UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-K

[X] ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934

For the fiscal year ended December 31, 2003

OR

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

For the transition period from _____to_____

Commission file number 005-79588

GTx, Inc.

(Exact name of registrant as specified in its charter)

Delaware	**62-1715807**
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification No.)
3 N. Dunlap Street, 3rd Floor Van Vleet Building Memphis, Tennessee	**38163**
(Address of principal executive offices)	(Zip Code)

(901) 523-9700

(Registrant's telephone number, including area code)

Securities registered pursuant to Section 12(b) of the Act: None
Securities registered pursuant to Section 12(g) of the Act:

Common Stock, par value $0.001 per share

(Title of Class)

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

Indicate by check mark if disclosure of delinquent filers pursuant to Item 405 of Regulation S-K is not contained herein, and will not be contained, to the best of registrant's knowledge, in definitive proxy or information statements incorporated by reference in Part III of this Form 10-K or any amendment to this Form 10-K. [ ]

Indicate by check mark whether the registrant is an accelerated filer (as defined in Exchange Act Rule 12b-2). Yes [ ] No [X]

The registrant's common stock was not publicly traded on the last business day of the registrant's most recently completed second fiscal quarter.

As of March 24,2004, there were 24,656,923 shares of GTx Common Stock $0.001 par value outstanding. The aggregate market value of the voting stock held by non-affiliates of the registrant was approximately $81,132,000 based on the closing sale price of such stock as reported by the Nasdaq National Market on March 23, 2004, assuming that all shares beneficially held by executive officers and members of the registrant's Board of Directors are shares owned by "affiliates," a status which each of the executive officers and directors may individually disclaim.

DOCUMENTS INCORPORATED BY REFERENCE
None

TABLE OF CONTENTS

PART I

ITEM 1. 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™ (Toremifene Citrate) tablets, 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. In March 2004, we entered into a joint collaboration and license agreement with Ortho Biotech Products L.P. for the continued clinical development of our second product candidate, andarine and specified backup SARM compounds. Andarine is the most advanced of our internally discovered portfolio of compounds designed to modulate the effects of hormones. Together with Ortho Biotech, we intend to continue to pursue the clinical development of andarine for the treatment of cachexia from various types of cancer and other chronic diseases. Cancer cachexia is a muscle wasting condition that is a potentially life-threatening complication of many cancers.

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 citrate, 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 is the standard medical treatment for patients who have advanced, recurrent or metastatic prostate cancer. Androgen deprivation therapy reduces blood levels of testosterone, the growth factor for 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, together with Ortho Biotech, 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 various types of cancer.

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 hormone receptors.

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	Four Phase I clinical trials completed	Phase II dose finding clinical trials for treatment of cachexia from various types of cancer scheduled to begin in 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 Pharmaceutical Group 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 the proof of a concept for treatment. 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 primarily from 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. 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 85 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 12 months and who have significant existing bone loss, 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. Our strategy is to continue to pursue the clinical development of andarine with Ortho Biotech for the treatment of cachexia from various types of cancer. We selected this indication because it represents a potentially large market and, we believe it has a relatively well-defined clinical and regulatory process. For cachexia from various types of cancer, we are developing andarine for the treatment of both men and women. Depending on the results of our initial development efforts, together with Ortho Biotech, we may also develop andarine for other diseases.

Andarine For The Treatment Of Cancer Cachexia

Scientific Overview. Cachexia is defined as the unintentional 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 four Phase I clinical trials of andarine in a total of 134 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. The details and design of Phase II clinical trials for andarine will be determined by a joint development committee to be established as a part of our joint collaboration with Ortho Biotech.

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. In March 2004, we entered into a joint collaboration and license agreement with Ortho Biotech Products L.P. for the continued clinical development of andarine and specified backup SARM compounds. Together with Ortho Biotech, we intend to continue to pursue the clinical development of andarine for the treatment of cachexia from various types of cancer. In addition, GTx and Ortho Biotech may develop andarine for the treatment of other causes of cachexia, including end stage renal disease, which represents a large potential market with unmet medical needs. Andarine could also potentially be developed and commercialized for other men's health indications. The terms of our agreement with Ortho Biotech are more fully described below in "Licenses and Collaborative Relationships – Ortho Biotech Products L.P."

Build Upon Our Other SARM And Other Drug Discovery Capabilities To Sustain Our Small Molecule Product Candidate Pipeline

We intend to develop our other SARMs as well as 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. We may seek one or more collaborators for the development and commercialization of our other SARM product candidates, including ostarine and prostarine.

Licenses and Collaborative Relationships

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

Ortho Biotech Products L.P.

Under a joint collaboration and license agreement with Ortho Biotech Products L.P., a wholly owned subsidiary of Johnson & Johnson, we will receive an upfront licensing fee of $6 million, additional licensing fees and milestone payments up to $82 million based on andarine and up to $45 million for each additional licensed compound achieving specific clinical development decisions or obtaining regulatory approvals. Johnson & Johnson Pharmaceutical Research & Development will be responsible for further clinical development and expenses related to andarine and other licensed SARM compounds including reimbursement of approximately $687,000 in expenses

for our recently completed Phase Id clinical trial for andarine. Ortho Biotech will be responsible for commercialization and expenses related to andarine and other licensed SARM compounds. If andarine is approved for commercial sale, Ortho Biotech will exclusively market andarine in the United States and markets outside the United States. Under the agreement, we have the option to co-promote andarine and the other licensed SARM compounds to urologists in the United States for indications specifically related to men's health. We will receive royalties on all sales throughout the worldwide licensed territory, as well as an additional royalty in excess of 20% on all co-promoted sales generated from urologists in the United States.

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 after commercialization of the product. 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 future royalty payments. 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 division 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, 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. Under our joint collaboration and license agreement with Ortho Biotech, the manufacturing of andarine will be transitioned to Ortho Biotech, and Ortho Biotech will be responsible for clinical supply and full-scale commercialization of andarine.

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 personnel, and we have limited 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 and to market andarine to urologists in the United States. We believe that the urology and medical oncology markets in the United States are 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 marketing andarine to address the cancer cachexia market would require a large sales force and because of the risks and costs of developing andarine for cachexia from various types of cancer, we have entered into a joint collaboration and license agreement with Ortho Biotech for the development and commercialization of andarine and specified backup SARM compounds. See “Licenses and Collaborative Relationships – Ortho Biotech Products L.P.”.

