacturing facility also produces commercial quantities of toremifene tablets for Fareston and complies with the FDA's current Good Manufacturing Practice regulations. The methods used to manufacture Acapodene are similar to those used to produce the 60 mg toremifene tablet that has been approved by the FDA for the treatment of advanced breast cancer and is marketed in the United States as Fareston. The raw materials necessary to manufacture toremifene are readily available, but Orion is our only supplier of toremifene tablets.

Orion may terminate its obligation to supply us with toremifene if:

- marketing approval for Acapodene for use in the licensed field is not granted in the United States by December 31, 2007 or upon the expiration or invalidation of the last valid claim of the licensed Orion patent rights in the United States; or

- subject to a prior notice requirement, if Orion permanently ceases the manufacture of toremifene.

Our license and supply agreement with Orion does not provide us with the current right to manufacture toremifene. In addition, under the terms of our agreement with Orion, we have agreed to purchase our requirements of toremifene tablets from Orion during the term of the agreement, which extends beyond the term of Orion's patents with respect to the composition of matter of toremifene. There are a number of circumstances in which Orion is required to grant manufacturing rights to us, including following termination of its supply obligation as set forth above, failure by Orion to supply product for 90 days or to supply product in dosages or formulations other than the dosages and formulations specified in the agreement or termination of the agreement by us following a breach by Orion. However, in the event that Orion terminates the license agreement as a result of a material breach of the agreement by us that is not cured, our bankruptcy or the acquisition of us by a direct competitor of Orion with respect to toremifene, we would not have the right to manufacture Acapodene until Orion's patents with respect to the composition of matter of toremifene expire.

We have entered into an agreement with ChemSyn Laboratories, a department of EaglePicher Technologies, LLC, under which ChemSyn has agreed to manufacture Andarine for us in a quantity that we believe is sufficient to supply clinical trials of Andarine for the treatment of cachexia from various types of cancer and initial commercialization of Andarine for this indication. We do not have a contract with ChemSyn for the supply of Andarine for full-scale commercialization. The active ingredient in Andarine is manufactured using a four-step synthetic process that uses commercially available starting materials and raw materials for each step. There are no complicated chemistries or unusual equipment required in the manufacturing process.

Competition

The biotechnology and biopharmaceutical industries are characterized by rapidly advancing technologies, intense competition and a strong emphasis on proprietary products. We face competition from many different sources, including commercial pharmaceutical and biotechnology enterprises, academic institutions, government agencies and private and public research institutions.

Many of our competitors have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, clinical trials, regulatory approvals and marketing approved products than we do. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. Our commercial opportunity will be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer side effects or are less expensive than any products that we may develop. 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 and technology licenses complementary to our programs or advantageous to our business.

Acapodene for the Reduction in the Incidence of Prostate Cancer in Men with High Grade PIN

Currently, there are no products that would compete with Acapodene for the treatment of high grade PIN to reduce the incidence of prostate cancer.

Acapodene for the Treatment of Side Effects of Androgen Deprivation Therapy

Currently, there are no products that have been approved by the FDA to treat multiple side effects of androgen deprivation therapy. We are aware of a number of marketed drugs that are prescribed off-label for the treatment of single side effects. For example, Evista, Eli Lilly's trade name for raloxifene, Fosamax, a bisphosphonate marketed by Merck, and Actonel, a bisphosphonate marketed by Aventis and Proctor & Gamble, are each prescribed off-label for the treatment of osteoporosis. Effexor, marketed by Wyeth Pharmaceuticals, Catapres, marketed by Boehringer Ingelheim, and Megace, marketed by Bristol Myers Squibb, are prescribed off-label to treat hot flashes caused by androgen deprivation therapy. External beam radiation is used to treat

gynecomastia. There are significant side effects associated with the off-label use of these drugs and radiation treatment. Most patients would need to take several different drugs and potentially receive radiation treatments to treat multiple side effects of androgen deprivation therapy. In contrast, we believe that Acapodene, as a single product candidate, has the potential to treat multiple side effects.

Andarine for the Treatment of Cancer Cachexia

There are no drugs that have been approved by the FDA for the treatment of cancer cachexia. Although there are two commercially available drugs, Nandrolone and Oxandrin, that are being prescribed off-label for the treatment of some types of cancer cachexia, chronic use of these drugs may result in bleeding liver cysts and liver cell tumors. Nandrolone is an oral steroid that is available from Steris Laboratories, a subsidiary of Watson Pharmaceuticals. Oxandrin, marketed by Savient Pharmaceuticals, is prescribed for the treatment of involuntary weight loss associated with severe trauma, chronic infection or intensive surgery, as well as off-label for cancer cachexia. Oxandrin is a tissue non-selective steroid that has the potential to stimulate latent prostate cancer and breast cancer and cause virilization in women. Both Nandrolone and Oxandrin, as steroid drugs, have the potential to cause severe liver toxicities. Andarine is not a steroid, and we believe that it will be tissue-selective.

In addition, as to both Acapodene and Andarine, there may be product candidates of which we are not aware at an earlier stage of development. If any are successfully developed and approved, they could compete directly with our product candidates, if approved for commercial sale.

Sales and Marketing

We do not currently have any sales and marketing capabilities. In order to commercialize any products that are approved for commercial sale, we must either develop a sales and marketing infrastructure or collaborate with third parties with sales and marketing experience. We plan to build a small, highly-focused, specialty sales and marketing infrastructure, which we expect to include 50 to 80 sales representatives, to market Acapodene to the relatively small and concentrated community of urologists and medical oncologists, principally urological oncologists, in the United States. We believe that the urology and medical oncology market in the United States is readily accessible by a limited sales and marketing presence due to the concentration of prescribing physicians. We plan to establish collaborations with pharmaceutical companies to commercialize Acapodene in Europe and Asia for prostate cancer-related conditions.

Because it would require a large sales force to address the cancer cachexia market and because of the risks and costs of developing Andarine for cachexia from various types of cancer, we plan to seek one or more collaborators for the development and commercialization of Andarine for cancer cachexia resulting from all types of cancer other than urological cancers. We also plan to seek a collaborator for potential Andarine indications requiring a large sales force. For Andarine indications for which the target physician market is likely to overlap with that of Acapodene, including cancer cachexia resulting from urological cancers and indications related to andropause, our plan is to market and sell Andarine ourselves or to co-promote it with a collaborator in the United States, and, in the rest of the world, to seek a collaborator.

Intellectual Property

We will be able to protect our technology from unauthorized use by third parties only to the extent it is covered by valid and enforceable patents or is effectively maintained as trade secrets. Accordingly, patents and other proprietary rights are an essential element of our business.

For Acapodene, in the United States and internationally we have a license from Orion under its patent covering the composition of matter of toremifene, the active pharmaceutical ingredient in

Acapodene. Our license rights are exclusive in North America and Japan. The patent will expire in the United States in 2009, in Japan in 2005 and in Australia, Italy, Sweden and Switzerland in 2008. This patent has already expired in the other European countries and is likely to expire in countries outside the United States before we commercialize Acapodene. As a result, outside of the United States and in the United States after 2009, we will need to rely primarily on the protection afforded by method of use patents that may issue in respect of our owned or licensed patent applications relating to the use of Acapodene for the relevant indications.

We have licensed from the University of Tennessee Research Foundation method of use patents in the United States and pending patent applications internationally related to the use of Acapodene for the reduction in the incidence of prostate cancer in men with high grade PIN. The method of use patents issued in the United States related to the use of Acapodene for this indication will expire in 2019.

We have our own pending method of use patent applications in the United States and internationally related to the use of Acapodene for the treatment of osteoporosis, gynecomastia and hot flashes as side effects of androgen deprivation therapy.

In all countries in which we hold or have licensed rights to patents or patent applications related to Acapodene, the composition of matter patents will expire before the method of use patents. Furthermore, with respect to the method of use of Acapodene for the treatment of osteoporosis, hot flashes and gynecomastia as side effects of androgen deprivation therapy worldwide and the method of use of Acapodene for the reduction in the incidence of prostate cancer in men with high grade PIN outside the United States, we have only pending patent applications. Method of use patents are more difficult to enforce than composition of matter patents because of the risk of off-label sale or use of the subject compounds.

In the event that patents issue in respect of our pending method of use patent applications, after the expiration of the patent covering the composition of matter of toremifene in a particular country, competitors could market and sell generic versions of toremifene at doses and in formulations that are bioequivalent to Acapodene for uses other than the indications for Acapodene covered by these pending method of use patent applications, and physicians would be permitted to prescribe generic versions of toremifene for indications that are protected by our or our licensors' method of use patents and pending patent applications. After the expiration of the patent covering the composition of matter of toremifene in a particular country, if patents do not issue in respect of our pending method of use patent applications related to the use of Acapodene for the treatment of osteoporosis, hot flashes and gynecomastia as side effects of androgen deprivation therapy worldwide and the method of use of Acapodene for the reduction in the incidence of prostate cancer in men with high grade PIN outside the United States, competitors could market and sell generic versions of toremifene at doses and in formulations that are bioequivalent to Acapodene for these indications.

Our license from Orion is limited to the use of toremifene for the prevention and treatment of prostate cancer and the prevention and treatment of osteoporosis, hot flashes and gynecomastia as side effects of androgen deprivation therapy in the treatment of prostate cancer. Orion has licensed Shire Pharmaceuticals Group in the United States and other parties elsewhere in the world to market, sell and distribute toremifene for the treatment of advanced breast cancer and could license other parties to market, sell and distribute toremifene for other indications in the United States and elsewhere. Shire's product is marketed as Fareston and is currently available only in a 60 mg dose. While we believe that the doses of Acapodene for the indications for which we are developing Acapodene will be different from the dose currently approved by the FDA for Fareston, there may be off-label use of Fareston in place of Acapodene for the indications for which we intend to seek regulatory approval of Acapodene. Additionally, after the expiration of the patent covering the composition of matter of toremifene in some countries, competitors could market and sell generic versions of Fareston in a 60 mg dose. Therefore, if Fareston becomes available at competitive prices

and in doses that are appropriate for the indications for which we are developing Acapodene, off-label sales of Fareston or generic versions of Fareston could reduce sales of Acapodene.

For Andarine, in the United States we have a license from the University of Tennessee Research Foundation under its patents related to the composition of matter and formulations of, and methods of using, the active pharmaceutical ingredient in Andarine. In the United States, the patents covering the composition of matter and formulations of the active pharmaceutical ingredient in Andarine will expire in 2021. We also have a license from the University of Tennessee Research Foundation to its pending patent applications in the United States related to methods of synthesizing the active pharmaceutical ingredient in Andarine and methods for treating cancer cachexia with Andarine. We also have a license from the University of Tennessee Research Foundation to pending patent applications internationally covering the composition of matter of the active pharmaceutical ingredient of Andarine, pharmaceutical compositions of Andarine, formulations of the active pharmaceutical ingredient in Andarine, methods of synthesis of the active pharmaceutical ingredient in Andarine, methods for treating cancer cachexia with Andarine and some other methods of using Andarine. We also have our own pending patent applications in the United States and internationally related to methods of using Andarine.

For Prostarine, we have a license from the University of Tennessee Research Foundation under its pending patent applications in the United States and internationally covering the composition of matter of the active pharmaceutical ingredient in Prostarine, pharmaceutical compositions and formulations of Prostarine and methods of synthesizing the active pharmaceutical ingredient in Prostarine. We also have our own pending patent applications in the United States and internationally related to methods for treating BPH using Prostarine.

For Ostarine, we have a license from the University of Tennessee Research Foundation under its pending patent applications in the United States and internationally covering the composition of matter of the active pharmaceutical ingredient in Ostarine, pharmaceutical compositions and formulations of Ostarine and methods of synthesizing the active pharmaceutical ingredient in Ostarine. We also have our own pending patent applications in the United States and internationally related to methods for treating male osteoporosis and andropause using Ostarine.

For Andromustine, we have pending patent applications of our own in the United States and rights to file internationally covering the composition of matter of the active pharmaceutical ingredient in Andromustine, pharmaceutical compositions of Andromustine, methods of synthesizing the active pharmaceutical ingredient in Andromustine and methods for treating prostate cancer that is not responsive to androgen deprivation therapy using Andromustine.

We also rely on trade secrets, technical know-how and continuing innovation to develop and maintain our competitive position. We seek to protect our proprietary information by requiring our employees, consultants, contractors, outside scientific collaborators and other advisors to execute non-disclosure and assignment of invention agreements on commencement of their employment or engagement, through which we seek to protect our intellectual property. Agreements with our employees also prevent them from bringing the proprietary rights of third parties to us. We also require confidentiality or material transfer agreements from third parties that receive our confidential data or materials.

Government Regulation

New Drug Development and Approval Process

Numerous governmental authorities in the United States and other countries extensively regulate the testing, clinical development, manufacturing and marketing of pharmaceutical products and ongoing research and development activities. In the United States, the FDA rigorously reviews pharmaceutical products under the Federal Food, Drug, and Cosmetic Act and regulations. Non-compliance with applicable requirements can result in administrative and judicial sanctions, including warning letters, clinical holds, fines, recall or seizure of products, injunctions, total or partial suspension of production, refusal of the government to approve marketing applications or allow entry into supply contracts, refusal to permit import or export of products, civil penalties, criminal prosecution and other actions affecting a company and its products. The FDA also has the authority to revoke previously granted marketing authorizations.

To secure FDA approval, an applicant must submit extensive preclinical and clinical data, as well as information about product manufacturing processes and facilities and other supporting information to the FDA for each indication to establish a product candidate's safety and effectiveness. The development and approval process takes many years, requires the expenditure of substantial resources and may be subject to delays or limitations of approval or rejection of the application. Even if the FDA approves a product, the approval is subject to post-marketing surveillance, adverse drug experience and other recordkeeping and reporting obligations, and may involve ongoing requirements for post-marketing studies. The FDA also may place conditions on any approvals that could restrict the commercial applications, advertising, promotion or distribution of these products. Product approvals may be withdrawn if compliance with regulatory standards is not maintained or if problems occur following initial marketing.

Preclinical and Clinical Testing

Preclinical studies involve laboratory evaluation of product characteristics and animal studies to assess the biological activity and safety of the product. In some cases, long-term preclinical studies are conducted while clinical studies are ongoing. The FDA, under its Good Laboratory Practices regulations, regulates preclinical studies. Violations of these regulations can, in some cases, lead to invalidation of the studies, requiring these studies to be replicated. When the preclinical testing is considered adequate by the sponsor to demonstrate the safety and scientific rationale for initial human studies, the results of the preclinical tests, together with manufacturing information and analytical data, are submitted to the FDA as part of an Investigational New Drug application, or IND. The IND becomes effective, if not rejected by the FDA, within 30 days after FDA receives the IND. The FDA may, at any time during the 30-day period after filing of an IND or at any future time, impose a clinical hold on proposed or ongoing clinical trials, on various grounds, including that the study subjects are or would be exposed to an unreasonable and significant health risk. If the FDA imposes a clinical hold, clinical trials cannot commence or recommence without FDA authorization and then only under terms authorized by the FDA.

Clinical trials involve the administration of the investigational product candidates to humans under the supervision of a qualified principal investigator. Clinical trials must be conducted in accordance with Good Clinical Practice, or GCP, under protocols submitted to the FDA as part of the IND. In addition, each clinical trial must be approved and conducted under the auspices of an Investigational Review Board, or IRB, and with patient informed consent. The IRB will consider, among other things, ethical factors and the safety of human subjects.

Clinical trials are conducted in three sequential phases, but the phases may overlap. Phase I clinical trials usually involve between 20 and 80 healthy human subjects or more, depending on the disease. The goal of the Phase I clinical trial is to establish initial data about the safety and tolerance of the product candidates in humans. In Phase II clinical trials, controlled studies are

conducted on an expanded population of patients with the targeted disease. The primary purpose of these tests is to evaluate the effectiveness of the drug candidate on the volunteer patients as well as to determine if there are any side effects or other risks associated with the drug. Phase III trials involve even larger patient populations, often with several hundred or even several thousand patients depending on the use for which the drug is being studied. Phase III trials are intended to establish the overall risk-benefit ratio of the drug and provide, if appropriate, an adequate basis for product labeling. During all clinical trials, physicians monitor the patients to determine effectiveness and to observe and report any reactions or other safety risks that may result from use of the drug candidate.

Product Formulation and Manufacture

Concurrent with clinical trials and preclinical studies, companies must develop information about the chemistry and physical characteristics of the drug and finalize a process for manufacturing the product. In addition, manufacturers, including contract manufacturers, are required to comply with the applicable FDA current Good Manufacturing Practice regulations. The current Good Manufacturing Practice regulations include requirements relating to quality control and quality assurance, as well as the corresponding maintenance of records and documentation. The manufacturing process must be capable of consistently producing quality batches of the product and the manufacturer must develop methods for testing the quality, purity and potency of the final drugs. Additionally, appropriate packaging must be selected and tested and chemistry stability studies must be conducted to demonstrate that the product does not undergo unacceptable deterioration over its shelf-life.

Compliance with current Good Manufacturing Practice regulations also is a condition of new drug application approval. The FDA must approve manufacturing facilities before they can be used in the commercial manufacture of drug products. In addition, manufacturing establishments are subject to preapproval inspections and unannounced periodic inspections.

New Drug Application Process

After the completion of the clinical trial phases of development, if the sponsor concludes that there is substantial evidence that the drug candidate is safe and effective for its intended use, the sponsor may submit a new drug application, or NDA, to the FDA. The application must contain all of the information on the drug candidate gathered to that date, including data from the clinical trials, and be accompanied by a user fee.

The FDA determines whether an NDA as submitted is acceptable for filing. The FDA may refuse to file an application, in which case the FDA retains one-half of the user fee. If the submission is accepted for filing, the FDA begins an in-depth review of the application. As part of this review, the FDA may refer the application to an appropriate advisory committee, typically a panel of clinicians, for review, evaluation and a recommendation. The FDA is not bound by the recommendation of an advisory committee. Under the Prescription Drug User Fee Act, or PDUFA, submission of an NDA with clinical data requires payment of a fee, with some exceptions. In return, FDA assigns a goal of six or 12 months from filing of the application to return of a first “complete response,” in which the FDA may approve the product or request additional information. There can be no assurance that an application will be approved within the performance goal timeframe established under PDUFA.

If the FDA evaluations of the NDA and the manufacturing facilities are favorable, the FDA may issue an approval letter authorizing commercial marketing of the drug candidate for specified indications. The FDA could also issue an approvable letter, which usually contains a number of conditions that must be met in order to secure final approval of the new drug application. When and if those conditions have been met to the FDA’s satisfaction, the FDA will issue an approval letter. On the other hand, if the FDA’s evaluation of the NDA submission or manufacturing facilities is not favorable, the FDA may refuse to approve the NDA or issue a non-approvable letter.

Marketing Approval and Post-marketing Obligations

If the FDA approves an application, the drug becomes available for physicians to prescribe. Periodic reports must be submitted to the FDA, including descriptions of any adverse reactions reported. The FDA may require post-marketing studies, also known as Phase IV studies, as a condition of approval. In addition to studies required by the FDA after approval, trials and studies are often conducted to explore new indications. The purpose of these trials and studies and related publications is to develop data to support additional indications for the drug, which must be approved by the FDA, and to increase its acceptance in the medical community. In addition, some post-marketing studies are done at the request of the FDA to develop additional information regarding the safety of a product.

Any products manufactured or distributed pursuant to FDA approvals are subject to continuing regulation by the FDA, including recordkeeping requirements, reporting of adverse experiences with the drug, drug sampling and distribution requirements, notifying the FDA and gaining its approval of certain manufacturing or labeling changes, complying with certain electronic records and signature requirements, and complying with FDA promotion and advertising requirements. Drug manufacturers and their subcontractors are required to register their establishments and are subject to periodic unannounced inspections for compliance with good manufacturing practice requirements. Also, newly discovered or developed safety or effectiveness data may require changes to a product's approved labeling, including the addition of new warnings and contraindications, or even in some instances revocation or withdrawal of the approval.

Drug Price Competition and Patent Term Restoration Act of 1984

Under the Drug Price Competition and Patent Term Restoration Act of 1984, known as the Hatch-Waxman Act, a portion of a product's patent term that was lost during clinical development and application review by the FDA may be restored. The Hatch-Waxman Act also provides for a statutory protection, known as exclusivity, against the FDA's acceptance or approval of certain competitor applications. The Hatch-Waxman Act also provides the legal basis for the approval of abbreviated new drug applications.

Patent term restoration can compensate for time lost during product development and the regulatory review process by returning up to five years of patent life for a patent that covers a new product or its use. This period is generally one-half the time between the effective date of an IND and the submission date of an NDA, plus the time between the submission date of an NDA and the approval of that application. Patent term restorations, however, are subject to a maximum extension of five years, and the patent term restoration cannot extend the remaining term of a patent beyond a total of 14 years. The application for patent term extension is subject to approval by the United States Patent and Trademark Office in conjunction with the FDA. It takes at least six months to obtain approval of the application for patent term extension.

The Hatch-Waxman Act also provides for a period of statutory protection for new drugs that receive NDA approval from the FDA. If a new drug receives NDA approval as a new chemical entity, meaning that the FDA has not previously approved any other new drug containing the same active entity, then the Hatch-Waxman Act prohibits an abbreviated new drug application or an NDA where the applicant does not own or have a legal right of reference to all of the data required for approval to be submitted by another company for a generic version of such drug, with some exceptions, for a period of five years from the date of approval of the NDA. The statutory protection provided pursuant to the Hatch-Waxman Act will not prevent the filing or approval of a full NDA, as opposed to an abbreviated new drug application or a new drug application in which the applicant does not own or have a legal right of reference to all of the data required for approval, for any drug, including, for example, a drug with the same active ingredient, dosage form, route of administration, strength and conditions of use. In order to obtain an NDA, however, a competitor would be required to conduct its own clinical trials. If NDA approval is received for a new drug containing an active ingredient that

was previously approved by the FDA but the NDA is for a drug that includes an innovation over the previously approved drug, for example, an NDA approval for a new indication or formulation of the drug with the same active ingredient, and if such NDA approval was dependent upon the submission to the FDA of new clinical investigations, other than bioavailability studies, then the Hatch-Waxman Act prohibits the FDA from making effective the approval of an abbreviated new drug application or a new drug application in which the applicant does not own or have a legal right of reference to all of the data required for approval for a generic version of such drug for a period of three years from the date of the NDA approval. This three year exclusivity, however, only covers the innovation associated with the NDA to which it attaches. Thus, the three year exclusivity does not prohibit the FDA, with limited exceptions, from approving abbreviated new drug applications or a new drug application in which the applicant does not own or have a legal right of reference to all of the data required for approval for drugs containing the same active ingredient but without the new innovation.

While the Hatch-Waxman Act provides certain patent restoration and exclusivity protections to innovator drug manufacturers, it also permits the FDA to approve abbreviated new drug applications for generic versions of their drugs. The abbreviated new drug application process permits competitor companies to obtain marketing approval for a drug with the same active ingredient for the same uses but does not require the conduct and submission of clinical studies demonstrating safety and effectiveness for that product. Instead of safety and effectiveness data, an abbreviated new drug application applicant needs only to submit data demonstrating that its product is bioequivalent to the innovator product as well as relevant chemistry, manufacturing and product data. The Hatch-Waxman Act also instituted a third type of drug application that requires the same information as an NDA including full reports of clinical and preclinical studies except that some of the information from the reports required for marketing approval comes from studies which the applicant does not own or have a legal right of reference. This type of application permits a manufacturer to obtain marketing approval for a drug without needing to conduct or obtain a right of reference for all of the required studies.

Finally, the Hatch-Waxman Act requires, in some circumstances, an abbreviated new drug application or a new drug application in which the applicant does not own or have a legal right of reference to all of the data required for approval applicant to notify the patent owner and the holder of the approved NDA of the factual and legal basis of the applicant's opinion that the patent is not valid or will not be infringed. Upon receipt of this notice, the patent owner and the NDA holder have 45 days to bring a patent infringement suit in federal district court and obtain a 30 month stay against the company seeking to reference the NDA. The NDA holder could still file a patent suit after the 45 days, but if they did, they would not have the benefit of the 30 month stay. Alternatively, after this 45-day period, the applicant may file a declaratory judgment action, seeking a determination that the patent is invalid or will not be infringed. Depending on the circumstances, however, the applicant may not be able to demonstrate a controversy sufficient to confer jurisdiction on the court. The discovery, trial and appeals process in such suits can take several years. If such a suit is commenced, the Hatch-Waxman Act provides a 30-month stay on the approval of the competitor's abbreviated new drug application or a new drug application in which the applicant does not own or have a legal right of reference to all of the data required for approval. If the litigation is resolved in favor of the competitor or the challenged patent expires during the 30-month period, unless otherwise extended by court order, the stay is lifted and the FDA may approve the application. Under regulations recently issued by the FDA, and essentially codified under the recent Medicare prescription drug legislation, the patent owner and the NDA holder have the opportunity to trigger only a single 30-month stay per abbreviated new drug application or a new drug application in which the applicant does not own or have a legal right of reference to all of the data required for approval. Once the abbreviated new drug application or a new drug application in which the applicant does not own or have a legal right of reference to all of the data required for approval applicant has notified the patent owner and the NDA holder of the infringement, the applicant cannot be subjected to another 30-month stay, even if the applicant becomes aware of additional patents that may be infringed by its product.

Pharmaceutical Pricing and Reimbursement

In both domestic and foreign markets, 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. The United States and state governments continue to propose and pass legislation designed to reduce the cost of healthcare. Adoption of new legislation could further limit reimbursement for pharmaceuticals.

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 and will continue to increase the pressure on pharmaceutical pricing.

Facilities

We sublease approximately 18,500 square feet of laboratory and office space in Memphis, Tennessee, under an operating lease through September 2005. This lease is terminable by either party on 90 days' notice. We believe that our existing facilities will be sufficient to meet our requirements through 2005.

Employees

As of December 31, 2003, we had 42 employees, of whom 11 were Ph.D.s and 3 were M.D.s. None of our employees is subject to a collective bargaining agreement. We believe that we have good relations with our employees.

Legal Proceedings

We are not currently involved in any material legal proceedings.

MANAGEMENT

Directors, Executive Officers and Other Key Employees

The following table sets forth information about our directors, executive officers and other key employees as of December 31, 2003.

Name	Age	Position(s)
Directors and Executive Officers		
J.R. Hyde, III(1)(2)(3)	61	Chairman of the Board of Directors
Mitchell S. Steiner, M.D., F.A.C.S.	43	Chief Executive Officer and Vice-Chairman of the Board of Directors
Marc S. Hanover	41	President, Chief Operating Officer and Director
Henry P. Doggrell	55	General Counsel and Secretary
Mark E. Mosteller	41	Chief Financial Officer
Rosemary Mazanet, M.D., Ph.D.(1)(2)(3)	48	Director
John H. Pontius(1)(2)(3)	48	Director
Other Key Employees		
K. Gary Barnette, Ph.D.	36	Director of Regulatory Affairs
T. Gary Bird, Ph.D.	51	Director of Manufacturing
Robert S. Boger, M.D.	57	Director of Clinical Development
Karen A. Veverka, Ph.D.	36	Director of Preclinical Development
Michael A. Whitt, Ph.D.	45	Director of Molecular Biology

(1) Member of the Compensation Committee
(2) Member of the Audit Committee
(3) Member of the Nominating and Corporate Governance Committee

J.R. Hyde, III has served as the Chairman of our Board of Directors since November 2000. Since 1989, Mr. Hyde has been the sole stockholder and President of Pittco Holdings, Inc., a private, institutional investment company. Since 1996, when Mr. Hyde made a substantial contribution to support Dr. Steiner's research, Mr. Hyde has been instrumental in forming and financing GTx and is our largest stockholder. Mr. Hyde was the Chairman of the Board of Directors of AutoZone, Inc. from 1986 to 1997 and the Chief Executive Officer of AutoZone from 1986 to 1996. He was also Chairman and Chief Executive Officer of Malone & Hyde, AutoZone's former parent company, from 1972 until 1988. Mr. Hyde is a director of AutoZone, Inc. and FedEx Corporation.

Mitchell S. Steiner, M.D., F.A.C.S., a co-founder of GTx, has served as our Chief Executive Officer and Vice-Chairman of our Board of Directors since our inception in September 1997. Prior to founding GTx, Dr. Steiner held numerous academic appointments, including Chairman and Professor of Urology, Director of Urologic Oncology and Research and the Chair of Excellence in Urologic Oncology at the University of Tennessee. Dr. Steiner holds a B.A. in Molecular Biology from Vanderbilt University and an M.D. from the University of Tennessee, and performed his surgery and urologic training at The Johns Hopkins Hospital.

Marc S. Hanover, a co-founder of GTx, has served as our President and Chief Operating Officer and a director since our inception in September 1997. Prior to joining GTx, Mr. Hanover was a founder of Equity Partners International, Inc., a private equity firm in Memphis, Tennessee, and participated as a founder and investor in three healthcare companies. From 1985 to 1997, Mr. Hanover was a Senior Vice President and a member of the Executive Management Committee of National Bank of Commerce, now National Commerce Financial Corporation, in Memphis, Tennessee. Mr. Hanover holds a B.S. in Biology from the University of Memphis and an M.B.A. in Finance from the University of Memphis.

Henry P. Doggrell has served as our General Counsel and Secretary since October 2001. From April 1998 to August 2001, Mr. Doggrell was Senior Vice President, Corporate Affairs at Buckeye Technologies, Inc., a specialty cellulose company, where he was responsible for matters including corporate finance, investor relations, mergers and acquisitions, intellectual property and licensing and strategic development. From 1996 to 1998, Mr. Doggrell served as General Counsel and Secretary of Buckeye Technologies. Prior to joining Buckeye Technologies, Mr. Doggrell was a partner of the Baker, Donelson, Bearman, Caldwell and Berkowitz law firm from 1988 to 1996, where he served as a member of the law firm management committee and Chair of the firm's Corporate Securities department. Mr. Doggrell holds a B.S. in Commerce from the University of Virginia and a J.D. from Vanderbilt University.

Mark E. Mosteller has served as our Chief Financial Officer since August 2001. From April 1997 to August 2001, Mr. Mosteller was an Executive Vice President of Union Planters Bank National Association, a subsidiary of Union Planters Corporation, a bank holding company, and Chief Operating Officer of Union Planters Mortgage, the mortgage division of Union Planters Bank National Association. From 1994 to 1997, Mr. Mosteller was the Chief Financial Officer of Boatmen's National Mortgage, Inc., the mortgage subsidiary of Boatmen's Bancshares, Inc. From 1984 to 1994, Mr. Mosteller was employed as an audit senior manager with Ernst & Young LLP. Mr. Mosteller is a certified public accountant and holds a B.S. in Accounting from the University of Tennessee.

Rosemary Mazanet, M.D., Ph.D. has served as a director since October 2001. Dr. Mazanet has served as Chief Scientific Officer and a General Partner of Oracle Partners, L.P., a private equity fund, since 1998. Prior to joining Oracle Partners, Dr. Mazanet served as the Director of Clinical Research at Amgen, Inc., a pharmaceutical company. Dr. Mazanet is a member of the Board of Directors of the University of Pennsylvania School of Medicine. She trained in internal medicine at the Brigham and Women's Hospital and in oncology at the Dana Farber Cancer Institute, both part of the Harvard Medical system, where she was a staff physician prior to joining Amgen. Dr. Mazanet holds a B.A. in Biology from the University of Virginia and an M.D. and a Ph.D. in Anatomy from the University of Pennsylvania.

John H. Pontius has served as a director since April 1999. Mr. Pontius has been the President of Pittco Management, LLC, since 1991. From 1986 to 1991, Mr. Pontius served as the chief financial officer of the City of Memphis, Tennessee. Mr. Pontius is a certified public accountant and holds a B.S. in Accounting from the University of Tennessee. Mr. Pontius has served as a member of the Board of Trustees of the University of Tennessee since 2002.

K. Gary Barnette, Ph.D. has served as our Director of Regulatory Affairs since December 2001. From May 1998 to December 2001, Dr. Barnette was Assistant Director and then Director, Regulatory Affairs at Solvay Pharmaceuticals, Inc., a specialty pharmaceutical company. From March 1995 to May 1998, Dr. Barnette was a Clinical Pharmacology and Biopharmaceutics Reviewer at the FDA, where he reviewed in the Divisions of Reproductive and Urologic Drug Products, Metabolic and Endocrine Drug Products and Gastrointestinal and Coagulation Drug Products. Dr. Barnette holds a B.S. in Biology from Salem College, and a Ph.D. in Basic Pharmaceutical Sciences from West Virginia University.

T. Gary Bird, Ph.D. has served as our Director of Manufacturing since October 2003. From 1995 to October 2003, Dr. Bird was a Senior Regulatory Scientist, Senior Quality Consultant and Quality Technical Advisor for Biotechnology in Corporate Quality Assurance at Eli Lilly and Company. Dr. Bird provided regulatory and quality direction to the biotechnology component of Eli Lilly with respect to facility construction and operation. From 1992 to 1995, Dr. Bird was the Assistant to the Deputy Director, Center for Biologics Evaluation and Research at the FDA. Dr. Bird holds a B.S. from the University of Memphis in Invertebrate Zoology/Chemistry, an M.S. from the University of Memphis in Invertebrate Zoology and a Ph.D. in Biochemistry/Entomology from Mississippi State University.

Robert S. Boger, M.D. has served as our Director of Clinical Development since May 2003. From January 2002 until he joined GTx, Dr. Boger was a private consultant specializing in medicine, pharmacology and clinical research. From 1997 to January 2002, Dr. Boger was Director of Clinical Research for Transplantation and Immunology for Novartis Pharmaceuticals. From 1996 to 1997, Dr. Boger served as Director of Medical Research and Clinical Science Leader of Roche's CellCeptTransplant program. Prior to joining Roche, Dr. Boger served as both Associate Director, Clinical Research and Medical Director, Renin Inhibitor Venture for Abbott Laboratories. Dr. Boger holds a B.A. in Biophysics from Amherst College and an M.D. from Harvard Medical School. Dr. Boger is board certified in internal medicine, nephrology and clinical pharmacology.

Karen A. Veverka, Ph.D. has served as our Director of Preclinical Development since August 2000. Dr. Veverka is a co-inventor of several patents held by GTx in the area of medical applications of SARMs. From 1996 to September 2000, Dr. Veverka was a post-doctoral research fellow at St. Jude Children's Research Hospital. Dr. Veverka holds a B.S. in Biochemistry from Kansas State University and a Ph.D. from Mayo Graduate School/The Mayo Foundation.

Michael A. Whitt, Ph.D. has served as our Director of Molecular Biology since April 2001. Dr. Whitt is the co-inventor of several patents licensed to GTx. Dr. Whitt has been on the faculty in the Department of Molecular Sciences at the University of Tennessee Health Sciences since 1991. Dr. Whitt holds a B.A. in Microbiology from the University of Kansas and a Ph.D. in Microbiology from the University of California, Davis. Dr. Whitt received his post-doctoral training at the Yale University School of Medicine.

Board Composition

Upon the completion of this offering, we will have an authorized Board of Directors consisting of five members. In accordance with the terms of our certificate of incorporation and bylaws, which will become effective upon completion of this offering, the 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 completion of this offering, the members of the classes will be divided as follows:

- the class I director will be Dr. Mazanet, and her term will expire at the annual meeting of stockholders to be held in 2005;
- the class II directors will be Mr. Hanover and Mr. Pontius, and their term will expire at the annual meeting of stockholders to be held in 2006; and
- the class III directors will be Dr. Steiner and Mr. Hyde, and their term will expire at the annual meeting of stockholders to be held in 2007.

Our certificate of incorporation that will become effective upon the completion of this offering provides that the authorized number of directors may be changed only by resolution of the Board of Directors. Any additional directorships resulting from an increase in the number of directors will be distributed between the three classes so that, as nearly as possible, each class will consist of one-third of the directors. This classification of the Board of Directors may have the effect of delaying or preventing changes in the control or management of GTx.

Our directors may be removed only for cause by the affirmative vote of the holders of a majority of our voting stock.

Board Committees

Our Board of Directors has an audit committee, a compensation committee and a nominating and corporate governance committee.

Audit Committee

Our audit committee consists of Mr. Hyde, Dr. Mazanet and Mr. Pontius. The functions of the audit committee include:

- meeting with our management periodically to consider the adequacy and effectiveness of our internal controls, the objectivity of our financial reporting and our accounting policies and practices;
- meeting with our independent auditors and with internal financial personnel regarding these matters;
- selecting, overseeing, compensating and engaging our independent auditors;
- reviewing our financial statements and reports and discussing the statements and reports with our management and our independent auditor, including any significant adjustments, management judgments and estimates, new accounting policies and disagreements with management; and
- reviewing our financial plans and reporting recommendations to our full board for approval and to authorize action.