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 either have been issued or may be issued 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 healthy human subjects.. The goal of the Phase I clinical trial is to establish initial data about the safety, tolerability and pharmacokinetic properties 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.

Employees

As of December 31, 2003, we had 42 employees of whom 11 were Ph.D.s and three 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.

Available Information

We file reports with the Securities and Exchange Commission ("SEC"), including annual reports on Form 10-K, quarterly reports on Form 10-Q, and other reports from time to time. The public may read and copy any materials filed with the SEC at the SEC's Public Reference Room at 450 Fifth Street, NW, Washington, DC 20549. The public may obtain information on the operation of the Public Reference Room by calling the SEC at 1-800-SEC-0330. We are an electronic filer and the SEC maintains an Internet site at www.sec.gov that contains the reports, proxy and information statements, and other information filed electronically. Our website address is www.gtxinc.com. Please note that these website addresses are provided as inactive textual references only. We make available free of charge through our website our Annual Report on Form 10-K, quarterly reports on Form 10-Q, current reports on Form 8-K, and all amendments to those reports as soon as reasonably practicable after such material is electronically filed with or furnished to the SEC. The information provided on our website is not part of this report, and is therefore not incorporated by reference unless such information is otherwise specifically referenced elsewhere in this report.

ADDITIONAL FACTORS THAT MIGHT AFFECT FUTURE RESULTS

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 December 31, 2003, we had a deficit accumulated during the development stage of $151.8 million, of which $113.0 million related to non-cash dividends and adjustments to the preferred stock redemption value. 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, except for the upfront license fee of $6 million to be received from Ortho Biotech for our joint collaboration for the development and commercialization of andarine and specified backup SARM compounds. 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, except with regard to andarine, for which all clinical development, sales and marketing and manufacturing expenses will be paid by Ortho Biotech or Johnson & Johnson Pharmaceutical Research & Development.

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 our initial public offering and our current cash resources and interest on these funds and committed payments under our research collaborative agreement will be sufficient to meet our projected operating requirements through at least 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;
- potential future licensing fees, milestone payments and royalty payments;
- 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, such as our license agreement with Ortho Biotech, 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 may not be 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
- our product candidates may not have 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 division 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, however, under our joint collaboration and license agreement with Ortho Biotech, Ortho Biotech will be responsible for the manufacture, packaging and supply of andarine for clinical trials and 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 and/or Ortho Biotech 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 are dependent on our collaborative arrangement with Ortho Biotech to develop and commercialize andarine, and we may be dependent upon additional collaborative arrangements to complete the development and commercialization of some of our other 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 additional 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, including our arrangement with Ortho Biotech, 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 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 15 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 are issued with respect to 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 15 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 we have limited marketing expertise. We have no experience as a company in the sales 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 $10 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 Our Common Stock

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

Our stock price is likely to be volatile. 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 significant 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.

Our officers, directors and largest stockholders will maintain the ability to control all matters submitted to stockholders for approval.

Our officers, directors and holders of 5% or more of our outstanding common stock beneficially own approximately 78% of our outstanding common stock. 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 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.

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 their shares, could reduce the market price of our common stock. As of March 24, 2004, we had 24,656,923 shares of common stock outstanding, of which 5,400,000 may be resold in the public market immediately. The remaining 19,256,923 shares, or 78% of our outstanding shares, 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
16,344,456 shares, or 66%	August 2, 2004 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.
2,912,467 shares, or 12%	August 7, 2004

Moreover, J.R. Hyde, III, Oracle Partners, L.P. and Memphis Biomed Ventures I, L.P., three of our largest stockholders, and their affiliates, who hold in the aggregate 11,407,917 shares of common stock, have rights, subject to some conditions, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders. Additionally, all shares of common stock that we may issue under our employee benefit plans can be freely sold in the public market upon issuance.

SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Annual Report on Form 10-K contains forward-looking statements. The forward-looking statements are contained principally in the sections entitled "Additional Factors That Might Affect Future Results," "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;
- potential future licensing fees, milestone payments and royalty payments;
- 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 Annual Report on Form 10-K in greater detail under the heading "Additional Factors That May Affect Future Results." 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 Annual Report on Form 10-K. You should read this Annual Report on Form 10-K and the documents that we reference in this Annual Report on Form 10-K and have filed as exhibits, 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.

ITEM 2. PROPERTIES

As of December 31, 2003, 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.

ITEM 3. LEGAL PROCEEDINGS

We are not currently involved in any material legal proceedings.

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

On November 26, 2003, our sole stockholder, acting by written consent, approved the merger of GTx, Inc., a Tennessee corporation, into GTx, Inc., a Delaware corporation.

PART II

ITEM 5. MARKET FOR THE REGISTRANT'S COMMON EQUITY AND RELATED STOCKHOLDER MATTERS

Our common stock began trading on the Nasdaq National Market under the symbol "GTXI" on February 3, 2004. Prior to that date, there was no established public trading market for our common stock. On March 23, 2004, the last sale price of our common stock on the Nasdaq National Market was $10.20 per share and there were approximately 38 holders of record of the common stock.

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.

The following table provides information regarding our equity compensation plans as of December 31, 2003:

Plan Category	Number of securities to be issued upon exercise of outstanding options, warrants and rights[1] (a)	Weighted-average exercise price of outstanding options, warrants and rights (b)	Number of securities remaining available for future issuance under equity compensation plans (excluding securities reflected in column (a)) (c)
Equity compensation plans approved by security holders	828,750	$ 6.18	453,050
Equity compensation plans not approved by security holders	—	—	—
Total	828,750	$ 6.18	453,050

[1] The 2004 Equity Incentive Plan has an aggregate of 1,500,000 shares of common stock reserved for issuance under the 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. The 2004 Non-Employee Directors' Stock Option Plan has an aggregate of 200,000 shares of common stock reserved for issuance under the plan, 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 under the plan during the prior calendar year. Furthermore, the Board of Directors has the authority under both plans, to designate a smaller number of shares by which the authorized number of shares of common stock will be increased.

For more information regarding our equity compensation plans, see page 51, "Benefit Plans."

Recent Sales of Unregistered Securities; Use of Proceeds from Registered Securities

In October 2001, we issued and sold an aggregate of 260,154 shares of our 8% Series C Cumulative Convertible Redeemable Preferred Stock to three accredited investors at $57.658 per share, for an aggregate offering price of $14,999,959. We claimed exemption from registration under the Securities Act of 1933, as amended (the "Securities Act") for the offer and sale of these securities by virtue of Section 4(2) of the Securities Act in that such sales and issuances did not involve a public offering. The recipients of securities in each of these transactions represented their intention to acquire the securities for investment only and not with view to or for sale in connection with any distribution thereof and appropriate legends were affixed to the share certificates and instruments issued in such transactions. All recipients had adequate access, through their relationships with us, to information about us.