Both our independent auditors and internal financial personnel will regularly meet privately with our audit committee and have unrestricted access to this committee.

Compensation Committee

Our compensation committee consists of Mr. Hyde, Dr. Mazanet and Mr. Pontius. The functions of the compensation committee include:

- reviewing and, as it deems appropriate, recommending to our Board of Directors, policies, practices and procedures relating to the compensation of our directors and executive officers and the establishment and administration of our employee benefit plans;
- exercising administrative authority under our stock plans and employee benefit plans; and
- advising and consulting with our officers regarding managerial personnel and development.

Nominating and Corporate Governance Committee

Our nominating and corporate governance committee consists of Mr. Hyde, Dr. Mazanet and Mr. Pontius. The functions of the nominating and corporate governance committee include:

- reviewing and recommending nominees for election as directors;
- assessing the performance of the Board of Directors;
- developing guidelines for board composition; and
- reviewing and administering our corporate governance guidelines and considering other issues relating to corporate governance.

Compensation Committee Interlocks and Insider Participation

From January 2003 through October 2003, Mr. Pontius, Dr. Steiner, our Chief Executive Officer, and Mr. Hanover, our President and Chief Operating Officer, served as the members of our Compensation Committee. In October 2003, Mr. Hyde and Dr. Mazanet replaced Dr. Steiner and Mr. Hanover as members of our Compensation Committee. None of our executive officers currently serves, or in the past year has served, as a member of the Board of Directors or compensation committee of any entity that has one or more executive officers serving on our Board of Directors or compensation committee.

Director Compensation

We have not provided cash compensation to any director for his or her service as a director. However, following the completion of this offering, we intend to provide cash compensation at a rate of $20,000 per year, payable quarterly to each non-employee director. We also intend to pay the chairman of the audit committee a fee of $5,000 per year, payable quarterly. In addition, we will reimburse directors for their reasonable expenses incurred in attending meetings of the Board of Directors.

Our 2004 Non-Employee Directors' Stock Option Plan, which will become effective upon completion of this offering, provides for the automatic grant of options to purchase shares of common stock to our non-employee directors except for Mr. Hyde and any other non-employee director who owns ten percent or more of the combined voting power of our outstanding securities. Prior to adoption of our 2004 Non-Employee Directors' Stock Option Plan, we did not make option grants to our non-employee directors. Upon completion of this offering, each of our non-employee directors, except for Mr. Hyde and any other non-employee director who owns ten percent or more of the voting power of our outstanding securities, will receive an initial option to purchase 10,000 shares of common stock and annual option grants to purchase 2,000 shares of common stock starting at the annual stockholders meeting to be held in 2005. Please refer to the section entitled "Benefit Plans — 2004 Non-Employee Directors' Stock Option Plan" for a more detailed explanation of the terms of these stock options.

Executive Compensation

The following table shows the compensation awarded or paid to, or earned by, our chief executive officer and our three other most highly compensated executive officers for the fiscal years ended December 31, 2002 and December 31, 2003 whose total annual salary and bonus exceeded $100,000. We refer to these executive officers in this prospectus as our "named executive officers."

Summary Compensation Table

		Annual Compensation	Long-Term Compensation Awards
Name and Principal Position	Fiscal Year	Salary ($)	Securities Underlying Options (#)
Mitchell S. Steiner, M.D., F.A.C.S.	2003	$311,666	—
Chief Executive Officer	2002	175,000	—
Marc S. Hanover	2003	180,000	—
President and Chief Operating Officer	2002	180,000	—
Henry P. Doggrell	2003	193,000	12,750
General Counsel and Secretary	2002	178,750	—
Mark E. Mosteller	2003	154,167	42,500
Chief Financial Officer	2002	135,417	17,000

Stock Option Grants in Last Fiscal Year

We have granted and will continue to grant options to our executive officers and employees under our benefit plans. The exercise price per share of each option granted during 2003 was equal to the fair market value of our common stock as determined by our Board of Directors on the date of grant.

The following table shows information regarding grants of stock options to our named executive officers during the fiscal year ended December 31, 2003. We have never granted any stock appreciation rights.

	Option Grants in Last Fiscal Year						
					Potential Realizable Value at Assumed Annual Rates of Stock Price Appreciation for Option Terms(2)		
Name	Number of Securities Underlying Options Granted(#)	Percent of Total Options Granted to Employees(%)(1)	Exercise Price Per Share($)	Expiration Date	0%($)	5%($)	10%($)
Mitchell S. Steiner, M.D., F.A.C.S.	—	—	—	—	—	—	—
Marc S. Hanover	—	—	—	—	—	—	—
Henry P. Doggrell	12,750	2.4%	$6.24	9/1/2013	$105,375	$221,642	$400,018
Mark E. Mosteller	17,000	3.2	6.24	8/1/2013	140,500	295,523	533,358
	25,500	4.8	6.24	9/1/2013	210,750	443,284	800,036

(1) Based on aggregate of 533,375 shares subject to options granted to our employees in 2003, including the named executive officers.

(2) Potential realizable values are computed by (1) multiplying the number of shares of common stock subject to a given option by the initial public offering price of $14.50, (2) assuming that the aggregate stock value derived from that calculation compounds at the annual 0%, 5% or 10% rate shown in the table for the entire ten-year term of the option and (3) subtracting from that result the aggregate option exercise price. The 0%, 5% and 10% assumed annual rates of stock price appreciation are mandated by the rules of the SEC and do not reflect our estimate or projection of future stock prices. Actual gains, if any, on stock option exercises will depend on the future performance of the common stock and the date on which the options are exercised.

Fiscal Year End Option Values

The following table sets forth the number of shares of common stock subject to vested and unvested stock options held as of December 31, 2003 by each of our named executive officers. Because there was no public market for our common stock as of December 31, 2003, amounts described in the following table under the heading “Value of Unexercised In-the-Money Options at December 31, 2003” are determined by multiplying the number of shares underlying the options by the difference between the initial public offering price of $14.50 per share and the per share option exercise price. None of our named executive officers exercised any stock options during 2003.

	Number of Securities Underlying Unexercised Options at December 31, 2003 (#)		Value of Unexercised In-the-Money Options at December 31, 2003 ($)	
Name	Exercisable	Unexercisable	Exercisable	Unexercisable
Mitchell S. Steiner, M.D., F.A.C.S.	—	—	—	—
Marc S. Hanover	—	—	—	—
Henry P. Doggrell	51,000	89,250	$393,540	$695,685
Mark E. Mosteller	—	85,000	—	679,200

Change in Control Arrangements

Our 1999 Stock Option Plan, 2000 Stock Option Plan, 2001 Stock Option Plan and 2002 Stock Option Plan provide that in the event of a change in control of us, all shares subject to option awards under the plans will immediately vest and be converted into cash, options or stock of equivalent value in the surviving organization under terms and conditions that substantially preserve the economic status of plan participants. For this purpose, a change in control includes (1) a sale or

disposition of more than 50% of our issued and outstanding voting stock; (2) a merger or consolidation in which our stockholders immediately before the transaction own less than 50% of the outstanding voting securities of the surviving entity immediately after the transaction; or (3) a sale or disposition of all or substantially all of our assets.

Our employment agreements with our executive officers and other key employees contain provisions triggered by a change of control. See “Employment Agreements.”

Our 2004 Equity Incentive Plan provides that in the event of specified corporate transactions, all outstanding options and stock appreciation rights under the incentive plan will be assumed, continued or substituted for by any surviving or acquiring entity. If the surviving or acquiring entity elects not to assume, continue or substitute for such awards, such equity awards will become fully vested, and, if applicable, exercisable and such equity awards will be terminated if not exercised prior to the effective date of the corporate transaction. Other forms of equity awards, such as restricted stock awards, may have their repurchase or forfeiture rights assigned to the surviving or acquiring entity. If such repurchase or forfeiture rights are not assigned, then such equity awards will become fully vested. Following specified change in control transactions, the vesting and exercisability of specified equity awards generally will be accelerated only if the awardee’s award agreement so specifies. The standard form of stock option agreement provides for the option to become fully vested and exercisable if the option holder’s service with the company or its successor terminates within 12 months after a change of control and the termination of service is a result of an involuntary termination without cause or a constructive termination.

Our 2004 Non-Employee Directors’ Stock Option Plan provides that in the event of specified corporate transactions, all outstanding options under the plan will be either assumed, continued or substituted for by any surviving entity. If the surviving or acquiring entity elects not to assume, continue or substitute for such options, the vesting and exercisability of such options will be accelerated in full and such options will be terminated if not exercised prior to the effective date of such corporate transaction. In the event of specified changes in control, the outstanding options under the Plan granted to non-employee directors will become fully vested and exercisable as of such change in control. In addition, such non-employee director’s options shall become fully vested and exercisable if such director must resign as a condition of the change in control.

Employment Agreements

Each of our named executive officers has entered into an employment agreement with us. These employment agreements provide for salary as well as other customary benefits and terms. Pursuant to their employment agreements, Dr. Steiner, Mr. Hanover, Mr. Doggrell and Mr. Mosteller are currently entitled to receive an annual salary of $375,000, $180,000, $202,000 and $160,000. In addition, our Board of Directors has the discretion to award bonus compensation to our named executive officers. Each employment agreement is terminable by either us or the named executive officer at any time. If we experience a change of control and the named executive officer’s employment is terminated without cause, or if the named executive officer terminates his employment for good reason, at any time within six months after the change in control, then such named executive officer will receive continued payment of his then base salary for a period of one year after the termination date. Dr. Steiner and Mr. Hanover have each agreed not to compete with us during the term of their employment and for a period of two years after their employment ends. If we undergo a change in control, the two year period will be shortened to one year.

Benefit Plans

1999 Stock Option Plan and 2000 Stock Option Plan

We adopted the 1999 Stock Option Plan in August 1999 and the 2000 Stock Option Plan in November 2000. Neither the 1999 Stock Option Plan nor the 2000 Stock Option Plan has a stated termination date. However, the committee of the Board of Directors that administers the 1999 Stock Option Plan and 2000 Stock Option Plan may terminate or suspend the 1999 Stock Option Plan and 2000 Stock Option Plan at any time. The 1999 Stock Option Plan and 2000 Stock Option Plan provide for the grant of nonstatutory stock options to directors, officers and employees.

Share Reserve. An aggregate of 24,650 shares of common stock are reserved for issuance under the 1999 Stock Option Plan. No options were outstanding under the 1999 Stock Option Plan as of December 31, 2003. An aggregate of 108,375 shares of common stock are reserved for issuance under the 2000 Stock Option Plan. Options to purchase an aggregate of 74,375 shares of common stock were outstanding under the 2000 Stock Option Plan as of December 31, 2003.

Shares subject to stock options that have expired or otherwise terminated under the 1999 Stock Option Plan or 2000 Stock Option Plan without having been exercised in full and grants that are settled in cash rather than stock again become available for the grant of awards under the 1999 Stock Option Plan or 2000 Stock Option Plan. Shares issued under the 1999 Stock Option Plan or 2000 Stock Option Plan may be previously unissued shares or reacquired shares bought on the market or otherwise.

Administration. The 1999 Stock Option Plan and 2000 Stock Option Plan are administered by a committee of our Board of Directors. Subject to the terms of the 1999 Stock Option Plan and 2000 Stock Option Plan, the committee determines the recipients, the number of stock options to be granted and the terms and conditions of the stock options. Subject to the limitations set forth below, the committee also determines the exercise price of options granted.

Stock Options. Stock options under the 1999 Stock Option Plan and 2000 Stock Option Plan are granted pursuant to stock option agreements. The exercise price for a stock option cannot be less than the fair market value of the common stock on the date of grant. Options granted under the 1999 Stock Option Plan and 2000 Stock Option Plan vest one-third on the third anniversary of the date of grant, one-third on the fourth anniversary of the date of grant, and one-third on the fifth anniversary of the date of grant. If the 1999 Stock Option Plan or the 2000 Stock Option Plan is terminated, all outstanding options will become fully vested and exercisable.

The term of stock options granted under the 1999 Stock Option Plan and 2000 Stock Option Plan may not exceed 10 years. If an optionee's service relationship with us ceases due to voluntary retirement, at or after age 65 or after age 55 with no fewer than 10 years of service, death, disability or involuntary termination, other than a termination for cause, but including any involuntary termination as a result of a change of control, any vested shares may be exercised at any time within 10 years following the date of grant of the option. If an optionee's relationship with us ceases for any other reason, any unvested option shall be forfeited immediately and the date of such termination will be the last date on which a vested option can be exercised. Any vested but unexercised options will terminate upon the optionee competing with us.

Acceptable consideration for the purchase of common stock issued under the 1999 Stock Option Plan and 2000 Stock Option Plan include cash or, at the discretion of the committee, common stock, a deferred payment arrangement or other legal consideration approved by the committee. Generally, an optionee may not transfer a stock option granted under the 1999 Stock Option Plan and 2000 Stock Option Plan, other than by will or the laws of descent and distribution unless the optionee holds a nonstatutory stock option that provides otherwise.

Changes in Control. The 1999 Stock Option Plan and 2000 Stock Option Plan provide that in the event of a change in control of us, all shares subject to option awards under the plans shall immediately vest and be converted into cash, options or stock of equivalent value in the surviving

organization under terms and conditions that substantially preserve the economic status of plan participants. For this purpose, a change in control includes (1) a sale or disposition of more than 50% of our issued and outstanding voting stock; (2) a merger or consolidation in which our stockholders immediately before the transaction own less than 50% of the outstanding voting securities of the surviving entity immediately after the transaction; or (3) a sale or disposition of all or substantially all of our assets.

2001 Stock Option Plan and 2002 Stock Option Plan

In October 2001, we adopted the 2001 Stock Option Plan. Our Board of Directors amended the 2001 Stock Option Plan in November 2001. The 2001 Stock Option Plan will terminate in October 2011 unless the Board of Directors terminates it earlier. In August 2002, we adopted the 2002 Stock Option Plan. The 2002 Stock Option Plan will terminate in August 2012 unless the Board of Directors terminates it earlier. The 2001 Stock Option Plan and the 2002 Stock Option Plan provide for the grant of options that are:

- incentive stock options, as defined under the Internal Revenue Code of 1986, as amended, or the Code, which may be granted solely to employees, including officers; and

- nonstatutory stock options, which may be granted to directors, employees, including officers, or consultants.

Share Reserve. An aggregate of 298,775 shares of common stock are reserved for issuance under the 2001 Stock Option Plan. Options to purchase an aggregate of 261,375 shares of common stock were outstanding under the 2001 Stock Option Plan as of December 31, 2003. An aggregate of 850,000 shares of common stock are reserved for issuance under the 2002 Stock Option Plan. Options to purchase an aggregate of 493,000 shares of common stock were outstanding under the 2002 Stock Option Plan as of December 31, 2003.

Shares subject to stock options that have expired or otherwise terminated under the 2001 Stock Option Plan or 2002 Stock Option Plan without having been exercised in full again become available for the grant of awards under the 2001 Stock Option Plan or 2002 Stock Option Plan. Shares issued under the 2001 Stock Option Plan or 2002 Stock Option Plan may be previously unissued shares or reacquired shares bought on the market or otherwise.

Administration. The 2001 Stock Option Plan and 2002 Stock Option Plan are administered by a committee of our Board of Directors. Subject to the terms of the 2001 Stock Option Plan and 2002 Stock Option Plan, the committee determines the recipients, the number and type of stock options to be granted and the terms and conditions of the stock options. Subject to the limitations set forth below, the committee also determines the exercise price of options granted.

Stock Options. Stock options are granted under the 2001 Stock Option Plan and 2002 Stock Option Plan pursuant to stock option agreements. The exercise price for an incentive stock option cannot be less than the fair market value of the common stock on the date of grant. There is no restriction on the exercise price for a nonstatutory stock option. Unless otherwise specified in an option agreement, options granted under the 2001 Stock Option Plan or 2002 Stock Option Plan vest one-third on the third anniversary of the date of grant, one-third on the fourth anniversary of the date of grant, and one-third on the fifth anniversary of the date of grant.

The term of stock options granted under the 2001 Stock Option Plan or 2002 Stock Option Plan may not exceed 10 years. Unless otherwise provided for in the stock option agreement, options granted under the 2001 Stock Option Plan or 2002 Stock Option Plan terminate three months after termination of the optionee's employment or service as a director of GTx or an affiliate unless (1) the termination is due to the optionee's disability, in which case the option may provide that it may be exercised at any time within one year following termination of employment or relationship; (2) the termination is due to the death of optionee or death occurs within three months after the termination of the optionee, in which case the option may provide that it may be exercised at any time within

18 months following the death of optionee; or (3) the termination is due to voluntary retirement, subject to some conditions, in which case the option may be exercised at any time within five years of the date of retirement subject to the express term of the option. Any vested but unexercised options will terminate upon the optionee competing with us.

Acceptable consideration for the purchase of common stock issued under the 2001 Stock Option Plan or 2002 Stock Option Plan include cash or, at the discretion of the committee, common stock, a deferred payment arrangement or other legal consideration approved by the committee. Generally, an optionee may not transfer a stock option granted under the 2001 Stock Option Plan or 2002 Stock Option Plan, other than by will or the laws of descent and distribution unless the optionee holds a nonstatutory stock option that provides otherwise.

Tax Limitations on Stock Option Grants. Incentive stock options may be granted only to our employees. The aggregate fair market value, determined at the time of grant, of shares of our common stock with respect to incentive stock options that are exercisable for the first time by an optionee during any calendar year under all of our stock plans may not exceed $100,000. The options or portions of options that exceed this limit are treated as nonstatutory stock options. No incentive stock option, and before our stock is publicly traded, no nonstatutory stock option, may be granted to any person who, at the time of the grant, owns or is deemed to own stock possessing more than 10% of our total combined voting power or any affiliate unless the following conditions are satisfied:

- the option exercise price must be at least 110% of the fair market value of the stock subject to the option on the date of grant; and
- the term of any incentive stock option award must not exceed five years from the date of grant.

Changes in Control. The 2001 Stock Option Plan and 2002 Stock Option Plan provide that in the event of a change in control of us, all shares subject to option awards under the plans shall immediately vest and be converted into cash, options or stock of equivalent value in the surviving organization under terms and conditions that substantially preserve the economic status of plan participants. For this purpose, a change in control includes (1) a sale or disposition of more than 50% of our issued and outstanding voting stock; (2) a merger or consolidation in which our stockholders immediately before the transaction own less than 50% of the outstanding voting securities of the surviving entity immediately after the transaction; or (3) a sale or disposition of all or substantially all of our assets.

2004 Equity Incentive Plan

We adopted and our stockholders approved our 2004 Equity Incentive Plan in January 2004 to become effective upon the closing of this offering. The 2004 Equity Incentive Plan will terminate when the Board of Directors terminates the plan. The 2004 Equity Incentive Plan provides for the grant of nonstatutory stock options, restricted stock awards, stock appreciation rights, phantom stock rights and other forms of equity compensation, which may be granted to employees, including officers, non-employee directors and consultants.

Share Reserve. An aggregate of 1,500,000 shares of common stock will be reserved for issuance under the 2004 Equity Incentive Plan, which amount will be increased annually on January 1st of each year, from 2005 until 2013, by five percent of the number of shares of common stock outstanding on such date. However, the Board of Directors has the authority to designate a smaller number of shares by which the authorized number of shares of common stock will be increased on such date. As of the date hereof, no shares of common stock have been issued under the 2004 Equity Incentive Plan.

The following types of shares issued under the 2004 Equity Incentive Plan may again become available for the grant of new awards under the 2004 Equity Incentive Plan: restricted stock that is

repurchased or forfeited prior to it becoming fully vested; shares withheld for taxes; shares used to pay the exercise price of an option in a net exercise; and shares tendered to the company to pay the exercise price of an option. In addition, shares subject to stock options that have expired or otherwise terminated without having been exercised in full may again become available for the grant of new awards under the 2004 Equity Incentive Plan. Shares issued under the 2004 Equity Incentive Plan may be previously unissued shares or reacquired shares bought on the market or otherwise.

Administration. Our Board of Directors will administer the 2004 Equity Incentive Plan. The Board of Directors may delegate authority to administer the 2004 Equity Incentive Plan to a committee. Subject to the terms of the 2004 Equity Incentive Plan, our Board of Directors or its authorized committee, the plan administrator, determines recipients, grant dates, the numbers and types of equity awards to be granted and the terms and conditions of the equity awards, including the period of their exercisability and vesting. Subject to the limitations set forth below, the plan administrator will also determine the exercise price of options granted, the purchase price for rights to purchase restricted stock and, if applicable, phantom stock and the strike price for stock appreciation rights. The plan administrator may also amend the terms of the plan and outstanding equity awards. Amendments to the 2004 Equity Incentive Plan are subject to shareholder approval to the extent required by law, rule or regulation. In addition, the plan administrator may amend an option to lower its exercise price or exchange an option for an option with a lower exercise price, another equity award, cash or any other valuable consideration or may take any other action that is treated as a repricing under generally accepted accounting principles.

Nonstatutory Stock Options. Nonstatutory stock options will be granted pursuant to nonstatutory stock option agreements. The plan administrator determines the exercise price for a nonstatutory stock option. Options granted under the 2004 Equity Incentive Plan vest at the rate specified in the option agreement.

Generally, the plan administrator determines the term of nonstatutory stock options granted under the 2004 Equity Incentive Plan. Unless the terms of an optionee's nonstatutory stock option agreement provide otherwise, if an optionee's service relationship with us, or any of our affiliates, ceases due to disability or death, the optionee, or his or her beneficiary, may exercise any vested options up to 12 months in the event of disability, 18 months in the event of death and 24 months in the event of retirement, after the date such service relationship ends. If an optionee's relationship with us, or any affiliate of ours, ceases for any reason other than disability, death or retirement the optionee may exercise any vested options up to three months from cessation of service, unless the terms of the stock option agreement provide for earlier or later termination.

Acceptable consideration for the purchase of common stock issued upon the exercise of a nonstatutory stock option will be determined by the plan administrator and may include cash, common stock previously owned by the optionee, a broker assisted exercise and the net exercise of the option.

Generally, an optionee may not transfer a nonstatutory stock option other than by will or the laws of descent and distribution unless the nonstatutory stock option agreement provides otherwise. However, an optionee may designate a beneficiary who may exercise the option following the optionee's death.

Restricted Stock Awards. Restricted stock awards are purchased through a restricted stock award agreement. The purchase price for restricted stock awards must be at least the par value of the stock. The purchase price for a restricted stock award may be payable in cash or the recipient's past or future services performed or to be performed for us or any of our affiliates. Rights to acquire shares under a restricted stock award may not be transferred other than by will or by the laws of descent and distribution.

Stock Appreciation Rights. Stock appreciation rights are granted pursuant to stock appreciation rights agreements. The plan administrator determines the strike price for a stock

appreciation right. A stock appreciation right granted under the 2004 Equity Incentive Plan vests at the rate specified in the stock appreciation right agreement.

The plan administrator determines the term of stock appreciation rights granted under the 2004 Equity Incentive Plan. If an awardee's service relationship with us, or any of our affiliates, ceases due to disability or death, the awardee, or his or her beneficiary, may exercise any vested stock appreciation right up to three months or such longer or shorter period of time provided in the stock appreciation rights agreement. Different post-termination exercise periods may be provided in the stock appreciation rights agreement for specific terminations of service such as death, disability or retirement.

Phantom Stock Awards. Phantom stock awards are granted pursuant to phantom stock award agreements. A phantom stock award may require the payment of at least par value. Payment of any purchase price may be made in any form of legal consideration acceptable to the plan administrator. Rights to acquire shares under a phantom stock agreement may not be transferred other than by will or by the laws of descent and distribution.

Other Equity Awards. The plan administrator may grant other awards based in whole or in part by reference to our common stock. The plan administrator will set the number of shares under the award, the purchase price, if any, the timing of exercise and vesting and any repurchase rights associated with such awards. Unless otherwise specifically provided for in the award agreement, such awards may not be transferred other than by will or by the laws of descent and distribution.

Changes in Control. In the event of specified corporate transactions, all outstanding options and stock appreciation rights under the incentive plan either will be assumed, continued or substituted for by any surviving or acquiring entity. If the surviving or acquiring entity elects not to assume, continue or substitute for such awards, such equity awards will become fully vested and exercisable and such equity awards will be terminated if not exercised prior to the effective date of the corporate transaction. Other forms of equity awards such as restricted stock awards may have their repurchase or forfeiture rights assigned to the surviving or acquiring entity. If such repurchase or forfeiture rights are not assigned, then such equity awards will become fully vested. Following specified change in control transactions, the vesting and exercisability of specified equity awards generally will be accelerated only if the awardee's award agreement so specifies. The standard form of stock option agreement provides for options to become fully vested and exercisable if an optionee is involuntarily terminated without cause or has a constructive termination, in either case, within twelve months after the change in control.

2004 Non-Employee Directors' Stock Option Plan

We adopted and our stockholders approved our 2004 Non-Employee Directors' Stock Option Plan in January 2004 to become effective upon the closing of this offering. The 2004 Non-Employee Directors' Stock Option Plan provides for the automatic grant of nonstatutory stock options to purchase shares of common stock to our non-employee directors who own less than ten percent of the combined voting power of our then outstanding securities.

Share Reserve. The aggregate number of shares of common stock that may be issued pursuant to options granted under the 2004 Non-Employee Directors' Stock Option Plan is 200,000 shares, which amount will be increased annually on January 1st of each year, from 2005 and until 2013, by the number of shares of common stock subject to options granted during the prior calendar year. However, the Board of Directors has the authority to designate a smaller number of shares by which the authorized number of shares of common stock will be increased. As of the date hereof, no shares of common stock have been issued under the 2004 Non-Employee Directors' Stock Option Plan.

Administration. Our Board of Directors will administer the 2004 Non-Employee Directors' Stock Option Plan. The exercise price of the options granted under the 2004 Non-Employee Directors' Stock Option Plan will be equal to the fair market value of the common stock on the date of grant; provided,

however that initial grants made within three months after the initial public offering will have an exercise price equal to the offering price. No option granted under the 2004 Non-Employee Directors' Stock Option Plan may be exercised after the expiration of ten years from the date it was granted. Options granted under the 2004 Non-Employee Directors' Stock Option Plan are transferable only to the extent permitted under the rules of a Form S-8 registration statement. In addition, such options are transferable by will or by the laws of descent and distribution. Such options are exercisable during the life of the optionee only by the optionee or a permitted transferee. An optionee may designate a beneficiary who may exercise the option following the optionee's death. An optionee whose service relationship with the us or any of our affiliates, whether as a non-employee director of the company or subsequently as an employee, director or consultant of either the company or an affiliate, ceases for any reason may exercise vested options for the term provided in the option agreement, three months generally, 12 months in the event of disability and 18 months in the event of death and 12 months after a termination of service occurring on or as a condition of a change in control.

Automatic Grants. Upon the completion of this offering, each eligible non-employee director will automatically be granted an option to purchase 10,000 shares of common stock, the initial grant. Any individual who becomes an eligible non-employee director after this offering will automatically be granted the initial grant upon election to the Board of Directors. Any person who is an eligible non-employee director on the day after an annual meeting of our stockholders, commencing with our annual meeting in 2005, automatically will be granted an option to purchase 2,000 shares of common stock, the annual grant, on such date; *provided*, *however*, that an eligible non-employee director will not receive an annual grant until the first annual meeting that is at least one year after the date of his or her initial grant. Initial grants and annual grants vest in three equal annual installments.

Changes in Control. In the event of specified corporate transactions, all outstanding options under the 2004 Non-Employee Directors' Stock Option Plan will be either assumed, continued or substituted for by any surviving entity. If the surviving or acquiring entity elects not to assume, continue or substitute for such options, such options will become fully vested and exercisable and such options will be terminated if not exercised prior to the effective date of such corporate transaction. In the event of specified changes in control, outstanding options granted under the 2004 Non-Employee Directors' Stock Option Plan granted to non-employee directors will become fully vested and exercisable as of the change in control. In addition, options held by non-employee directors will become fully vested and exercisable if such director must resign from our Board of Directors as a condition of a change in control.

401(k) Plan

We maintain a retirement and deferred savings plan for our employees. The retirement and deferred savings plan is intended to qualify as a tax-qualified plan under Section 401 of the Code. The retirement and deferred savings plan provides that each participant may contribute up to 15% of his or her pre-tax compensation, up to a statutory limit, which for most employees is $13,000 in 2004. Under the plan, each employee is fully vested in his or her deferred salary contributions. Employee contributions are held and invested by the plan's trustee. The retirement and deferred savings plan also permits us to make discretionary contributions and matching contributions, subject to established limits and a vesting schedule. To date, we have not made any discretionary contributions to the retirement and deferred savings plan on behalf of participating employees.

Limitations on Directors' Liability and Indemnification Agreements

As permitted by Delaware law, we have adopted provisions in our certificate of incorporation and bylaws, both of which will become effective upon the completion of this offering, that limit or eliminate the personal liability of directors for a breach of their fiduciary duty of care as a director. The duty of care generally requires that, when acting on behalf of the corporation, a director exercise an informed business judgment based on all material information reasonably available to him or her.

Consequently, a director will not be personally liable to us or our stockholders for monetary damages or breach of fiduciary duty as a director, except for liability for:

- any breach of the director's duty of loyalty to us or our stockholders;
- any act or omission not in good faith or that involves intentional misconduct or a knowing violation of law;
- any act related to unlawful stock repurchases, redemptions or other distributions or payments of dividends; or
- any transaction from which the director derived an improper personal benefit.

These limitations of liability do not limit or eliminate our rights or any stockholder's rights to seek non-monetary relief, such as injunctive relief or rescission. These provisions will not alter a director's liability under federal securities laws. Our certificate of incorporation that will become effective upon the completion of this offering also authorizes us to indemnify our officers, directors and other agents to the fullest extent permitted under Delaware law.

As permitted by Delaware law, our bylaws also provide that:

- we will indemnify our directors, officers, employees and other agents to the fullest extent permitted by law;
- we may advance expenses to our directors, officers, employees and other agents in connection with a legal proceeding to the fullest extent permitted by law; and
- the rights provided in our bylaws are not exclusive.

We believe that indemnification under our bylaws covers at least negligence and gross negligence on the part of indemnified parties. Our bylaws also permit us to secure insurance on behalf of any officer, director, employee or other agent for any liability arising out of his or her actions in connection with their services to us, regardless of whether our bylaws permit such indemnification. We have obtained such insurance.

In addition to the indemnification provided for in our certificate of incorporation and bylaws, we have entered, and intend to continue to enter, into separate indemnification agreements with each of our directors and executive officers which may be broader than the specific indemnification provisions contained in the Delaware General Corporation Law. These indemnification agreements may require us, among other things, to indemnify our directors and executive officers for some expenses, including attorneys' fees, judgments, fines and settlement amounts incurred by a director or executive officer in any action or proceeding arising out of his service as one of our directors or executive officers, or any of our subsidiaries or any other company or enterprise to which the person provides services at our request. We believe that these provisions and agreements are necessary to attract and retain qualified individuals to serve as directors and executive officers. There is no pending litigation or proceeding involving any of our directors or executive officers to which indemnification is required or permitted, and we are not aware of any threatened litigation or proceeding that may result in a claim for indemnification.

CERTAIN RELATIONSHIPS AND RELATED TRANSACTIONS

The following is a description of transactions since May 1999 to which we have been a party, in which the amount involved in the transaction exceeds $60,000, and in which any of our directors, executive officers or holders of more than five percent of our capital stock had or will have a direct or indirect material interest, other than the employment agreements described elsewhere.

Preferred Stock Issuances

We sold shares of our preferred stock in private financings as follows:

- 200,000 shares of our Series A preferred stock at a price of $7.275 per share in May 1999;
- 277,500 shares of our Series B preferred stock at a price of $18.018 per share in July 2000;
- 260,154 shares of our Series C preferred stock at a price of $57.658 per share in October 2001;
- 164,765 shares of our Series D preferred stock at a price of $66.762 per share in July 2002; and
- 329,536 shares of our Series E preferred stock at a price of $60.692 per share in August 2003.

Upon the closing of this offering, all of these shares of preferred stock and dividends accrued thereon through December 31, 2003 will convert into 11,456,905 shares of our common stock.

The investors in these financings included the following executive officers, directors, holders of more than five percent of our securities and the immediate family members and affiliated entities of each:

Investors	Series A Preferred Stock	Series B Preferred Stock	Series C Preferred Stock	Series D Preferred Stock	Series E Preferred Stock
Directors					
J.R. Hyde, III	200,000	277,500	77,718	74,894	283,777
John H. Pontius	—	—	—	—	1,648
Executive Officers					
Mark E. Mosteller	—	—	—	—	824
Henry P. Doggrell	—	—	—	—	1,236
Immediate Family Members					
Patricia B. Pontius	—	—	—	—	1,648
Kathryn K. Mosteller	—	—	—	—	824
Beverly R. Doggrell	—	—	—	—	412
5% Stockholders					
Entities affiliated with Oracle Partners, L.P.	—	—	173,436	74,894	16,478
Affiliated Entities					
Pittco Associates, L.P.(1)	—	—	9,000	—	—
Memphis Biomed Ventures I, L.P.(2)	—	—	—	14,977	16,477
Equity Partners XII, LLC(3)	—	—	—	—	6,212

(1) Pittco Associates, L.P. is affiliated with both Mr. Hyde and Mr. Pontius.

(2) Memphis Biomed Ventures I, L.P. is affiliated with Mr. Hyde.

(3) Mr. Hanover is the sole managing member of Equity Partners XII, LLC.

Registration Rights Agreements

We have entered into registration rights agreements with three of our preferred stockholders and their affiliates and transferees. Pursuant to the registration rights agreements, if we propose to register any of our securities under the Securities Act either for our own account or for the account of other security holders after this offering, as of December 31, 2003, the holders of registration rights will be entitled to include their 11,344,619 shares of common stock in the related registration statement. In addition, as of December 31, 2003, the holders of approximately 11,055,761 shares of common stock and their transferees may require us, on not more than two occasions from each holder of demand registration rights at any time after the closing of this offering, to file a registration statement under the Securities Act with respect to their shares of common stock. For more information concerning the registration rights agreements, please see "Description of Capital Stock — Registration Rights."

Indemnification Agreements

We have entered into separate indemnification agreements with our directors and executive officers, in addition to the indemnification provided for in our bylaws. See "Management — Limitations on Directors' Liability and Indemnification Agreements."

Transactions with Mr. Hyde

In July 2001, we borrowed $4.25 million from Mr. Hyde pursuant to the terms of a promissory note that bore interest at a rate of 9% per annum. All amounts due under the note were paid in full in October 2001. We paid Mr. Hyde $71,000 of interest in 2001. During 2003, we paid to Pittco, Inc., an affiliate of Mr. Hyde's, lease payments totaling $10,352 for the use of Pittco's airplane.

PRINCIPAL STOCKHOLDERS

The following table sets forth information as of December 31, 2003 regarding the beneficial ownership of our common stock by:

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

The number of shares owned and percentage ownership in the following table is based on 7,735,848 shares of common stock outstanding on December 31, 2003, the conversion of all outstanding shares of our preferred stock and the dividends accrued thereon through December 31, 2003 into 11,456,905 shares of common stock and the issuance of 5,400,000 shares in this offering. The information assumes no exercise of the underwriters' over-allotment option.

Each individual or entity shown on the table has furnished information with respect to beneficial ownership. Except as otherwise indicated below, the address of each officer, director and five percent stockholder listed below is c/o GTx, Inc., 3 N. Dunlap Street, 3rd Floor, Van Vleet Building, Memphis, Tennessee 38163.

We have determined beneficial ownership in accordance with the rules of the Securities and Exchange Commission. These rules generally attribute beneficial ownership of securities to persons who possess sole or shared voting power or investment power with respect to those securities. In addition, the rules include shares of common stock issuable pursuant to the exercise of stock options that are either immediately exercisable or exercisable within 60 days of December 31, 2003. These shares are deemed to be outstanding and beneficially owned by the person holding those options for the purpose of computing the percentage ownership of that person, but they are not treated as outstanding for the purpose of computing the percentage ownership of any other person. Unless otherwise indicated, the persons or entities identified in this table have sole voting and investment power with respect to all shares shown as beneficially owned by them.

Name and Address of Beneficial Owner	Number of Shares Owned	Percentage of Shares Outstanding	
		Before Offering	After Offering
5% Stockholders			
Entities affiliated with Oracle Partners, L.P.(1) 200 Greenwich Avenue Greenwich, CT 06830	2,616,923	13.6%	10.6%
Directors and Named Executive Officers			
J.R. Hyde, III(2)	9,483,346	49.4	38.6
Mitchell S. Steiner, M.D., F.A.C.S.(3)	5,662,147	29.5	23.0
Marc S. Hanover(4)	2,042,322	10.6	8.3
Mark E. Mosteller(5)	14,452	*	*
Henry P. Doggrell(6)	159,801	*	*
John H. Pontius(7)	970,536	5.1	3.9
Rosemary Mazanet, M.D., Ph.D.	—	—	—
All executive officers and directors as a group (7 persons)(8)	16,616,545	86.3	67.4

* Represents beneficial ownership of less than 1% of our outstanding common stock.