In July 2002, we issued and sold an aggregate of 164,765 shares of our 8% Series D Cumulative Convertible Redeemable Preferred Stock to four accredited investors at $66.762 per share for an aggregate offering price of $11,000,041. We claimed exemption from registration under the Securities Act for the offer and sale of these securities by virtue of Section 4(2) of the Securities Act in that such offers and sales did not involve a public offering. The recipients of securities in each of these transactions represented their intention to acquire the securities for investment only and not with view to or for sale in connection with any distribution thereof and appropriate legends were affixed to the share certificates and instruments issued in such transactions. All recipients had adequate access, through their relationships with us, to information about us.

In August 2003, we issued and sold an aggregate of 329,536 shares of our 8% Series E Cumulative Convertible Redeemable Preferred Stock to 11 accredited investors at $60.692 per share for an aggregate offering price of $20,000,199. We claimed exemption from registration under the Securities Act for the offer and sale of these securities by virtue of Section 4(2) of the Securities Act and Rule 506 of Regulation D. The offer and sale did not involve any public offering, were made without general solicitation or advertising and each purchaser was a sophisticated investor with access to all relevant information necessary to evaluate the investment and represented to us that the shares were being acquired for investment.

On February 6, 2004, as a result of our initial public offering, all of our issued and outstanding shares of preferred stock were converted into 11,521,075 shares of common stock in accordance with the terms of such preferred stock.

We have granted stock options under our stock option plans covering an aggregate of 828,750 shares of common stock (net of exercises, expirations and cancellations) as of December 31, 2003, at exercise prices ranging from $2.24 to $7.85 per share. Options to purchase an aggregate of 850 shares of common stock have been exercised for an aggregate purchase price of $800.00, or a weighted exercise price of $0.94 per share. We claimed exemption from registration under the Securities Act for the sale and issuance of securities in the transactions described in this paragraph by virtue of Section 4(2) of the Securities Act and Rule 701 promulgated under Section 3(b) of the Securities Act as a transaction under a compensatory benefit plan under Rule 701.

On February 2, 2004, the SEC declared effective our first registration statement, filed on Form S-1 (File No. 333-109700) under the Securities Act in connection with the initial public offering of our common stock. Goldman Sachs & Co., SG Cowen Securities Corporation, and Lazard Frères & Co. LLC acted as the underwriters for the offering.

Our common stock began trading on The Nasdaq National Market under the trading symbol "GTXI" on February 3, 2004. We sold 5,400,000 shares of common stock in our initial public offering at $14.50 per share. The offering terminated after the sale of all of the securities registered on the registration statement and the expiration of the underwriters' over-allotment option. The aggregate gross proceeds from the shares of common stock sold were $78.3 million. We paid the underwriters a commission of $5.5 million and incurred offering expenses estimated at $2.8 million. After deducting the underwriters' commission and the estimated offering expenses, we received net proceeds of approximately $70.0 million. We invested the net proceeds in short-term securities and expect to use the net proceeds to fund our clinical trials and other research and development activities and for general corporate purposes. In addition, we may use a portion of the net proceeds to acquire equipment, products, technologies or businesses, although we currently have no commitments or agreements relating to any of these types of transactions. None of the expenses, or application of the net proceeds, were paid, directly or indirectly, to directors, officers or persons owning 10% or more of our common stock or to their associates, or to our affiliates.

ITEM 6. 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 audited financial statements, notes thereto and other financial information included elsewhere in this Annual Report on Form 10-K. We derived the selected financial data for each of the five fiscal years in the period ended December 31, 2003 from our audited financial statements.

We computed pro forma net loss per share for the year ended December 31, 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 our initial public offering as if such conversion occurred on 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,				
	1999	2000	2001	2002	2003
	(in thousands, except share and per share data)				
Statement of Operations Data:					
Operating expenses:					
Research and development	$ 518	$ 2,679	$ 5,744	$ 9,285	$ 10,468
General and administrative	256	1,203	2,187	2,405	3,512
Depreciation	45	80	215	332	357
Total operating expenses	819	3,962	8,146	12,022	14,337
Other income:					
Interest income	69	150	83	156	143
Total other income	69	150	83	156	143
Net loss	(750)	(3,812)	(8,063)	(11,866)	(14,194)
Accrued preferred stock dividends	(83)	(297)	(790)	(2,147)	(3,436)
Adjustment to preferred stock redemption value	—	(21,077)	(57)	(7,220)	(77,844)
Net loss attributable to common stockholders	$ (833)	$ (25,186)	$ (8,910)	$ (21,233)	$ (95,474)
Net loss per share attributable to common stockholders, basic and diluted:	$ (.11)	$ (3.26)	$ (1.15)	$ (2.75)	$ (12.34)
Weighted average shares used in computing net loss per share attributable to common stockholders, basic and diluted:	7,734,998	7,734,998	7,734,998	7,734,998	7,735,125
Pro forma net loss per share attributable to common stockholders — basic and diluted					$ (0.83)
Shares used in computing pro forma net loss per share attributable to common stockholders — basic and diluted					17,018,655

	As of December 31,				
	1999	2000	2001	2002	2003
Balance Sheet Data:					
Cash and cash equivalents	$1,542	$ 2,667	$ 8,834	$ 8,925	$ 14,769
Working capital	1,435	2,241	8,544	7,654	12,775
Total assets	1,678	3,201	10,117	10,030	17,310
Cumulative redeemable convertible preferred stock	1,538	27,912	43,702	64,026	165,292
Deficit accumulated during development stage	(949)	(26,135)	(35,045)	(56,278)	(151,752)
Total stockholders' equity(deficit)	21	(25,165)	(34,075)	(55,308)	(150,231)

ITEM 7. 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 Annual Report on Form 10-K. 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 "Additional Factors That May Affect Future Results" and elsewhere in this Annual Report on Form 10-K.

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. Together with Ortho Biotech, we are initially developing our second product candidate, andarine, for the treatment of cachexia 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 seek collaborators to commercialize our product candidates where the target physician market is broader than urologists and medical oncologists and outside the United States, as we have done with Ortho Biotech for andarine and specified backup SARM compounds.