(1) Consists of 693,092 shares held by Oracle Partners, L.P., 1,750,552 shares held by Oracle Investment Management, Inc. and 173,279 shares held by Oracle Institutional Partners, L.P. Larry N. Feinberg is the managing member of the general partner of Oracle Partners, L.P. and Oracle Institutional Partners, L.P. and the President of Oracle Investment Management, Inc. Mr. Feinberg disclaims beneficial ownership of these shares except to the extent of his pecuniary interest in the named entities.

(2) Includes 90,915 shares held by Pittco Associates, L.P., an entity controlled by Mr. Hyde, 1,045,418 shares held by trusts with respect to which Mr. Hyde may be deemed to have beneficial ownership, 288,858 shares held by Memphis Biomed Ventures I, L.P., an entity controlled by Mr. Hyde, and 187,006 shares held by Mr. Hyde's wife, of which Mr. Hyde disclaims beneficial ownership.

(3) Includes 4,897,156 shares held by LD, Jr., LLC, an entity owned by Dr. Steiner and 764,991 shares held by trusts with respect to which Dr. Steiner may be deemed to have beneficial ownership.

(4) Includes 819,479 shares held by Equity Partners XII, LLC, an entity controlled by Mr. Hanover, and 921,221 shares held by trusts with respect to which Mr. Hanover may be deemed to have beneficial ownership.

(5) Includes 7,226 shares held by Mr. Mosteller's wife of which Mr. Mosteller disclaims beneficial ownership.

(6) Includes 96,090 shares held by trusts with respect to which Mr. Doggrell may be deemed to have beneficial ownership, 51,000 shares that Mr. Doggrell has the right to acquire within 60 days of December 31, 2003 through the exercise of stock options and 3,613 shares held by Mr. Doggrell's wife, of which Mr. Doggrell disclaims beneficial ownership.

(7) Includes 856,718 shares held by trusts of which Mr. Pontius is the trustee, 17,474 shares held by trusts of which Mr. Pontius' wife is the trustee and 48,172 shares beneficially owned by Mr. Pontius' wife. Mr. Pontius disclaims beneficial ownership of the shares held by trusts of which his wife is trustee and shares beneficially owned by her.

(8) Includes 51,000 shares that Mr. Doggrell has the right to acquire within 60 days of December 31, 2003 through the exercise of stock options. For purposes of determining the number of shares beneficially owned by directors and executive officers as a group, any shares beneficially owned by more than one director or officer are counted only once.

DESCRIPTION OF CAPITAL STOCK

The following description of our capital stock gives effect to the amendment and restatement of our certificate of incorporation and bylaws, which will occur upon the closing of this offering, and the conversion of our preferred stock and dividends accrued thereon through December 31, 2003 into 11,456,905 shares of common stock, which will occur upon the closing of this offering, as if such conversion had occurred on December 31, 2003.

Upon the closing of this offering, our authorized capital stock will consist of 60,000,000 shares of common stock, par value $0.001 per share, and 5,000,000 shares of preferred stock, par value $0.001 per share.

Common Stock

Outstanding Shares

As of December 31, 2003, we had 36 stockholders, 7,735,848 shares of common stock issued and outstanding and 1,231,955 shares of preferred stock issued and outstanding, which, together with dividends accrued thereon through December 31, 2003, are convertible into 11,456,905 shares of common stock. In addition, as of December 31, 2003, options to purchase 828,750 shares of common stock were issued and outstanding. Based on our outstanding capital stock as of December 31, 2003, upon completion of this offering, there will be 24,592,753 shares of common stock outstanding, assuming no exercise of the underwriters' over-allotment option or exercise of outstanding stock options.

Voting Rights

Each holder of our common stock is entitled to one vote for each share on all matters submitted to a vote of the stockholders, including the election of directors. Under our certificate of incorporation and bylaws, our stockholders will not have cumulative voting rights. Because of this, the holders of a majority of the shares of common stock entitled to vote in any election of directors can elect all of the directors standing for election, if they should so choose.

Dividends

Subject to preferences that may be applicable to any then outstanding preferred stock, holders of common stock are entitled to receive ratably those dividends, if any, as may be declared from time to time by the Board of Directors out of legally available funds.

Liquidation

In the event of our liquidation, dissolution or winding up, holders of common stock will be entitled to share ratably in the net assets legally available for distribution to stockholders after the payment of all of our debts and other liabilities and the satisfaction of any liquidation preference granted to the holders of any outstanding shares of preferred stock.

Rights and Preferences

Holders of common stock have no preemptive, conversion or subscription rights, and there are no redemption or sinking fund provisions applicable to the common stock. The rights, preferences and privileges of the 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 in the future.

Fully Paid and Nonassessable

All of our outstanding shares of common stock are, and the shares of common stock to be issued pursuant to this offering will be, fully paid and nonassessable.

Preferred Stock

Upon the closing of this offering, the Board of Directors will have the authority, without further action by the stockholders, to issue up to 5,000,000 shares of preferred stock in one or more series, to establish from time to time the number of shares to be included in each such series, to fix the rights, preferences and privileges of the shares of each wholly unissued series and any qualifications, limitations or restrictions thereon, and to increase or decrease the number of shares of any such series, but not below the number of shares of such series then outstanding. The Board of Directors may authorize the issuance of preferred stock with voting or conversion rights that could adversely affect the voting power or other rights of the holders of the common stock. The issuance of preferred stock, while providing flexibility in connection with possible acquisitions and other corporate purposes, could, among other things, have the effect of delaying, deferring or preventing a change in control of GTx and may adversely affect the market price of the common stock and the voting and other rights of the holders of common stock.

Registration Rights

Demand Registration Rights

As of December 31, 2003, at any time after the closing of this offering, the holders of 11,055,761 shares of our common stock and their transferees may require us, on not more than two occasions from each holder of demand rights, to file a registration statement under the Securities Act with respect to their shares of common stock, and we will be required to use our best efforts to effect the registration.

Piggyback Registration Rights

As of December 31, 2003, at any time after the closing of this offering, if we propose to register any of our securities under the Securities Act either for our own account or for the account of other stockholders, the holders of approximately 11,344,619 shares of common stock will be entitled to notice of the registration and will be entitled to include their shares of common stock in the registration statement. These registration rights are subject to specified conditions and limitations, including the right of the underwriters to limit the number of shares included in any such registration under some circumstances.

Expenses of Registration

We will pay all expenses relating to any demand or piggyback registration, other than underwriting discounts and commissions.

Expiration

These registration rights expire only upon the sale of all shares of common stock that have registration rights. However, we are not required to maintain the effectiveness of a registration statement if the shares of common stock included in such registration statement may be sold without restriction pursuant to Rule 144(k) under the Securities Act.

Delaware Anti-Takeover Law and Certain Provisions of our Certificate of Incorporation and Bylaws

Delaware Law

We are governed by Section 203 of the Delaware General Corporation Law. In general, Section 203 prohibits a public Delaware corporation from engaging in a "business combination" with an "interested stockholder" for a period of three years after the date of the transaction in which the person became an interested stockholder, unless the business combination is approved in a prescribed manner. A "business combination" includes mergers, asset sales or other transactions resulting in a financial benefit to the stockholder. An "interested stockholder" is a person who, together with affiliates and associates, owns, or within three years, did own, 15% or more of the corporation's outstanding voting stock. These provisions may have the effect of delaying, deferring or preventing a change in our control.

Certificate of Incorporation and Bylaw Provisions

Our certificate of incorporation and bylaws that will become effective upon the completion of this offering provide that our Board of Directors will be divided into three classes of directors, with each class serving a staggered three-year term. The classification system of electing directors may tend to discourage a third party from making a tender offer or otherwise attempting to obtain control of us and may maintain the composition of our current Board of Directors, as the classification of the Board of Directors generally increases the difficulty of replacing a majority of directors. In addition, our certificate of incorporation will:

- provide that any action required or permitted to be taken by our stockholders must be effected at a duly called annual or special meeting of stockholders and may not be effected by any consent in writing;
- provide that the authorized number of directors may be changed only by resolution of the Board of Directors; and
- eliminate cumulative voting for the election of directors.

In addition, our bylaws that will become effective upon completion of this offering will provide that special meetings of our stockholders may be called only by the chairman of the Board of Directors, our chief executive officer or by the Board of Directors pursuant to a resolution adopted by a majority of the directors then in office.

These and other provisions contained in our certificate of incorporation and bylaws could delay or discourage some types of transactions involving an actual or potential change in our control or change in our management, including transactions in which stockholders might otherwise receive a premium for their shares over then current prices, and may limit the ability of stockholders to remove current management or approve transactions that stockholders may deem to be in their best interests and, therefore, could adversely affect the price of our common stock.

Nasdaq National Market Listing

The common stock has been approved for quotation on the Nasdaq National Market under the symbol "GTXI".

Transfer Agent and Registrar

The Transfer Agent and Registrar for our common stock is EquiServe. The transfer agent's address is 525 Washington Blvd., P.O. Box 2533, Suite 4691, 9th Floor, Jersey City, NJ 07310.

SHARES ELIGIBLE FOR FUTURE SALE

Prior to this offering, there has been no public market for our common stock. Market sales of shares or the availability of shares for sale may decrease the market price of our common stock prevailing from time to time. As described below, only a portion of our outstanding shares of common stock will be available for sale shortly after this offering due to contractual and legal restrictions to resale. Nevertheless, sales of substantial amounts of common stock in the public market after these restrictions lapse, or the perception that such sales could occur, could adversely affect the market price of the common stock and could impair our future ability to raise capital through the sale of our equity securities.

Future sales of our common stock and the availability of our common stock for sale may depress the market price for our common stock. Upon completion of this offering, 24,592,753 shares of common stock will be outstanding, assuming no exercise of the underwriters' over-allotment option and no exercise of options. All of the 5,400,000 shares sold in this offering will be freely tradable. The remaining 19,192,753 shares of common stock, based on the number of shares outstanding as of December 31, 2003, are restricted as a result of securities laws or lock-up agreements. 16,302,695 of these shares will be available for sale in the public market 180 days after the date of this prospectus, subject to early release from lock-up agreements as described below. The remaining 2,890,058 shares will be available for sale in the public market between 180 and 365 days after the date of this prospectus. A portion of the restricted shares will be subject to volume limitations pursuant to Rule 144.

Rule 144

In general, under Rule 144 under the Securities Act of 1933, as currently in effect, a person who has beneficially owned shares of our common stock for at least one year would be entitled to sell within any three-month period a number of shares that does not exceed the greater of:

- one percent of the number of shares of our common stock then outstanding, which will equal 245,927 shares immediately after this offering; or
- the average weekly trading volume of our common stock on the Nasdaq National Market during the four calendar weeks preceding the filing of a notice on Form 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.

Rule 144(k)

Under Rule 144(k), a person who is not deemed to have been one of our affiliates at any time during the 90 days preceding a sale, and who has beneficially owned the shares proposed to be sold for at least two years, including the holding period of any prior owner other than an affiliate, is entitled to sell the shares without complying with the manner of sale, public information, volume limitation or notice provisions of Rule 144.

Rule 701

Rule 701, as currently in effect, permits resales of shares in reliance upon Rule 144 but without compliance with some restrictions of Rule 144, including the holding period requirement. Our employees, officers, directors or consultants who purchased shares under a written compensatory plan or contract may be entitled to rely on the resale provisions of Rule 701, but all holders of Rule 701 shares are required to wait until 90 days after the date of this prospectus before selling their shares.

Lock-Up Agreements

Each of our officers, directors and stockholders and the holders of substantially all of our outstanding options have agreed, subject to specified exceptions, that, without the prior written consent of Goldman, Sachs & Co., they will not, directly or indirectly, sell, offer, contract to sell, transfer the economic risk of ownership in, make any short sale, pledge or otherwise dispose of any shares of our capital stock or any securities convertible into or exchangeable or exercisable for or any other rights to purchase or acquire our capital stock for a period of 180 days from the date of this prospectus. Goldman, Sachs & Co. may, in its sole discretion, permit early release of shares subject to the lock-up agreements.

Registration Rights

Upon completion of this offering, the holders of 11,344,619 shares of our common stock, or their transferees, will be entitled to rights with respect to the registration of their shares under the Securities Act. Registration of these shares under the Securities Act would result in the shares becoming freely tradable without restriction under the Securities Act immediately upon the effectiveness of the registration. See “Description of Capital Stock — Registration Rights.”

Stock Options

Immediately after this offering, we intend to file with the SEC a registration statement under the Securities Act covering the 2,981,800 shares of common stock reserved for issuance under our stock option plans. The registration statement is expected to be filed and become effective as soon as practicable after the closing of this offering. Accordingly, shares registered under the registration statement will, subject to Rule 144 volume limitations applicable to affiliates and the lock-up agreements described above, be available for sale in the open market.

UNDERWRITING

GTx and the underwriters named below have entered into an underwriting agreement with respect to the shares being offered. Subject to certain conditions, each underwriter has severally agreed to purchase the number of shares indicated in the following table. Goldman, Sachs & Co., SG Cowen Securities Corporation and Lazard Frères & Co. LLC are the representatives of the underwriters.

Underwriters	Number of Shares
Goldman, Sachs & Co.	2,942,500
SG Cowen Securities Corporation	1,605,000
Lazard Frères & Co. LLC	802,500
Morgan Keegan & Company, Inc.	50,000
Total	5,400,000

The underwriters are committed to take and pay for all of the shares being offered, if any are taken, other than the shares covered by the option described below unless and until this option is exercised.

If the underwriters sell more shares than the total number set forth in the table above, the underwriters have an option to buy up to an additional 810,000 shares from GTx to cover such sales. They may exercise that option for 30 days. If any shares are purchased pursuant to this option, the underwriters will severally purchase shares in approximately the same proportion as set forth in the table above.

The following table shows the per share and total underwriting discounts and commissions to be paid to the underwriters by GTx. Such amounts are shown assuming both no exercise and full exercise of the underwriters' option to purchase 810,000 additional shares.

	Paid By GTx	
	No Exercise	Full Exercise
Per Share	$1.015	$1.015
Total	$5,481,000	$6,303,150

Shares sold by the underwriters to the public will initially be offered at the initial public offering price set forth on the cover of this prospectus. Any shares sold by the underwriters to securities dealers may be sold at a discount of up to $0.609 per share from the initial public offering price. Any such securities dealers may resell any shares purchased from the underwriters to certain other brokers or dealers at a discount of up to $0.100 per share from the initial public offering price. If all the shares are not sold at the initial public offering price, the representatives may change the offering price and the other selling terms.

GTx and its officers, directors and principal stockholders have agreed with the underwriters not to dispose of or hedge any of their common stock or securities convertible into or exchangeable for shares of common stock during the period from the date of this prospectus continuing through the date 180 days after the date of this prospectus, except with the prior written consent of Goldman, Sachs & Co. This agreement does not apply to the issuance of shares by GTx pursuant to any existing employee benefit plans. See "Shares Eligible for Future Sale" for a discussion of certain transfer restrictions.

At the request of GTx, the underwriters are reserving for sale, at the initial public offering price, to directors, officers, employees and friends through a directed share program up to 5% of the shares being offered. The number of shares available for sale to the general public in the offering will be reduced to the extent these persons purchase these reserved shares. Any reserved shares not so purchased will be offered to the general public on the same basis as the other shares offered under this prospectus.

Prior to the offering, there has been no public market for the shares. The initial public offering price has been negotiated among GTx and the representatives. Among the factors considered in determining the initial public offering price of the shares, in addition to prevailing market conditions, were GTx's historical performance, estimates of the business potential and earnings prospects of GTx, an assessment of GTx's management and the consideration of the above factors in relation to market valuation of companies in related businesses.

The common stock will be quoted on the Nasdaq National Market under the symbol "GTXI".

A prospectus in electronic format may be made available on a website maintained by one or more of the representatives of the underwriters and may also be made available on a website maintained by the other underwriter. 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 representatives of the underwriters to underwriters that may make Internet distributions on the same basis as other allocations.

In connection with the offering, the underwriters may purchase and sell shares of common stock in the open market. These transactions may include short sales, stabilizing transactions and purchases to cover positions created by short sales. Short sales involve the sale by the underwriters of a greater number of shares than they are required to purchase in the offering. "Covered" short sales are sales made in an amount not greater than the underwriters' option to purchase additional shares from GTx in the offering. The underwriters may close out any covered short position by either exercising their option to purchase additional shares or purchasing shares in the open market. In determining the source of shares to close out the covered short position, the underwriters will consider, among other things, the price of shares available for purchase in the open market as compared to the price at which they may purchase additional shares pursuant to the option granted to them. "Naked" short sales are any sales in excess of such option. 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 the offering. Stabilizing transactions consist of various bids for or purchases of common stock made by the underwriters in the open market prior to the completion of the offering.

The underwriters may also impose a penalty bid. This occurs when a particular underwriter repays to the underwriters a portion of the underwriting discount received by it because the representatives have repurchased shares sold by or for the account of such underwriter in stabilizing or short covering transactions.

Purchases to cover a short position and stabilizing transactions may have the effect of preventing or retarding a decline in the market price of GTx's stock, and together with the imposition of a penalty bid, may stabilize, maintain or otherwise affect the market price of the common stock. As a result, the price of the common stock may be higher than the price that otherwise might exist in the open market. If these activities are commenced, they may be discontinued at any time. These transactions may be effected on the Nasdaq National Market, in the over-the-counter market or otherwise.

Each underwriter has represented that: (1) it has not offered or sold and, prior to the expiry of a period of six months from the closing date, will not offer or sell any shares to persons in the United Kingdom except to persons whose ordinary activities involve them in acquiring, holding, managing or disposing of investments, as principal or agent, for the purposes of their businesses or otherwise in circumstances which have not resulted and will not result in an offer to the public in the United Kingdom within the meaning of the Public Offers of Securities Regulations 1995; (2) it has only communicated or caused to be communicated and will only communicate or cause to be communicated any invitation or inducement to engage in investment activity, within the meaning of section 21 Financial Services and Markets Act of 2000, or the FSMA, received by it in connection with the issue or sale of any shares in circumstances in which section 21(1) of the FSMA does not

apply to GTx; and (3) it has complied and will comply with all applicable provisions of the FSMA with respect to anything done by it in relation to the shares in, from or otherwise involving the United Kingdom.