Through December 31, 2003, we did not generate any product revenue, and we 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 December 31, 2003, we had a deficit accumulated during the development stage of $151.8 million, of which $113.0 million related to non-cash dividends and adjustments to the preferred stock redemption value. 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 $113.0 million related to our cumulative redeemable convertible preferred stock. See “Critical Accounting Policies —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.

In March 2004, we entered into a joint collaboration and license agreement with Ortho Biotech Products L.P., a wholly owned subsidiary of Johnson & Johnson. Under the agreement, we will receive an upfront licensing fee of $6 million, be reimbursed for development expenses of approximately $687,000 for our recently completed Phase Id clinical trial for andarine, and receive additional licensing fees and milestone payments up to $82 million based on andarine and up to $45 million for each additional licensed compound achieving specific clinical development decisions or obtaining regulatory approvals. Johnson & Johnson Pharmaceutical Research & Development will be responsible for further clinical development and expenses related to andarine and other licensed SARM compounds. Ortho Biotech will be responsible for commercialization and expenses related to andarine and other licensed SARM compounds. If andarine is approved for commercial sale, Ortho Biotech will exclusively market andarine in the United States and markets outside the United States. Under the agreement, we have the option to co-promote andarine and the other licensed SARM compounds to urologists in the United States for indications specifically related to men’s health. We will receive royalties on all sales, as well as an additional royalty on all co-promoted sales generated from urologists in the United States.

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 year ended December 31, 2003 and 77% of our total operating expenses for the year ended December 31, 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,			Inception Through December 31
			2001	2002	2003	2003
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	$2,436	$3,168	$ 2,833	$ 11,124
Side effects of androgen deprivation therapy	Pivotal Phase III clinical trial	Pivotal Phase III clinical trial initiated in November 2003	—	807	1,625	2,432
SARM Program						
Andarine						
Cachexia from various types of cancer	Four Phase I clinical trials completed	Phase II clinical trial for treatment of cachexia from various types of cancer scheduled to begin in 2004	2,430	4,134	5,046	11,751
Other Product Candidates	Preclinical		878	1,176	964	3,572
Total research and development spending			$5,744	$9,285	$10,468	$ 28,879

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 "Additional Factors That May Affect Future Results" section of this Annual Report on Form 10-K, 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 "Additional Factors That May Affect Future Results" section of this Annual Report on Form 10-K.

General and Administrative Expenses

General and administrative expenses consist primarily of salaries and other related costs for personnel serving executive, finance, legal, human resources, information technology, and public relations functions. Other costs include facility costs not otherwise included in research and development expense and professional fees for legal, accounting, and public relations services. We expect that our general and administrative expenses will increase as we add personnel, comply with the reporting obligations applicable to public companies and develop our sales and marketing functions. From inception through December 31, 2003, we spent an aggregate of $9.7 million on general and administrative expenses.

Results of Operations

Comparison of Years Ended December 31, 2003 and December 31, 2002

Research and Development. Research and development expenses increased 12.7% to $10.5 million for the year ended December 31, 2003 from $9.3 million for the year ended December 31, 2002. Research and development expenses for the year ended December 31, 2003 included amortization of non-cash stock-based compensation expense of $472,000. The increases in research and development expenses included increased expenditures of approximately $818,000 related to two Phase II clinical trials and the preparation for and initiation of a pivotal Phase III clinical trial of Acapodene for the treatment of side effects of androgen deprivation therapy. In addition, we incurred additional expenses of approximately $912,000 related to the completion of Phase I clinical trials for andarine and the continued development of andarine and other product candidates in our SARM program. These increases were offset by a reduction 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 $335,000 and a reduction in research and development spending on other product candidates of approximately $212,000.

We expect that research and development expenditures will continue to increase substantially during 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, and (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. Under the terms of our collaboration with Ortho Biotech, Johnson & Johnson Pharmaceutical Research and Development will be responsible for future clinical development and expenses of andarine. 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 costs increased 46.0% to $3.5 million for the year ended December 31, 2003 from $2.4 million for the year ended December 31, 2002. The increase was primarily due to an increase in personnel related expenses of approximately $424,000 and an increase in professional fees of approximately $379,000. The increase in general and administrative expenses for the year ended December 31, 2003 included amortization of non-cash stock-based compensation expense of $78,000.

Interest Income. Interest income decreased 8.3% to approximately $143,000 for the year ended December 31, 2003 from approximately $156,000 for the year ended December 31, 2002. The decrease was the 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 year ended December 31, 2003 was an increase of $77.8 million, or $56.07 per share, as compared to an increase of $7.2 million, or $9.10 per share, for the year ended December 31, 2002. The per share redemption value was $57.66 as of December 31, 2001, $66.76 as of December 31, 2002 and $122.83 as of December 31, 2003. The increase in the redemption values for the years ended December 31, 2003 and 2002 were the result of the achievement of significant milestones in clinical trials and general market conditions. In addition, the per share redemption value at December 31, 2003 was determined based on the estimated projected midpoint of the range of the Company's initial public offering price per common share at the date the accompanying financial statements were prepared. See "Critical Accounting Policies —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 $298,000 for the year ended December 31, 2002 as compared to the prior year.

General and Administrative. General and administrative expenses increased 10.0% 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 88.0% 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.

Liquidity and Capital Resources

Through December 31, 2003, we did not generate any product revenue, and we 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 December 31, 2003, we had a deficit accumulated during the development stage of $151.8 million, of which $113.0 million related to non-cash dividends and adjustments to the preferred stock redemption value.

The following table summarizes our issuances of preferred stock through December 31, 2003:

Series	Date	Approximate Gross Proceeds
		(in thousands)
A	May 1999	$ 1,455
B	July 2000	5,000
C	October 2001	15,000
D	July 2002	11,000
E	August 2003	20,000

At December 31, 2003, we had cash and cash equivalents of $14.8 million, compared to $8.9 million at December 31, 2002. On February 6, 2004, we completed an initial public offering of 5.4 million shares of Common Stock at a price of $14.50 per share, resulting in net proceeds of approximately $70 million.

Net cash used in operating activities was $13.0 million and $10.6 million for the years ended December 31, 2003 and 2002, respectively. The use of cash in both periods resulted primarily from funding our net losses.

Net cash used in investing activities was $108,000 and $313,000 for the years ended December 31, 2003 and 2002, respectively. The use of cash in both periods was primarily for the purchase of office and research and development equipment. We currently expect to make expenditures for capital equipment and leasehold improvements of up to $3.3 million in 2004.

Net cash provided by financing activities, was $19.0 million for the year ended December 31, 2003 and $11.0 million for the year ended December 31, 2002. Net cash provided by financing activities for the year ended December 31, 2003 included $20.0 million which resulted from the sale of preferred stock which was offset by

approximately $1.0 million of professional fees paid by the Company in connection with the filing of a registration statement with the SEC related to our public offering.

We believe that the net proceeds from our initial public offering, our current cash resources and interest on these funds, and committed payments under our research collaborative agreement will be sufficient to meet our projected operating requirements through at least the end of 2005. This estimate does not include payments that we may receive as milestone payments under our joint collaboration and license agreement with Ortho Biotech.

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 "Additional Factors That May Affect Future Results" section of this Annual Report on Form 10-K. 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 achievement of certain milestone events under our joint collaboration and license agreement with Ortho Biotech;
- 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, such as our arrangement with Ortho Biotech, as well as through interest income earned on cash balances. With the exception of payments that we may receive under our collaboration with Ortho Biotech, 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, such as our arrangement with Ortho Biotech, 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. At December 31, 2003, we had contractual obligations related to a facilities lease as follows:

	Payment Due by Period (in thousands)				
	Total	Less than 1 year	1-3 years	5 years	More than 5 years
Contractual obligations	$354	$ 202	$ 152	$ —	$—

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 our initial public 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 non-cash deferred stock-based compensation of approximately $4.1 million and are amortizing the related expense over the service period, which is generally five years. Our compensation expense for 2003 would have decreased from $550,000 to $424,000 and our diluted net loss per share attributable to common stockholders would have been approximately $0.01 lower had we recognized an expense equal to the estimated fair market value of employee stock options granted through December 31, 2003 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 in Item 8 of this Annual Report on Form 10-K.

Preferred Stock Redemption Value

Unless the preferred stock is previously converted into common stock pursuant to mandatory or optional conversion features governing the preferred stock, the preferred stock is subject to redemption on or after August 31, 2006, at the option of the preferred shareholder. The per share redemption price is equal to the greater of 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. At December 31,

2003, the per share redemption value was determined based on the estimated projected midpoint on the range of the company's initial public offering price per common share. The changes in redemption value affect the loss attributable to common stockholders.

Recent Accounting Pronouncements

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 March 15, 2004. The Company does not have any ownership in any variable interest entities as of December 31, 2003. 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 impact of SFAS No. 150 and has determined that its financial instruments (common stock and preferred stock) will not be affected.

ITEM 7A. 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.

ITEM 8. FINANCIAL STATEMENTS AND SUPPLEMENTARY DATA

The Independent Auditor's Report, Financial Statements and Selected Quarterly Financial Data are set forth on pages F-1 to F-20 of this Annual Report on Form 10-K.

ITEM 9. CHANGES IN AND DISAGREEMENTS WITH ACCOUNTANTS ON ACCOUNTING AND FINANCIAL DISCLOSURE

There have been no disagreements with our independent accountants on any matter of accounting principles or practices or financial statement disclosure.

ITEM 9A. CONTROLS AND PROCEDURES

Evaluation of Disclosure Controls and Procedures

We maintain disclosure controls and procedures, that are designed to ensure that information required to be disclosed in the reports that we file or submit under the Securities Exchange Act is recorded, processed, summarized, and reported within the time periods specified in the SEC's rules and forms and that such information is accumulated and communicated to our management, including our Chief Executive Officer and Chief Financial Officer, as appropriate, to allow timely decisions regarding required disclosure. Our Chief Executive Officer and

Chief Financial Officer carried out an evaluation of the effectiveness of the design and operation of our disclosure controls and procedures as of the end of the period covered by this report. Based on the evaluation of these disclosure controls and procedures, the Chief Executive Officer and Chief Financial Officer concluded that our disclosure controls and procedures were effective. It should be noted that any system of controls, however well designed and operated, can provide only reasonable, and not absolute, assurance that the objectives of the system are met. In addition, the design of any control system is based in part upon certain assumptions about the likelihood of future events. Because of these and other inherent limitations of control systems, there can be no assurance that any design will succeed in achieving its stated goals under all potential future conditions, regardless of how remote.

Changes in Internal Control Over Financial Reporting

There were no changes in our internal control over financial reporting during the fourth quarter 2003 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

PART III

ITEM 10. DIRECTORS AND EXECUTIVE OFFICERS OF THE REGISTRANT

The following table sets forth information about our directors, executive officers and other key employees as of March 24, 2004.

Name	Age	Position(s)
Directors and Executive Officers		
J.R. Hyde, III[1][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
Andrew Clarkson[2][3]	66	Director
J. Kenneth Glass[1][2]	57	Director
Rosemary Mazanet, M.D., Ph.D.[3]	48	Director
John H. Pontius[1][2]	48	Director
Other Key Employees		
K. Gary Barnette, Ph.D.	36	Director of Regulatory Affairs
T. Gary Bird, Ph.D.	52	Director of Manufacturing
Robert S. Boger, M.D.	57	Director of Clinical Development
Greg A. Deener	42	Director of Marketing & Sales
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. Dr. Steiner continues to maintain an affiliation with the Department of Urology at the University of Tennessee College of Medicine, as a Professor of Urology.

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.

Andrew M. Clarkson has served as a director since March 2004. From 1996 to 2002, Mr. Clarkson was a part-time employee and strategic consultant with AutoZone, Inc. and from 1995 to 2001 he served as a director, and Chairman of the Finance Committee, of AutoZone, Inc. Mr. Clarkson was previously Chief Financial Officer and Director of Malone and Hyde, Inc. Prior to that time, Mr. Clarkson held senior financial positions at General Foods Corporation and as a Vice President and Treasurer of F. W. Woolworth. Mr. Clarkson graduated from Oxford University, attended McGill University and earned his MBA from The Harvard Business School. He currently serves on the Boards of Amphenol Corporation (NYSE), Royal Furniture, and TruckPro, Inc.

J. Kenneth Glass has served as a director since March 2004. Mr. Glass is Chairman of the Board, President and Chief Executive Officer of First Tennessee Bank National Corporation ("First Tennessee") and First Tennessee Bank National Association ("First Tennessee Bank"). Mr. Glass was elected Chairman of the Board of First Tennessee effective January 1, 2004, and President and Chief Executive Officer of First Tennessee in July 2002. From July 2001 through July 2002, Mr. Glass was President and Chief Operating Officer of First Tennessee. From April 1999 through July 2001, he was President — Retail Financial Services of First Tennessee Bank and from April 2000 through July 2001, President – Retail Financial Services of First Tennessee. Mr. Glass received his B.A. in Accounting from Harding University and graduated from Harvard Business School's Advanced Management Program. Mr. Glass is a director of FedEx Corporation and First Tennessee.