The shares may not be offered or sold, transferred or delivered, as part of their initial distribution or at any time thereafter, directly or indirectly, to any individual or legal entity in the Netherlands other than to individuals or legal entities who or which trade or invest in securities in the conduct of their profession or trade, which includes banks, securities intermediaries, insurance companies, pension funds, other institutional investors and commercial enterprises which, as an ancillary activity, regularly trade or invest in securities.

No underwriter has offered or sold, or will offer or sell, in Hong Kong, by means of any document, any shares other than to persons whose ordinary business it is to buy or sell shares or debentures, whether as principal or agent, or under circumstances which do not constitute an offer to the public within the meaning of the Companies Ordinance (Cap. 32) of Hong Kong, nor has it issued or had in its possession for the purpose of issue, nor will it issue or have in its possession for the purpose of issue, any invitation or advertisement relating to the shares in Hong Kong (except as permitted by the securities laws of Hong Kong) other than with respect to shares which are intended to be disposed of to persons outside Hong Kong or to be disposed of only to persons whose business involves the acquisition, disposal, or holding of securities (whether as principal or as agent).

This prospectus has not been registered as a prospectus with the Monetary Authority of Singapore. Accordingly, this prospectus and any other document or material in connection with the offer or sale, or invitation or subscription or purchase, of the shares may not be circulated or distributed, nor may the shares be offered or sold, or be made the subject of an invitation for subscription or purchase, whether directly or indirectly, to persons in Singapore other than under circumstances in which such offer, sale or invitation does not constitute an offer or sale, or invitation for subscription or purchase, of the shares to the public in Singapore.

Each underwriter has acknowledged and agreed that the shares have not been registered under the Securities and Exchange Law of Japan and are not being offered or sold and may not be offered or sold, directly or indirectly, in Japan or to or for the account of any resident of Japan, except pursuant to an exemption from the registration requirements of the Securities and Exchange Law of Japan and in compliance with any other applicable requirements of Japanese law.

The underwriters do not expect sales to discretionary accounts to exceed five percent of the total number of shares offered.

GTx estimates that its share of the total expenses of the offering, excluding underwriting discounts and commissions, will be approximately $2.3 million.

GTx has agreed to indemnify the several underwriters against certain liabilities, including liabilities under the Securities Act of 1933.

Certain of the underwriters and their respective affiliates may in the future perform various financial advising and investment banking services for GTx, for which they may receive customary fees and expenses.

VALIDITY OF THE COMMON STOCK

The validity of the shares of common stock offered hereby and certain other legal matters will be passed upon for us by Cooley Godward LLP, Palo Alto, California. Certain legal matters will also be passed upon for us by Bass, Berry & Sims PLC, Memphis, Tennessee. Certain legal matters will be passed upon for the underwriters by Hale and Dorr LLP, Boston, Massachusetts.

EXPERTS

The financial statements of GTx, Inc. as of December 31, 2002 and 2001 and for each of the three years in the period ended December 31, 2002 appearing in this prospectus and registration statement, have been audited by Ernst & Young LLP, independent auditors, as set forth in their report thereon appearing elsewhere herein, and are included in reliance upon such report, given on the authority of such firm as experts in accounting and auditing.

WHERE YOU CAN FIND MORE INFORMATION

We have filed with the Securities and Exchange Commission, or SEC, a registration statement on Form S-1 under the Securities Act of 1933 with respect to the shares of common stock offered under this prospectus. This prospectus does not contain all of the information in the registration statement and the exhibits. For further information with respect to us and our common stock, we refer you to the registration statement and to the exhibits. Statements contained in this prospectus as to the contents of any contract or any other document referred to are not necessarily complete, and in each instance, we refer you to the copy of the contract or other document filed as an exhibit to the registration statement. Each of these statements is qualified in all respects by this reference.

You can read our SEC filings, including the registration statement, over the Internet at the SEC's web site at www.sec.gov. You may also read and copy any document we file with the SEC at its public reference facility at 450 Fifth Street, N.W., Washington, D.C. 20549. You may also obtain copies of the document at prescribed rates by writing to the Public Reference Section of the SEC at 450 Fifth Street, N.W., Washington, D.C. 20549. Please call the SEC at 1-800-SEC-0330 for further information on the operation of the public reference facility.

Upon completion 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 SEC. We also intend to furnish our stockholders with annual reports containing our financial statements audited by an independent public accounting firm and quarterly reports containing our unaudited financial information.

GTx, Inc.

(a development stage company)

INDEX TO FINANCIAL STATEMENTS

REPORT OF ERNST & YOUNG LLP, INDEPENDENT AUDITORS

Board of Directors and Stockholders

GTx, Inc.

We have audited the accompanying balance sheets of GTx, Inc. (a development stage company) as of December 31, 2002 and 2001, and the related statements of operations, cumulative redeemable convertible preferred stock and stockholders' equity (deficit) and cash flows for each of the three years in the period ended December 31, 2002. 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 auditing standards generally accepted in the United States. Those standards require that we plan and perform the audits to obtain reasonable assurance about whether the financial statements are free of material misstatement. An audit includes examining, on a test basis, evidence supporting the amounts and disclosures in the financial statements. An audit also includes assessing the accounting principles used and significant estimates made by management, as well as evaluating the overall financial statement presentation. We believe that our audits provide a reasonable basis for our opinion.

Since the date of completion of our audit of the accompanying financial statements and initial issuance of our report thereon dated May 9, 2003, which report contained an explanatory paragraph regarding the Company's ability to continue as a going concern, the Company, as discussed in the second paragraph of Note 2, has completed an issuance of preferred stock with net proceeds of approximately $20 million and plans, if additional funding efforts are unsuccessful, to reduce its cash expenditures such that it will continue its operations beyond December 31, 2004. Therefore, the conditions that raised substantial doubt about whether the Company will continue as a going concern no longer exist.

In our opinion, the financial statements referred to above present fairly, in all material respects, the financial position of GTx, Inc. (a development stage company) at December 31, 2002 and 2001, and the results of its operations and its cash flows for each of the three years in the period ended December 31, 2002, in conformity with accounting principles generally accepted in the United States.

/s/ Ernst & Young LLP

Memphis, Tennessee

May 9, 2003,
Except for Note 1, as to which the date is
December 1, 2003, and
except for Note 13, as to which the date is
January 14, 2004

GTx, Inc.

(a development stage company)

BALANCE SHEETS

(in thousands, except share data)

	December 31, 2001	December 31, 2002	September 30, 2003 (unaudited)	Pro Forma Stockholders' Equity at September 30, 2003 (unaudited)
ASSETS				
Current assets:				
Cash and cash equivalents	$ 8,834	$ 8,925	$ 19,788	
Acapodene inventory	154	—	121	
Prepaid expenses and other current assets	46	41	338	
Total current assets	9,034	8,966	20,247	
Property and equipment, net	1,083	1,064	860	
Total assets	$ 10,117	$ 10,030	$ 21,107	
LIABILITIES, CUMULATIVE CONVERTIBLE PREFERRED STOCK AND STOCKHOLDERS' EQUITY (DEFICIT)				
Current liabilities:				
Accounts payable	$ 261	$ 601	$ 747	
Accrued expenses	229	711	1,220	
Total current liabilities	490	1,312	1,967	
8% Cumulative Redeemable Convertible Preferred Stock, at redemption value:				
Series A, $0.001 par value; 200,000 shares authorized, issued and outstanding at all periods, liquidation value of $1,770 at December 31, 2001, $1,889 at December 31, 2002 and $1,983 at September 30, 2003 (unaudited)	11,847	13,855	25,691	
Series B, $0.001 par value; 277,500 shares authorized, issued and outstanding at all periods, liquidation value of $5,581 at December 31, 2001, $5,989 at December 31, 2002 and $6,313 at September 30, 2003 (unaudited)	16,581	19,671	36,882	
Series C, $0.001 par value; 450,000 shares authorized, 260,154 issued and outstanding at all periods, liquidation value of $15,274 at December 31, 2001, $16,496 at December 31, 2002 and $17,468 at September 30, 2003 (unaudited)	15,274	19,102	37,213	
Series D, $0.001 par value; 300,000 shares authorized, 164,765 issued and outstanding at December 31, 2002 and September 30, 2003, liquidation value of $0 at December 31, 2001, $11,398 at December 31, 2002 and $12,073 at September 30, 2003 (unaudited)	—	11,398	22,238	
Series E, $0.001 par value; 450,000 shares authorized, 329,536 issued and outstanding at September 30, 2003, liquidation value of $0 at December 31, 2001 and December 31, 2002 and $20,236 at September 30, 2003 (unaudited)	—	—	40,954	
Total cumulative redeemable convertible preferred stock	43,702	64,026	162,978	
Stockholders' equity (deficit):				
Common stock, $0.001 par value: 25,000,000 shares authorized; 7,735,000 shares issued and outstanding at December 31, 2001 and 2002 and September 30, 2003 (unaudited); 19,031,780 shares outstanding on a pro forma basis (unaudited)	8	8	8	19
Deferred stock compensation	—	—	(3,408)	(3,408)
Additional paid-in capital	962	962	4,453	167,689
Deficit accumulated during the development stage	(35,045)	(56,278)	(144,891)	(145,160)
Total stockholders' (deficit) equity	(34,075)	(55,308)	(143,838)	$ 19,140
Total liabilities and stockholders' deficit	$ 10,117	$ 10,030	$ 21,107	

The accompanying notes are an integral part of these financial statements.

GTx, Inc.

(a development stage company)

STATEMENTS OF OPERATIONS

(in thousands, except share and per share data)

	Year Ended December 31,			Nine Months Ended September 30,		Cumulative Period from September 24, 1997 (date of inception) to September 30, 2003
	2000	2001	2002	2002	2003	
				(unaudited)	(unaudited)	(unaudited)
Operating expenses:						
Research and development	$ 2,679	$ 5,744	$ 9,285	$ 6,408	$ 7,123	$ 25,534
General and administrative	1,203	2,187	2,405	1,830	2,339	8,569
Depreciation	80	215	332	240	264	955
Total operating expenses	3,962	8,146	12,022	8,478	9,726	35,058
Other income:						
Research and development income	—	—	—	—	—	225
Interest income	150	83	156	105	79	579
Total other income	150	83	156	105	79	804
Net loss	(3,812)	(8,063)	(11,866)	(8,373)	(9,647)	(34,254)
Accrued preferred stock dividends	(297)	(790)	(2,147)	(1,466)	(2,300)	(5,617)
Adjustments to preferred stock redemption value	(21,077)	(57)	(7,220)	(7,147)	(76,666)	(105,020)
Net loss attributable to common stockholders	$ (25,186)	$ (8,910)	$ (21,233)	$ (16,986)	$ (88,613)	$(144,891)
Net loss per share attributable to common stockholders, basic and diluted:	$ (3.26)	$ (1.15)	$ (2.75)	$ (2.20)	$ (11.46)	
Weighted average shares used in computing net loss per share attributable to common stockholders, basic and diluted	7,735,000	7,735,000	7,735,000	7,735,000	7,735,000	
Pro forma net loss per share attributable to common stockholders, basic and diluted (unaudited)			$ (0.80)		$ (0.59)	
Shares used in computing pro forma net loss per share attributable to common stockholders, basic and diluted (unaudited)			14,811,786		16,455,728	

The accompanying notes are an integral part of these financial statements.

GTx, Inc.

(a development stage company)

STATEMENTS OF CUMULATIVE REDEEMABLE CONVERTIBLE PREFERRED STOCK AND

STOCKHOLDERS' EQUITY (DEFICIT)
For the Period From September 24, 1997 (date of inception) To September 30, 2003
(in thousands, except share and per share data)

	Cumulative Redeemable Convertible Preferred Stock		Stockholders' Equity (Deficit)					
			Common Stock		Deferred Stock Compensation	Additional Paid-in Capital	Deficit Accumulated During the Development Stage	Total Stockholders' Equity (Deficit)
	Shares	Amount	Shares	Amount				
Balances at September 24, 1997	—	$ —	—	$ —	$ —	$ —	$ —	$ —
Issuance of common stock	—	—	7,650,000	—	—	—	—	—
Net loss	—	—	—	—	—	—	—	—
Balances at December 31, 1997	—	—	7,650,000	—	—	—	—	—
Issuance of common stock	—	—	850,000	8	—	962	—	970
Net loss	—	—	—	—	—	—	(116)	(116)
Balances at December 31, 1998	—	—	8,500,000	8	—	962	(116)	854
Sale of Series A Redeemable Convertible Preferred Stock at $7.275	200,000	1,455	—	—	—	—	—	—
Preferred stock dividends	—	83	—	—	—	—	(83)	(83)
Net loss	—	—	—	—	—	—	(750)	(750)
Balances at December 31, 1999	200,000	1,538	8,500,000	8	—	962	(949)	21
Sale of Series B Redeemable Convertible Preferred Stock at $18.018	277,500	5,000	—	—	—	—	—	—
Preferred stock dividends	—	297	—	—	—	—	(297)	(297)
Preferred stock adjustment to redemption value	—	21,077	—	—	—	—	(21,077)	(21,077)
Common stock redemption	—	—	765,000	—	—	—	—	—
Net loss	—	—	—	—	—	—	(3,812)	(3,812)
Balances at December 31, 2000	477,500	27,912	7,735,000	8	—	962	(26,135)	(25,165)
Sale of Series C Redeemable Convertible Preferred Stock at $57.658, net of issuance costs of $57	260,154	14,943	—	—	—	—	—	—
Preferred stock dividends	—	790	—	—	—	—	(790)	(790)
Preferred stock adjustment to redemption value	—	57	—	—	—	—	(57)	(57)
Net loss	—	—	—	—	—	—	(8,063)	(8,063)
Balances at December 31, 2001	737,654	43,702	7,735,000	8	—	962	(35,045)	(34,075)
Sale of Series D	164,765	10,957	—	—	—	—	—	—

Redeemable Convertible Preferred Stock at $66.762, net of issuance costs of $43								
Preferred stock dividends	—	2,147	—	—	—	—	(2,147)	(2,147)
Preferred stock adjustment to redemption value	—	7,220	—	—	—	—	(7,220)	(7,220)
Net loss	—	—	—	—	—	—	(11,866)	(11,866)
Balances at December 31, 2002	902,419	64,026	7,735,000	8	—	962	(56,278)	(55,308)
Sale of Series E Redeemable Convertible Preferred Stock at $60.692, net of issuance costs of $14	329,536	19,986	—	—	—	—	—	—
Preferred stock dividends	—	2,300	—	—	—	—	(2,300)	(2,300)
Preferred stock adjustment to redemption value	—	76,666	—	—	—	—	(76,666)	(76,666)
Deferred stock- based compensation	—	—	—	—	(3,491)	3,491	—	—
Amortization of stock-based compensation	—	—	—	—	83	—	—	83
Net loss	—	—	—	—	—	—	(9,647)	(9,647)
Balances at September 30, 2003 (unaudited)	1,231,955	$162,978	7,735,000	$ 8	$(3,408)	$4,453	$(144,891)	$(143,838)

The accompanying notes are an integral part of these financial statements.

GTx, Inc.

(a development stage company)

STATEMENTS OF CASH FLOWS

(in thousands)

	Year Ended December 31,			Nine Months Ended September 30,		Cumulative Period from September 24, 1997 (date of inception) to September 30, 2003
	2000	2001	2002	2002	2003	
				(unaudited)	(unaudited)	(unaudited)
Cash flows from operating activities:						
Net loss	$ (3,812)	$ (8,063)	$ (11,866)	$ (8,373)	$ (9,647)	$ (34,254)
Adjustments to reconcile net loss to net cash used in operating activities:						
Depreciation	80	215	332	240	264	955
Stock-based compensation expense	—	—	—	—	83	83
Changes in assets and liabilities:						
Acapodene inventory	—	(154)	154	—	(121)	(121)
Prepaid expenses and other assets	(16)	(18)	5	8	(297)	(338)
Accounts payable	(5)	261	340	89	146	747
Accrued expenses	340	(225)	482	537	509	1,220
Net cash used in operating activities	(3,413)	(7,984)	(10,553)	(7,499)	(9,063)	(31,708)
Cash flows from investing activities:						
Purchase of property and equipment	(462)	(792)	(313)	(295)	(60)	(1,815)
Net cash used in investing activities	(462)	(792)	(313)	(295)	(60)	(1,815)
Cash flows from financing activities:						
Proceeds from issuance of notes payable — related party	—	4,250	—	—	—	4,250
Payment of notes payable — related party	—	(4,250)	—	—	—	(4,250)
Proceeds from issuance of common stock	—	—	—	—	—	970
Proceeds from issuance of preferred stock, net	5,000	14,943	10,957	10,957	19,986	52,341
Net cash provided by financing activities	5,000	14,943	10,957	10,957	19,986	53,311
Net increase (decrease) in cash and cash equivalents	1,125	6,167	91	3,163	10,863	19,788
Cash and cash equivalents, beginning of period	1,542	2,667	8,834	8,834	8,925	—
Cash and cash equivalents, end of period	$ 2,667	$ 8,834	$ 8,925	$11,997	$19,788	$ 19,788
Supplemental schedule of non-cash investing and financing activities:						
Preferred stock dividends	$ 297	$ 790	$ 2,147	$ 1,466	$ 2,300	$ 5,617
Preferred stock adjustment to redemption value	$21,077	$ 57	$ 7,220	$ 7,147	$76,666	$105,020

The accompanying notes are an integral part of these financial statements.

GTx, Inc.

(a development stage company)

NOTES TO FINANCIAL STATEMENTS

(in thousands, except share and per share data)

1. Organization

GTx, Inc. (the "Company") is a biopharmaceutical company dedicated to the discovery, development and commercialization of therapeutics primarily related to the treatment of serious men's health conditions. The Company's drug discovery and development programs are focused on small molecules that selectively modulate the effects of estrogens and androgens, two essential classes of hormones. The Company currently has two product candidates that are in human clinical trials. The Company is developing Acapodene, its most advanced product candidate, through clinical trials for two separate indications: (1) a Phase IIb clinical trial for the reduction in the incidence of prostate cancer in men with precancerous prostate lesions and (2) a pivotal Phase III clinical trial for the treatment of serious side effects of advanced prostate cancer therapy. The Company is initially developing its second product candidate, Andarine, for the treatment of cachexia from various types of cancer. Andarine is the most advanced of its internally discovered portfolio of compounds designed to modulate the effects of hormones. The Company plans to build a specialized sales and marketing capability to market its product candidates directly to the relatively small and concentrated community of urologists and medical oncologists in the United States and seek collaborators to commercialize its product candidates where the target physician market is broader than urologists and medical oncologists and outside the United States.

The Company was incorporated in Tennessee on September 24, 1997. On September 4, 2003, the Company formed a wholly-owned subsidiary in the State of Delaware with 25,000,000 authorized shares of common stock with a par value of $0.001 per share and 1,975,000 shares of preferred stock with a par value of $0.001 per share. On December 1, 2003, the Company was merged into the subsidiary to effect a reincorporation in Delaware. The financial statements reflect the capital structure of the Delaware subsidiary from the Company's inception. From its inception through the merger with the Company, the Delaware subsidiary had no assets or liabilities.

2. Significant Accounting Policies

Basis of Presentation

From September 24, 1997 (inception) through December 31, 2002, the Company has been primarily engaged in research and development, clinical development, and raising capital and is still in a development stage. The Company operates as one business segment.

The accompanying financial statements have been prepared on a going concern basis, which contemplates the realization of assets and the satisfaction of liabilities in the normal course of business. The Company has experienced negative cash flows from operations since inception and had an accumulated deficit at December 31, 2002 and September 30, 2003 (unaudited) of approximately $56,278 and $144,891, respectively. The Company's accumulated deficit at September 30, 2003 (unaudited) resulted primarily from funding its operating losses as well as non-cash dividends and adjustments to preferred stock redemption value of $110,637. The Company has funded its activities to date almost exclusively from debt and equity financings. In August 2003, the Company issued additional preferred stock (see Note 13) for proceeds of approximately $20,000. The Company will continue to require substantial funds to continue research and development, including preclinical studies and clinical trials of its product candidates, and to commence sales and marketing efforts, if the FDA or other regulatory approvals are obtained. Management's plans in order to meet its operating cash flow requirements include an initial public offering of its common stock, as well as entering into research collaborations through licensing opportunities, which will provide funding for certain research projects. While the Company believes that it will be successful in

GTx, Inc.
(a development stage company)

NOTES TO FINANCIAL STATEMENTS — (Continued)

obtaining the necessary financing to fund its operations, there are no assurances that such additional funding will be achieved. In that event, the Company has the intent and ability to reduce its cash expenditures by delaying its initiation of certain research and development efforts such that it will continue its operations beyond December 31, 2004.

Unaudited Interim Financial Information

The interim financial statements for the nine months ended September 30, 2002 and September 30, 2003 and the cumulative period from September 24, 1997 to September 30, 2003, together with the related notes, are unaudited and have been prepared on the same basis as the annual financial statements. In the opinion of management, all adjustments, consisting of normal recurring adjustments necessary for the fair presentation of the financial statements, have been included. The results of operations of any interim period are not necessarily indicative of the results of operations for the full year or any other interim period.

Use of Estimates

The preparation of financial statements in conformity with accounting principles generally accepted in the United States 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 period. Actual amounts and results could differ from those estimates.

Preferred Stock Redemption Value

In connection with the public filing for an initial registration of its common stock, the Company changed its accounting policy to recognize changes in the redemption value of its preferred stock immediately as they occur and adjust the carrying value of the preferred stock to equal the redemption value at the end of each reporting period. Previously, the Company had adjusted the carrying value of its preferred stock to its liquidation value at the end of each reporting period.

The preferred stock is subject to redemption on or after August 31, 2006, at a price per share equal to the greater of the liquidation value, which includes accrued dividends, or the fair value calculated on an as-if converted to common stock basis. The Company determines redemption value (fair value) considering factors such as the share price of preferred stock issuances, achievement of significant milestones in clinical trials and general market conditions. The changes in redemption value affect the loss attributable to common stockholders.

Cash and Cash Equivalents

The Company considers highly liquid investments with initial maturities of three months or less to be cash equivalents.

Acapodene Inventory

Acapodene inventory consists of a drug that is manufactured by a third-party and delivered to the Company as a finished good. Inventories are stated at the lower of cost (first-in, first-out method) or market. The inventory is expensed by the Company at the time it is sent to clinical trial facilities.

GTx, Inc.
(a development stage company)

NOTES TO FINANCIAL STATEMENTS — (Continued)

Property and Equipment

Property and equipment is recorded at cost. Depreciation of equipment and furniture and fixtures is computed based on the straight-line method over estimated useful lives of three to five years. Amortization of leasehold improvements is recognized over the shorter of the lease term or the estimated useful life of the leasehold improvement.

Impairment

The Company accounts for long-lived assets in accordance with Statement of Financial Accounting Standards ("SFAS") No. 144, *Accounting for the Impairment or Disposal of Long-Lived Assets and for Long-Lived Assets to be Disposed of*, which requires that companies consider whether events or changes in facts and circumstances, both internally and externally, may indicate that an impairment of long-lived assets held for use are present. Management periodically evaluates the carrying value of long-lived assets and has determined that there was no impairment as of December 31, 2001 and December 31, 2002. Should there be impairment in the future, the Company would recognize the amount of the impairment based on the expected future cash flows from the impaired assets. The cash flow estimates would be based on management's best estimates, using appropriate and customary assumptions and projections at the time.

Fair Value of Financial Instruments

Financial instruments consist of cash and cash equivalents, accounts payable and preferred stock. The carrying values of cash and cash equivalents and accounts payable approximate the fair value due to the short-term nature of such instruments. Preferred stock is carried at redemption value which approximates fair value.

Concentration of Risks

Financial instruments that potentially subject the Company to a concentration of credit risk consist of cash and cash equivalents. The Company maintains its cash and cash equivalents in accounts with several major financial institutions in the United States. Deposits in these institutions may exceed the amount of insurance provided on such deposits. The amounts in excess of FDIC insurance amounts are $8,734 and $8,625 at December 31, 2001 and December 31, 2002, respectively.

The Company faces competition from established pharmaceutical and biotechnology companies, as well as from academic institutions, government agencies and private and public research institutions. Various products are currently marketed or sold and used off-label for some of the diseases and conditions that the Company is targeting, and a number of companies are or may be developing new treatments. In addition, physicians are permitted to prescribe legally available drugs for uses that are not described in the drug's labeling and that differ from those uses tested and approved by the FDA. Such off-label uses are common across medical specialties. The occurrence of such off-label uses could significantly reduce the Company's ability to market and sell any products that it may develop.

Currently, the Company relies on Orion Corporation as a single source supplier for Acapodene, and the Company is currently purchasing Andarine from ChemSyn Laboratories, a department of EaglePicher Technologies, LLC, as a single supplier. Establishing additional or replacement suppliers for Acapodene or Andarine may take a substantial amount of time, and in some circumstances the Company's agreement with Orion may prevent it from obtaining an alternate supplier with respect to

GTx, Inc.
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NOTES TO FINANCIAL STATEMENTS — (Continued)

Acapodene. If the Company has to switch to a replacement supplier, the Company may face additional regulatory delays, and the manufacture and delivery of Acapodene or Andarine could be interrupted for an extended period of time, which may delay completion of the Company's clinical trials or commercialization of Acapodene or Andarine. If the Company is unable to obtain an adequate supply of Acapodene or Andarine, its clinical trials will be delayed. As a result, regulatory approval of Acapodene or Andarine could be delayed, or may not be received at all.

Research and Development Costs

The Company expenses research and development costs in the period in which they are incurred. These costs consist of direct and indirect costs associated with specific projects as well as fees paid to various entities that perform research and clinical trial studies on behalf of the Company.

Patent Costs

The Company expenses patent costs, including legal expenses, in the period in which they are incurred. Patent expenses are included in general and administrative expenses in the Company's statements of operations.

Income Taxes

The Company accounts for deferred taxes by recognition of deferred tax assets and liabilities for the expected future tax consequences of events that have been included in the financial statements or tax returns. Under this method, deferred tax assets and liabilities are determined based on the difference between the financial statement and tax bases of assets and liabilities using enacted tax rates in effect for the year in which the differences are expected to reverse.

Research and Development Income

Indigo, a Johnson & Johnson subsidiary, and Johnson & Johnson Development Corporation, ("JJDC") entered into an option agreement with the Company on March 9, 1998. The option agreement was established to allow Indigo and JJDC to determine their level of interest in establishing an exclusive worldwide license with respect to the Company's gene therapy products and related technology. The agreement required the Company during the period of the agreement, which ended in June 1998, to not negotiate with other third parties related to gene therapy products and related technology. Upon expiration of the option, the Company recognized research and development income of $225 for the option proceeds. The Company is no longer pursuing any research and development related to gene therapy products or technology.

Stock Compensation

Accounting Principles Board Opinion No. 25, *Accounting for Stock Issued to Employees* ("APB No. 25"), and its related interpretations are applied to measure compensation expense for stock-based compensation plans. The Company complies with the disclosure provisions of Statement of Financial Accounting Standards No. 123., *Accounting for Stock-Based Compensation* ("SFAS No. 123"), as amended by SFAS No. 148, *Accounting for Stock-Based Compensation, Transition and Disclosure.* Under APB No. 25, unearned stock compensation is based on the difference, if any, on the date of grant, between the fair value of the Company's common stock and the exercise price. See Note 11 for a description of the plans and the assumptions underlying the pro forma calculations below.

GTx, Inc.
(a development stage company)

NOTES TO FINANCIAL STATEMENTS — (Continued)

If compensation cost for stock-based compensation plans had been determined under SFAS 123, pro forma stock option compensation expense and net loss attributable to common stockholders, assuming all options were valued on the date of grant using the minimum value option pricing model, would have been as follows:

	Years Ended December 31,			Nine Months Ended September 30,	
	2000	2001	2002	2002	2003
				(unaudited)	
Net loss attributable to common stockholders, as reported	$(25,186)	$(8,910)	$(21,233)	$(16,986)	$(88,613)
Add: Employee stock-based compensation expense included in reported net earnings	—	—	—	—	83
Deduct: Employee stock-based compensation determined under fair value method	(4)	(37)	(115)	(84)	(190)
Adjusted net loss attributable to common stockholders	$(25,190)	$(8,947)	$(21,348)	$(17,070)	$(88,720)
Pro forma SFAS 123 disclosure:					
Net loss attributable to common stockholders per common share:					
As reported, basic and diluted	$ (3.26)	$ (1.15)	$ (2.75)	$ (2.20)	$ (11.46)
As adjusted, basic and diluted	$ (3.26)	$ (1.16)	$ (2.76)	$ (2.21)	$ (11.47)

Net Loss Per Share

Basic net loss per share attributable to common stockholders is calculated based on the weighted average number of common shares outstanding during the period. Diluted net loss per share attributable to common stockholders would give effect to the dilutive effect of potential common stock consisting of stock options and convertible preferred stock.

The 765,000 common shares that were redeemed in 2000 were excluded from the weighted average common shares outstanding because the shares were contingently returnable to the Company if the holder’s employment terminated prior to a certain date. These shares were treated as stock options in the earnings per share calculation.

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NOTES TO FINANCIAL STATEMENTS — (Continued)

A reconciliation of shares used in the calculation is as follows:

	Years Ended December 31,			Nine Months Ended September 30,	
	2000	2001	2002	2002	2003
				(unaudited)	
Basic net loss per share attributable to common shareholders:					
Numerator					
Net loss attributable to common stockholders	$ (25,186)	$ (8,910)	$ (21,233)	$ (16,986)	$ (88,613)
Denominator					
Weighted average common shares outstanding	7,735,000	7,735,000	7,735,000	7,735,000	7,735,000
Net loss per share attributable to common stockholders, basic and diluted	$ (3.26)	$ (1.15)	$ (2.75)	$ (2.20)	$ (11.46)
Pro Forma					
Net loss as reported			$ (11,866)		$ (9,647)
Shares used above			7,735,000		7,735,000
Pro forma adjustments to reflect assumed weighted average effect of conversion of preferred stock (unaudited)			7,076,786		8,720,728
Shares used in computing pro forma net loss per share attributable to common stockholders, basic and diluted (unaudited)			14,811,786		16,455,728
Pro forma basic and diluted net loss per share (unaudited)			$ (0.80)		$ (0.59)

Pro forma net loss per share for the year ended December 31, 2002 and the nine months ended September 30, 2003 is computed using the weighted average number of shares of common stock outstanding, including the pro forma effects of the automatic conversion of the Company's preferred stock into shares of common stock effective upon the closing of the offering as if such conversion occurred on January 1, 2002 and January 1, 2003 or at the date of the original issuance, if later. The resulting pro forma adjustments include an increase in the weighted average shares used to compute basic and diluted net loss per share attributable to common stockholders of 7,076,786 shares and 8,720,728 shares for the year ended December 31, 2002 and for the nine months ended September 30, 2003, respectively. The calculation of pro forma net loss per share attributable to common stockholders excludes incremental common stock issuable upon exercise of options, as their effect would be antidilutive.

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NOTES TO FINANCIAL STATEMENTS — (Continued)

The following outstanding stock options and convertible preferred stock (on an as converted to common stock basis) were excluded from the computation of diluted net loss per share attributable to common stockholders as they had an antidilutive effect:

	Years Ended December 31,			Nine Months Ended September 30,	
	2000	2001	2002	2002	2003
				(unaudited)	
Shares issuable upon exercise of stock options	133,875	328,100	363,375	363,375	799,000
Shares issuable upon conversion of convertible preferred stock	4,114,765	6,442,660	8,151,679	8,055,663	11,296,780
	4,248,640	6,770,760	8,515,054	8,419,038	12,095,780

Comprehensive Loss

The Company has adopted the provisions of SFAS No. 130, *Comprehensive Income*. SFAS 130 establishes standards for the reporting and display of comprehensive income and its components for general purpose financial statements. For all periods presented, there were no differences between net loss and comprehensive loss.

Recent Accounting Pronouncements

In December 2002, the FASB issued SFAS No. 148, which provides alternative methods of transition for a voluntary change to the fair value based method of accounting for stock-based employee compensation. SFAS No. 148 also requires that disclosures of the pro forma effect of using the fair value method of accounting for stock-based employee compensation be displayed more prominently and in a tabular format. Additionally, SFAS No. 148 requires disclosure of the pro forma effect in interim financial statements. The transition and annual disclosure requirements of SFAS No. 148 are effective for fiscal years ended after December 15, 2002. The interim disclosure requirements are effective for interim periods beginning after December 15, 2002. The adoption of this standard did not have a material impact on the Company's financial statements.

In January 2003, the FASB issued FASB Interpretation No. 46 ("FIN 46"), *Consolidation of Variable Interest Entities, an Interpretation of ARB No. 51*. FIN 46 requires certain variable interest entities to be consolidated by the primary beneficiary of the entity if the equity investors in the entity do not have the characteristics of a controlling financial interest or do not have sufficient equity at risk for the entity to finance its activities without additional subordinated financial support from other parties. FIN 46 is effective immediately for all new variable interest entities created or acquired after January 31, 2003. For variable interest entities created or acquired prior to February 1, 2003, the provisions of FIN 46 must be applied for the first interim or annual period ending after December 15, 2003. The Company does not have any ownership in any variable interest entities as of December 31, 2002. The Company will apply the consolidation requirement of FIN 46 in future periods if it should own any interest in a variable interest entity.

In May 2003, the FASB issued SFAS No. 150, *Accounting for Certain Financial Instruments with Characteristics of both Liabilities and Equity*. SFAS No. 150 establishes standards for how an issuer classifies and measures certain financial instruments with characteristics of both liability and equity. SFAS No. 150 is effective for the Company's financial instruments entered into or modified after May 31, 2003, and otherwise is effective on July 1, 2003. The Company has evaluated the

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NOTES TO FINANCIAL STATEMENTS — (Continued)

impact of SFAS No. 150 and has determined that its financial instruments (common stock and preferred stock) will not be affected unless the terms of these financial instruments are modified.

3. Property and Equipment

Property and equipment consist of the following:

	December 31,	
	2001	2002
Leasehold improvements	$ 108	$ 113
Equipment	1,198	1,494
Furniture and fixtures	102	114
	1,408	1,721
Less: accumulated depreciation and amortization	325	657
	$1,083	$1,064

Depreciation expense for the years ended December 31, 2000, 2001, and 2002 was $80, $215, and $332, respectively.

4. Accrued Expenses

Accrued expenses consist of the following:

	December 31,	
	2001	2002
Travel	$ 27	$ –
Professional fees	109	–
Research and development	71	246
Clinical trial	14	449
Other	8	16
	$229	$711

5. Cumulative Redeemable Convertible Preferred Stock

In 1999, the Company authorized and issued 200,000 shares of 8% Series A Cumulative Redeemable Convertible Preferred Stock (“Series A”) to a common stockholder of the Company for $1,455. In 2000, the Company authorized and issued 277,500 shares of 8% Series B Cumulative Convertible Redeemable Preferred Stock (“Series B”) to the same common stockholder of the Company for $5,000. In 2001, the Company authorized 450,000 shares and issued 260,154 shares (86,718 shares were issued to a common and preferred stockholder of the Company) of 8% Series C Cumulative Convertible Redeemable Preferred Stock (“Series C”) for $14,943. In 2002, the Company authorized 300,000 and issued 164,765 shares (74,894 shares were issued to a common and preferred stockholder of the Company) of 8% Series D Cumulative Convertible Redeemable Preferred Stock (“Series D”) for $10,957.

The Company is authorized to issue 140,000 shares of a series of preferred stock designated as Series A-2 Convertible Preferred Stock (“Series A-2”). No shares of the Series A-2 are currently outstanding. Series A-2 will be issued only in payment of dividends accrued on Series A.

GTx, Inc.
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NOTES TO FINANCIAL STATEMENTS — (Continued)

The Company is authorized to issue 157,500 shares of a series of preferred stock designated as Series B-2 Convertible Preferred Stock ("Series B-2"). No shares of Series B-2 are currently outstanding. Series B-2 will be issued only in payment of dividends accrued on Series B.

Significant terms of the Series A, Series A-2, Series B, Series B-2, Series C and Series D are as follows:

- Shares of Series A, Series A-2, Series B, Series B-2, Series C and Series D shall be redeemed at the election of the respective holders at any time on or after August 31, 2006 at a price per share equal to the greater of the liquidation value, which includes accrued dividends, or the fair value calculated on an if converted to common stock basis. The per share liquidation value of Series A and Series A-2 is $7.28, Series B and Series B-2 is $18.02, Series C is $57.66 and Series D is $66.76, in each case, plus accrued dividends. The preferred stock per share redemption value was $57.66, $57.66, $66.76, $66.76 and $122.83 as of December 31, 2000, 2001 and 2002 and as of September 30, 2002 and 2003 (unaudited), respectively. If for any reason, the Company defaults on its obligation to pay all or any of the redemption price, then the unpaid principal portion will bear interest at a rate of 14% per year. The default provisions were amended upon the issuance of the Series E Cumulative Convertible Redeemable Preferred Stock ("Series E") (see Note 13).