Rosemary Mazanet, M.D., Ph.D. has served as a director since October 2001. Dr. Mazanet is currently the Chief Executive Officer of Breakthrough Therapeutics. From 1998 through 2004, Dr. Mazanet was Chief Scientific Officer and a General Partner of Oracle Partners, L.P. 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 Trustees 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 on faculty 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. 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.

Gregory A. Deener has served as our Director of Marketing and Sales since February 2004. Mr. Deener has over 20 years of experience in Marketing and Sales and has launched a Urology medicine within the U.S. From 1996 to December 2003, Mr. Deener served as a Marketing Director for GlaxoSmithKline in various roles within the U.S. and Europe. Most recently Mr. Deener was responsible for the launch of Avodart, a urology medicine for BPH. From 1983 to 1996, Mr. Deener worked for Procter & Gamble in Brand Management and Sales. Mr. Deener holds a B.S. in Business Administration from the University of North Carolina at Chapel Hill.

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

We have an authorized Board of Directors consisting of seven members, a majority of whom are "independent" under the Nasdaq Marketplace Rules and applicable SEC rules. In accordance with the terms of our certificate of incorporation and bylaws, the Board of Directors is divided into three classes, Class I, Class II and Class III, with each class serving staggered three-year terms as follows:

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

Our certificate of incorporation 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. Clarkson, Mr. Glass and Mr. Pontius. Mr. Clarkson and Mr. Glass are "independent directors" for Audit Committee purposes under applicable Nasdaq and SEC rules. Mr. Pontius is not an independent director because he is an executive officer of a company owned by Mr. Hyde, who is our affiliate. However, because we completed our initial public offering in 2004, the Nasdaq Marketplace Rules and applicable SEC rules allow Mr. Pontius to serve as a member of our Audit Committee until February 3, 2005. Mr. Pontius' service as a member of the Audit Committee will not adversely affect the ability of the Audit Committee to act independently of management or to satisfy its legal obligations. 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, Mr. Pontius and Mr. Glass. 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. Clarkson. 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 and in March 2004, Mr. Glass replaced Mr. Pontius as a member of the 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 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 an additional 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 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. Each of 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, has received an initial option to purchase 10,000 shares of common stock and will receive 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.

CODE OF BUSINESS CONDUCT AND ETHICS

We have adopted a code of business conduct and ethics for our employees, including our Chief Executive Officer, Chief Financial Officer and other executives. A copy of this code is included as Exhibit 14.1 with this Form 10-K Annual Report.

AUDIT COMMITTEE FINANCIAL EXPERT

All of the members of our Audit Committee are Audit Committee financial experts. Mr. Clarkson and Mr. Glass are independent directors for Audit Committee purposes. Mr. Pontius is not an independent director because he is an executive officer of a company owned by Mr. Hyde, who is our affiliate. However, because we completed our initial public offering in 2004, the Nasdaq Marketplace Rules and applicable SEC rules allow Mr. Pontius to serve as a member of our Audit Committee until February 3, 2005. Mr. Pontius' service as a member of the Audit Committee will not adversely affect the ability of the Audit Committee to act independently of management or to satisfy its legal obligations.

SECTION 16(a) BENEFICIAL OWNERSHIP REPORTING COMPLIANCE

Depending on the results of our initial development efforts, together with Ortho Biotech, we may also develop andarine for other diseases. We were not subject to the reporting requirements of Section 16(a) of the Exchange Act for the fiscal year ended December 31, 2003. Accordingly, none of our directors, executive officers or beneficial owners of more than 10% of our equity securities was required to file reports under Section 16(a) of the Exchange Act.

ITEM 11. 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 each of the last three fiscal years whose total annual salary and bonus exceeded $100,000. We refer to these executive officers in this Annual Report on Form 10-K 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	—
	2001	175,000	—
Marc S. Hanover	2003	180,000	—
President and Chief Operating Officer	2002	180,000	—
	2001	180,000	—
Henry P. Doggrell	2003	193,000	12,750
General Counsel and Secretary	2002	178,750	—
	2001	43,750	127,500
Mark E. Mosteller	2003	154,167	42,500
Chief Financial Officer	2002	135,417	17,000
	2001	50,721	25,500

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 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 2004 Non-Employee Directors' Stock Option 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 and our stockholders approved 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 either 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 and our stockholders approved 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 and our stockholders approved 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. 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 are 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.

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 stockholder 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. 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, options to purchase 40,000 shares of common stock have been granted 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 our 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 consummation of our initial public offering, each eligible non-employee director was granted an option to purchase 10,000 shares of common stock, the initial grant. Any individual who becomes an eligible non-employee director 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 a statutory limit, which for most employees was $12,000 in 2003. 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 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 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 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.

ITEM 12. SECURITY OWNERSHIP OF CERTAIN BENEFICIAL OWNERS AND MANAGEMENT AND RELATED STOCKHOLDER MATTERS

The following table sets forth information as of March 24, 2004 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 24,656,923 shares of common stock outstanding on March 24, 2004.

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 SEC. 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 March 24, 2004. 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 Outstanding Shares
5% Stockholders		
Entities affiliated with Oracle Partners, L.P.[1] 200 Greenwich Avenue Greenwich, CT 06830	2,637,360	10.7%
Directors and Named Executive Officers		
J.R. Hyde, III[2]	9,590,207	38.9
Mitchell S. Steiner, M.D., F.A.C.S.[3]	5,662,147	23.0
Marc S. Hanover[4]	2,042,745	8.3
Mark E. Mosteller[5]	15,064	*
Henry P. Doggrell[6]	161,914	*
John H. Pontius[7]	973,055	3.9
Rosemary Mazanet, M.D., Ph.D	—	—
J. Kenneth Glass	2,000	*
Andrew M. Clarkson	25,000	*
All executive officers and directors as a group (9 persons)[8]	16,753,778	67.8

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

(1) Consists of 698,446 shares held by Oracle Partners, L.P., 1,764,297 shares held by Oracle Investment Management, Inc. and 174,617 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 91,628 shares held by Pittco Associates, L.P., an entity controlled by Mr. Hyde, 1,047,713 shares held by trusts with respect to which Mr. Hyde may be deemed to have beneficial ownership, 291,093 shares held by Memphis Biomed Ventures I, L.P., an entity controlled by Mr. Hyde, and 216,462 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 819,902 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 7,282 shares held by Mr. Mosteller's wife of which Mr. Mosteller disclaims beneficial ownership.

(6) Includes 96,104 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 March 24, 2004 through the exercise of stock options, 4,641 shares held by Mr. Doggrell's wife, and 1,000 shares held in a joint account with Mr. Doggrell's adult child, both of which Mr. Doggrell disclaims beneficial ownership.

(7) Includes 859,013 shares held by trusts of which Mr. Pontius is the trustee, 21,520 shares held by trusts of which Mr. Pontius' wife is the trustee and 46,261 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 March 24, 2004 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.

ITEM 13. CERTAIN RELATIONSHIPS AND RELATED TRANSACTIONS

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.

In August 2003, we issued and sold an aggregate of 329,536 shares of our 8% Series E Cumulative Convertible Preferred Stock to 11 accredited investors at $60.692 per share for an aggregate offering price of $20,000,199. In this transaction,

- Oracle Partners, L.P. and its affiliates, who own in excess of 5% of our outstanding voting securities, purchased approximately $1,000,000 of our Series E Preferred Stock, in the aggregate;
- Mr. Hyde, our largest stockholder and the Chairman of the Board of Directors, and his affiliate, Memphis Biomed Ventures I., L.P., purchased approximately $18,223,000 of our Series E Preferred Stock, in the aggregate;
- Mr. Pontius, a member of our Board of Directors, and his spouse purchased approximately $200,000 of our Series E Preferred Stock, in the aggregate;
- Equity Partners XII, LLC, which is an affiliate of Mr. Hanover, our President, purchased approximately $377,000 of our Series E Preferred Stock;
- Mr. Doggrell, our General Counsel and Secretary, and his spouse purchased approximately $100,000 of our Series E Preferred Stock; and

- Mr. Mosteller, our Chief Financial Officer, and his spouse purchased approximately $100,000 of our Series E Preferred Stock.

Registration Rights Agreements

We have entered into registration rights agreements with three of our 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, the holders of registration rights will be entitled to include their 11,407,917 shares of common stock in the related registration statement. In addition, the holders of approximately 11,116,824 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 to file a registration statement under the Securities Act with respect to their shares of common stock.

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.

ITEM 14. PRINCIPAL ACCOUNTANT FEES AND SERVICES

The Audit Committee of the Board of Directors has appointed Ernst & Young LLP as the Company's independent auditors to audit the financial statements of the Company and to perform other accounting services, if appropriate, for the year ending December 31, 2004.

Fees paid to Ernst & Young for services provided during the years ended December 31, 2003 and 2002, are presented below. We did not engage Ernst & Young to perform financial information systems design or implementation services during the year. "Audit Fees" include fees associated with the annual audits and our initial public offering. "Audit-Related Fees" include fees associated with assurance and related services related to the performance of the audit. "Tax Fees" include fees associated with tax compliance, tax advice, and tax planning.

	Audit Fees	Audit-Related Fees	Tax Fees	All Other Fees
2003	$540,000	$ 1,500	$5,196	$ —
2002	$ 21,500	$ —	$5,000	$ 194

The Audit Committee has pre-approved specific audit and professional services for the first quarter of 2004. The Audit Committee will pre-approve Ernst & Young's specific audit and professional services for the remainder of 2004 at the next scheduled Audit Committee meeting. Fees for other non-audit services arising during the year that are permissible under the SEC's rules on Auditor Independence must be pre-approved by the Audit Committee Chair, who must report such approvals at the next meeting of the Audit Committee.

PART IV

ITEM 15. EXHIBITS, FINANCIAL STATEMENT SCHEDULES, AND REPORTS ON FORM 8-K

(a)(1) The following documents are filed as part of this Annual Report in Form 10-K:

Number	Description
F-1	Index to Financial Statements
F-2	Report of Ernst & Young LLP, Independent Auditors
F-3	Balance Sheets at December 31, 2002 and 2003
F-4	Statements of Operations for the years ended December 31, 2001, 2002 and 2003 and for the cumulative period from inception to December 31, 2003
F-5	Statements of Cumulative Redeemable Convertible Preferred Stock and Stockholders' (Deficit) Equity for the period from inception to December 31, 2003, and for the years ended December 31, 2001, 2002 and 2003
F-6	Statements of Cash Flows for the years ended December 31, 2001, 2002 and 2003 and for the cumulative period from inception to December 31, 2003
F-7	Notes to Financial Statements

(a)(2) Financial statement schedules are omitted as they are not applicable.

(a)(3) See 14(c) below.

(b) None

(c) Exhibits

Number	Description
3.1	Restated Certificate of Incorporation of GTx, Inc. filed February 6, 2004, as amended(1)
3.2	Amended and Restated Bylaws of GTx, Inc.(1)
4.1	Reference is made to Exhibits 3.1 and 3.2
4.2	Specimen of Common Stock Certificate(1)
4.3	Amended and Restated Registration Rights Agreement between Registrant and Oracle Partners, L.P. dated August 7, 2003(1)
4.4	Amended and Restated Registration Rights Agreement between Registrant and J. R. Hyde, III dated August 7, 2003(1)
4.5	Amended and Restated Registration Rights Agreement between Registrant and Memphis Biomed Ventures dated August 7, 2003(1)
10.1	Genotherapeutics, Inc. 1999 Stock Option Plan(1)
10.2	GTx, Inc. 2000 Stock Option Plan(1)
10.3	GTx, Inc. 2001 Stock Option Plan(1)
10.4	GTx, Inc. 2002 Stock Option Plan(1)
10.5	2004 Equity Incentive Plan and Form of Stock Option Agreement(1)
10.6	2004 Non-Employee Directors' Stock Option Plan and Form of Stock Option Agreement(1)
10.7	Reserved
10.8	Employment Agreement dated October 1, 2003, between Registrant and Mitchell S. Steiner, M.D.(1)
10.9	Employment Agreement dated October 1, 2003, between Registrant and Marc S. Hanover(1)
10.10	Employment Agreement dated October 1, 2003, between Registrant and Mark E. Mosteller(1)