- Shares of Series A, Series A-2, Series B, Series B-2, Series C and Series D shall be converted into shares of common stock at the election of the respective holders at any time or automatically upon the closing of a Qualified Public Offering. As defined in the Company's Certificate of Incorporation, a Qualified Public Offering is an offering to the public of common stock or convertible securities in which (i) the net proceeds to the Company are not less than $25,000 and (ii) the price per share of common stock, or common stock equivalent in the case of convertible securities, is not less than $13.57 (adjusted for stock splits, stock dividends and other similar changes to the common stock) (see Note 13). The number of shares issuable upon conversion will be determined by dividing the applicable aggregate liquidation value by the applicable conversion price. As a result of the issuance of Series E in August 2003, the conversion price of the Series D was reduced to $7.75 per share (see Note 13). The per share conversion prices for shares of preferred stock are as follows: Series A-$0.86, Series B-$2.12, Series C, A-2 and B-2-$6.78, Series D-$7.75 and Series E-$7.14 as a result of the stock split in January 2004 (see Note 13).

- Shares of Series A, Series B, Series C and Series D have voting rights equivalent to the number of shares of common stock into which they are convertible.

- Dividends on shares of Series A, Series B, Series C and Series D accrue, compound annually after the date of issuance of Series C, which was October 5, 2001, are cumulative at the annual rate of 8% of the respective liquidation value and are payable at such time as such shares are converted or redeemed (including liquidation). Each such dividend will be payable solely in shares of Series A-2 for Series A, Series B-2 for Series B, Series C for Series C and Series D for Series D at the time of conversion or redemption with the number of shares determined by dividing the amount of accrued dividends by the per share liquidation value of the applicable preferred stock.

- In the event of a liquidation, dissolution, or winding up of the Company, prior to the holders of common stock, the holders of Series A, Series A-2, Series B, Series B-2, Series C and Series D shall receive an amount equal to the aggregate liquidation value including all accrued dividends. If the funds available for distribution to the holders of Series A,

GTx, Inc.
(a development stage company)

NOTES TO FINANCIAL STATEMENTS — (Continued)

Series A-2, Series B, Series B-2, Series C or Series D are insufficient, then the assets to be distributed shall be distributed ratably among the preferred stockholders based upon the aggregate liquidation value.

- In accordance with the Company's certificate of incorporation, on or after the Series C or the Series D issuance dates, as applicable, if the Company issues or sells, or is deemed to have issued or sold any shares of its common stock for a consideration per share less than the conversion price with respect to Series C or Series D, then immediately upon such issue or sale, or deemed issue or sale, the conversion price shall be reduced to the conversion price determined by multiplying the conversion price in effect immediately prior to such issuance or sale by a price adjustment factor. The price adjustment factor causes the holders of the Series C and/or Series D stock to hold an adjusted number of shares equal to their total ownership before such issuance. If such a transaction occurs, the increase in preferred shares for the Series C and/or Series D holders will be accounted for as a deemed dividend by the Company. As a result of the issuance of Series E in August 2003, the conversion price of the Series D was reduced to $7.75 per share (see Note 13).

6. Common Stock

The Company's certificate of incorporation authorizes the Company to issue 25,000,000 shares of common stock with $0.001 par value per share. The Company's certificate of incorporation authorizes no other classes of common stock. The Company is prohibited from declaring dividends on common stock while any shares of preferred stock are outstanding.

The Company had reserved shares of its authorized common stock for future issuance as summarized in the table below:

	December 31, 2002	September 30, 2003
		(Unaudited)
For conversion of Series A	1,763,963	1,777,860
For conversion of Series B	2,504,500	2,552,261
For conversion of Series C	2,431,986	2,575,202
For conversion of Series D	1,451,230	1,557,430
For conversion of Series E	—	2,834,027
Outstanding employee stock options	363,375	799,000
Possible future issuance under stock option plans	915,025	483,650
	9,430,079	12,579,430

7. Notes Payable-Related Party

Demand notes of $4,250 were issued in 2001 to a holder of common stock of the Company to fund working capital needs. In October 2001, a portion of the proceeds from the issuance of Series C was used to repay all outstanding principal and accrued interest on the notes payable-related party. Interest expense incurred on the notes payable-related party based on an annual interest rate of 9% was $71 in 2001, which was included in general and administrative expenses in the Company's Statement of Operations.

GTx, Inc.
(a development stage company)

NOTES TO FINANCIAL STATEMENTS — (Continued)

8. License, Research and Development Agreements

License Agreements

In August 2002, the Company executed an Amended and Restated Exclusive License Agreement with The University of Tennessee Research Foundation ("UTRF") granting the Company a worldwide exclusive license under its method of use patents relating to Acapodene to market, distribute and sell licensed products, licensed processes or generic products. Under the terms of the agreement, the Company is required (i) to make annual maintenance fee payments and (ii) to make future royalty payments.

The amended license agreement with UTRF superseded a 1998 license agreement related to chemoprevention of prostate cancer between the Company and UTRF. Under the 1998 license agreement, the Company reimbursed UTRF for certain patent expenses incurred by UTRF and agreed to make sublicense fee payments and future royalty payments.

In June 2002, the Company executed two Amended and Restated Exclusive License Agreements with UTRF granting the Company worldwide exclusive licenses under its method of use patents relating to Andarine to market, distribute and sell licensed products, licensed processes or generic products. Under the terms of the agreements, the Company is required (i) to make annual maintenance fee payments and (ii) to make future royalty payments.

The amended license agreement with UTRF superseded a 2000 license agreement related to ARTA between the Company and UTRF. Under the 2000 license agreement, the Company reimbursed UTRF for certain patent expenses incurred by UTRF and agreed to make sublicense fee payments and future royalty payments.

Sponsored Research Agreement

The Company entered into a series of sponsored research agreements with the research foundation of a major university for one of the Company's programs. Under the terms of the agreements, the Company will reimburse the research foundation for the cost of research performed on the Company's behalf, in accordance with the terms of the agreements. The estimated cost of the research to be performed over a four-year period is approximately $4,000. The Company incurred expenses of $1,638, $956, $682, and 3,276 under these agreements for the years ended December 31, 2000, December 31, 2001, December 31, 2002, and from inception to December 31, 2002, respectively, which were included in research and development costs in the Company's Statements of Operations. The Company has the right to terminate the sponsored research agreement at any time. Upon termination, the Company will reimburse the research foundation for all research costs incurred on the Company's behalf not yet reimbursed by the Company.

Contract Research Organization ("CRO")

In 2000, the Company began a Phase IIb clinical trial for Acapodene for the reduction in the incidence of prostate cancer in men with high grade PIN. The last patient is scheduled to complete the trial in May 2004. The Company incurred expenses related to the Phase IIb clinical trial for the years ended December 31, 2000, 2001 and 2002 of approximately $1,290, $2,299 and $2,802, respectively, and approximately $6,391 from inception to December 31, 2002. The Company has specified rights to terminate the clinical trial and pay the CROs for fees incurred for the clinical trial not yet reimbursed by the Company.

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(a development stage company)

NOTES TO FINANCIAL STATEMENTS — (Continued)

In 2002, the Company began two additional Phase II clinical trials for Acapodene. These Phase II clinical trials are expected to be completed in 2003. The Company incurred expenses related to the Phase II clinical trials for the year ended December 31, 2002 of approximately $680, which was included in research and development costs in the Company's Statements of Operations. The Company estimates the total cost of these clinical trials to be approximately $936.

In 2002, the Company completed a Phase I clinical trial for Andarine. The Company incurred expenses related to the clinical trial for the year ended December 31, 2002 of approximately $370, which was included in research and development costs in the Company's Statements of Operations.

License and Supply Agreement

In 2000, the Company entered into a license and supply agreement with Orion Corporation for one of the Company's products. Under the terms of the agreement, the Company paid an initial license fee of $400 and is required to make future sublicense fee payments in the event the Company grants a sublicense under the licensed patents and future royalty payments in the event the Company sells products developed from the licensed patents.

9. Income Taxes

Deferred income taxes reflect the net tax effects of temporary differences between the carrying amounts of assets and liabilities for financial reporting purposes and the amounts used for income tax purposes.

The principal components of the Company's net deferred income taxes consist of the following:

	December 31,	
	2002	2001
Deferred income tax assets:		
Net operating loss carryforwards	$ 9,134	$ 4,906
Research credits	783	390
Cash basis method	496	84
Total deferred tax assets	10,413	5,380
Deferred income tax liabilities:		
Depreciation	50	31
Total deferred tax liabilities	50	31
Net deferred income tax assets	10,363	5,349
Valuation allowance	(10,363)	(5,349)
	$ —	$ —

At December 31, 2002, the Company has net operating loss carryforwards of approximately $23,420, which expire for federal purposes from 2020 through 2022 and for state purposes from 2015 to 2017, and research credits, which expire from 2013 through 2022. Utilization of the net operating loss carryforwards and credits may be subject to a substantial annual limitation due to an ownership change as 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.

GTx, Inc.
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NOTES TO FINANCIAL STATEMENTS — (Continued)

10. Operating Leases

The Company leases laboratory facilities and office space pursuant to leases accounted for as operating leases. Rent expense was approximately $34, $155, $170 and $401 for the years ended December 31, 2000, December 31, 2001, December 31, 2002, and from inception to December 31, 2002, respectively.

11. Stock Option Plans

In 1999, 2000, 2001 and 2002, the Company adopted the Genotherapeutics, Inc. Stock Option Plan ("1999 Plan"), the GTx, Inc. 2000 Stock Option Plan ("2000 Plan"), the GTx, Inc. 2001 Stock Option Plan ("2001 Plan") and the GTx, Inc. 2002 Stock Option Plan ("2002 Plan"), respectively (collectively, the "Plans"). The Plans provide for the Company to issue options to directors, officers and employees of the Company. The options are granted with an exercise price per share as determined by the Board of Directors. The exercise price per share will not be less than the fair market value of the stock on the date of grant. The Board of Directors cannot issue more than 25,500 options under the 1999 Plan, 108,375 options under the 2000 Plan, 298,775 options under the 2001 Plan and 850,000 options under the 2002 Plan in the aggregate at any time. The options generally vest one-third on the third anniversary, one-third on the fourth anniversary, and one-third on the fifth anniversary of the grant date. However, 127,500 of the 2001 options vest one-fifth per year beginning on the first anniversary of the date the options were granted. All options expire no later than the tenth anniversary of the grant date. In the event of a change in control of the Company, all stock options will become fully vested and be converted to cash, options or stock of equivalent value. None of the Company's stock options were exercisable at December 31, 2001 or 2000. At December 31, 2002, 34,000 of the Company's stock options were exercisable.

The following is a summary of option transactions:

	Options	Weighted Average Exercise Price Per Share
Balances at December 31, 1997 and 1998	—	
Options granted	25,500	$0.94
Balances at December 31, 1999	25,500	0.94
Options granted	108,375	2.24
Balances at December 31, 2000	133,875	1.99
Options granted	237,575	6.78
Options forfeited	(43,350)	2.32
Balances at December 31, 2001	328,100	5.42
Options granted	46,750	7.17
Options forfeited	(11,475)	3.41
Balances at December 31, 2002	363,375	5.71
Options granted (unaudited)	450,500	6.24
Options forfeited (unaudited)	(14,875)	7.40
Balances at September 30, 2003 (unaudited)	799,000	$5.97

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NOTES TO FINANCIAL STATEMENTS — (Continued)

The following table summarizes information about stock options outstanding at December 31, 2002:

Exercise Price	Options Outstanding	Weighted Average Remaining Contractual Life (years)	Weighted Average Exercise Price	Options Exercisable
$0.94	25,500	6.58	$0.94	8,500
2.24	57,375	7.92	2.24	—
6.78	263,500	8.81	6.78	25,500
7.85	17,000	9.75	7.85	—
	363,375	8.55	$5.71	34,000

The Company accounts for its Plans in accordance with APB Opinion No. 25. Prior to June 30, 2003, the Company did not recognize compensation expense for stock options because the exercise price of the stock options equaled or exceeded the market price of the underlying stock on the date of grant, which is the measurement date. In anticipation of the Company's initial public offering, the Company has determined that, for financial reporting purposes, the estimated value of its common stock was in excess of the exercise price for stock options issued to employees subsequent to June 30, 2003. Accordingly, the Company has recorded deferred stock-based compensation and is amortizing the related expense over the service period, which is generally five years. If the alternative method of accounting for stock incentive plans prescribed by SFAS No. 123 had been followed, the Company's net loss would have increased by approximately, $4, $37, and $115 for the years ended December 31, 2000, 2001, and 2002, respectively. The pro forma disclosures may not be representative of that to be expected in future years. The weighted average fair value of options granted was determined using the minimum value option pricing model assuming no expected dividends, a risk-free interest rate of 6.32% and a weighted average expected life of 10 years for the 1999, a risk-free interest rate of 5.47% and a weighted average expected life of 10 years for the 2000 grants, a risk-free interest rate of 4.24% and a weighted average expected life of 8 years for the 2001 grants, and a weighted average risk-free interest rate of 4.76% and a weighted average expected life of 8 years for the 2002 grants. The weighted average grant date fair value of options granted were $0.83, $1.65, and $1.75 for the years ended December 31, 2000, December 31, 2001, and December 31, 2002, respectively.

12. Employee Benefit Plan

In 2000, the Company established a 401(k) retirement savings plan that is available to all regular employees who have reached age 21. The plan is intended to qualify under Section 401(k) of the Internal Revenue Code of 1986, as amended. The plan provides that each participant may contribute up to 15% of their pre-tax compensation (up to a statutory limit, which was $11 in calendar year 2002). Employee contributions are held in the employees' name and invested by the plan's trustee. The plan also permits the Company to make matching contributions, subject to established limits. To date, the Company has not made any matching contributions to the plan on behalf of participating employees.

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NOTES TO FINANCIAL STATEMENTS — (Continued)

13. Subsequent Events

Issuance of Series E

On August 7, 2003, the Company authorized 450,000 shares and issued 329,536 shares of Series E at a purchase price of $60.69 per share resulting in gross cash proceeds of $20,000. The Company incurred issuance costs of $14 related to this series. Upon the issuance of Series E, the default provisions of all outstanding preferred stock were amended. If for any reason the Company defaults on its obligation to pay all or any portion of the redemption price, then the unpaid principal portion will bear interest at the greater of the prime rate plus 4% or 8%. Series E has similar terms to the other series of preferred stock. As a result of the issuance of Series E, the conversion price of the Series D was reduced to $7.75 per share as a result of the anti-dilution provisions of the Company's Certificate of Incorporation.

Initial Public Offering

In October 2003, the Board of Directors authorized the Company to file a Registration Statement with the Securities and Exchange Commission ("SEC") permitting the Company to sell shares of common stock in an initial public offering ("IPO"). In January 2004, the stockholders agreed that the IPO would constitute a Qualified Public Offering under the Company's Certificate of Incorporation (see Note 5). Upon the closing of the IPO, all shares of the Series A, Series A-2, Series B, Series B-2, Series C and Series E preferred stock will automatically convert into shares of common stock at a 8.5-for-1 conversion ratio and all shares of the Series D preferred stock will automatically convert into shares of common stock at a 8.61-for-1 conversion ratio.

Based on the Company's outstanding shares as of December 31, 2003, if the IPO is closed, all of the cumulative redeemable convertible preferred stock outstanding and dividends accrued thereon through December 31, 2003 will automatically convert into approximately 11,456,912 shares of common stock.

Issuance of Stock Options

On May 21, 2003, the Company issued 25,500 stock options under the 2001 stock option plan. The shares were issued at an exercise price of $6.24 a share. The shares vest one-third on the third anniversary, one-third on the fourth anniversary and one-third on the fifth anniversary of the grant date. The weighted average fair value of the options granted was $1.39. The weighted average fair value was determined using the minimum value option pricing model assuming no expected dividends, a risk-free interest rate of 3.15% and a weighted average expected life of 8 years.

On August 1, 2003, the Company issued 187,000 stock options under the 2002 stock option plan. The shares were issued at an exercise price of $6.24 a share. The shares vest one-third on the third anniversary, one-third on the fourth anniversary and one-third on the fifth anniversary of the grant date. The weighted average fair value of options granted was $1.84. The weighted average fair value was determined using the minimum value option pricing model assuming no expected dividends, a risk-free interest rate of 4.36% and a weighted average expected life of 8 years.

On September 1, 2003, the Company issued 238,000 stock options under the 2002 stock option plan. The shares were issued at an exercise price of $6.24 a share. The shares vest one-third on the third anniversary, one-third on the fourth anniversary and one-third on the fifth anniversary of the grant date. The weighted average fair value of options granted was $1.84. The weighted average fair value was determined using the minimum value option pricing model assuming no expected dividends, a risk-free interest rate of 4.36% and a weighted average expected life of 8 years.

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NOTES TO FINANCIAL STATEMENTS — (Continued)

On January 14, 2004, the Company adopted its 2004 Equity Incentive Plan and 2004 Non-Employee Directors' Stock Option Plan, both of which will become effective upon consummation of the Company's initial public offering of its common stock. The Company may issue awards for up to 1,500,000 shares of common stock under the 2004 Equity Incentive Plan and options for up to 200,000 shares of common stock under the 2004 Non-Employee Directors' Stock Option Plan.

Stock Split

On January 14, 2004, the Company effected an 8.5-for-1 stock split of its common stock in the form of a stock dividend. All common stock share and per share amounts in these financial statements have been adjusted retroactively to reflect the stock split. In connection with the stock split, the Company amended its Certificate of Incorporation to authorize 25,000,000 shares of common stock and 1,975,000 shares of preferred stock.

No dealer, salesperson or other person is authorized to give any information or to represent anything not contained in this prospectus. You must not rely on any unauthorized information or representations. This prospectus is an offer to sell only the shares offered hereby, but only under circumstances and in jurisdictions where it is lawful to do so. The information contained in this prospectus is current only as of its date.

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Through and including February 27, 2004 (the 25th day after the date of this prospectus), all dealers effecting transactions in these securities, whether or not participating in this offering, may be required to deliver a prospectus. This is in addition to a dealer's obligation to deliver a prospectus when acting as an underwriter and with respect to an unsold allotment or subscription.

5,400,000 Shares

GTx, Inc.

Common Stock

Goldman, Sachs & Co.

SG Cowen
Lazard

Representatives of the Underwriters