10.11	Employment Agreement dated October 1, 2003, between Registrant and Henry P. Doggrell(1)
10.12	Form of Indemnification Agreement(1)
10.13	Lease Agreement, dated March 7, 2001, between The University of Tennessee and TriStar Enterprises, Inc.(1)
10.14	Sublease Agreement dated October 1, 2000, as amended, between Registrant and TriStar Enterprises, Inc.(1)
10.15†	Amended and Restated License and Supply Agreement dated October 22, 2001, between Registrant and Orion Corporation(1)

Number	Description
10.16†	Amendment No. 1 to the License and Supply Agreement dated March 5, 2003, between Registrant and Orion Corporation(1)
10.17†	Production and Manufacturing Agreement dated September 9, 2002, between Registrant and ChemSyn Laboratories (Department of EaglePicher Technologies, LLC)(1)
10.18†	Amendment No. 1 to the Production and Manufacturing Agreement dated September 30, 2003, between Registrant and ChemSyn Laboratories (Department of EaglePicher Technologies, LLC)(1)
10.19†	Quotation Agreement dated August 8, 2003 between Registrant and EaglePicher Pharmaceutical Services(1)
10.20†	Amended and Restated Exclusive License Agreement dated June 3, 2002, between Registrant and University of Tennessee Research Foundation(1)
10.21†	Amended and Restated Exclusive License Agreement dated June 14, 2003, between Registrant and University of Tennessee Research Foundation(1)
10.22†	Amended and Restated Exclusive License Agreement dated August 30, 2003, between Registrant and University of Tennessee Research Foundation(1)
10.23	Amendment No. 2 to the License and Supply Agreement dated December 29, 2003, between Registrant and Orion Corporation(1)
14.1	Code of Ethics
23.1	Consent of Ernst & Young LLP
31.1	Certification of Chief Executive Officer Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
31.2	Certification of Chief Financial Officer Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
32.1	Certification of Chief Executive Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
32.2	Certification of Chief Financial Officer Pursuant to 18. U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002

† Confidential treatment requested. The redacted portions have been filed separately with the SEC as required by Rule 406 of Regulation C.

(1) Incorporated by reference to the same exhibit filed with GTx's Registration Statement on Form S-1 (File No. 333-109700).

(d) None

SIGNATURES

Pursuant to the requirements of Section 13 or 15(d) of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized.

GTx, Inc.

By /s/ Mitchell S. Steiner

Mitchell S. Steiner, M.D., F.A.C.S.
Chief Executive Officer, Vice Chairman and Director

Date: March 26, 2004

Pursuant to the requirements of the Securities Exchange Act of 1934, this report has been signed below by the following persons on behalf of the registrant and in the capacities and on the dates indicated.

		Date
/s/ J. R. Hyde, III J. R. Hyde, III	Chairman of the Board of Directors	March 26, 2004
/s/ Mitchell S. Steiner Mitchell S. Steiner, M.D. F.A.C.S.	Chief Executive Officer, Vice Chairman and Director	March 26, 2004
/s/ Mark E. Mosteller Mark E. Mosteller	Chief Financial Officer (Principal Financial and Accounting Officer)	March 26, 2004
/s/ Marc S. Hanover Marc S. Hanover	President and Director	March 26, 2004
/s/ Andrew M. Clarkson Andrew M. Clarkson	Director	March 26 2004
/s/ J. Kenneth Glass J. Kenneth Glass	Director	March 26, 2004
/s/ Rosemary Mazanet Rosemary Mazanet, M.D., Ph.D.	Director	March 26, 2004
/s/ John H. Pontius John H. Pontius	Director	March 26 2004

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, 2003 and 2002, and the related statements of operations, cumulative redeemable convertible preferred stock and stockholders' (deficit) equity and cash flows for each of the three years in the period ended December 31, 2003 and for the period from September 24, 1997 (date of inception) through December 31, 2003. 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 audit 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.

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, 2003 and 2002, and the results of its operations and its cash flows for each of the three years in the period ended December 31, 2003 and for the period from September 24, 1997 (date of inception) through December 31, 2003, in conformity with accounting principles generally accepted in the United States.

/s/ Ernst & Young LLP

Memphis, Tennessee
January 30, 2004,
except for Note 13, as to which
the date is March 16, 2004

GTx, Inc.
(a development stage company)
BALANCE SHEETS
(in thousands, except share data)

	December 31,		Pro Forma December 31,
	2002	2003	2003
			(unaudited)
ASSETS			
Current assets:			
Cash and cash equivalents	$ 8,925	$ 14,769	$ 14,769
Inventory	—	194	194
Prepaid expenses	41	61	61
Total current assets	8,966	15,024	15,024
Property and equipment, net	1,064	815	815
Deferred initial public offering costs	—	1,471	1,471
Total assets	$ 10,030	$ 17,310	$ 17,310
LIABILITIES, CUMULATIVE CONVERTIBLE PREFERRED STOCK AND STOCKHOLDERS' (DEFICIT) EQUITY			
Current liabilities:			
Accounts payable	$ 601	$ 461	$ 461
Accrued expenses	711	1,788	1,788
Total current liabilities	1,312	2,249	2,249
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 $1,889 at December 31, 2002 and $2,017 at December 31, 2003	13,855	25,763	—
Series B, $0.001 par value; 277,500 shares authorized, issued and outstanding at all periods, liquidation value of $5,989 at December 31, 2002 and $6,429 at December 31, 2003	19,671	37,129	—
Series C, $0.001 par value; 450,000 shares authorized, 260,154 issued and outstanding at all periods, liquidation value of $16,496 at December 31, 2002 and $17,817 at December 31, 2003	19,102	37,955	—
Series D, $0.001 par value; 300,000 shares authorized, 164,765 issued and outstanding at December 31, 2003 and 2002, liquidation value of $11,398 at December 31, 2002 and $12,310 at December 31, 2003	11,398	22,681	—
Series E, $0.001 par value; 450,000 shares authorized, 329,536 issued and outstanding at December 31, 2003; liquidation value of $20,636 at December 31, 2003	—	41,764	—
Total cumulative redeemable convertible preferred stock	64,026	165,292	—
Commitments and contingencies			
Stockholders' (deficit) equity:			
Common stock, $0.001 par value: 60,000,000 shares authorized; 7,734,998 shares issued and outstanding at December 31, 2002 and 7,735,848 shares issued and outstanding at December 31, 2003; 19,192,753 shares outstanding on a pro forma basis (unaudited)	8	8	19
Deferred stock compensation	—	(3,505)	(3,505)
Additional paid-in capital	962	5,018	170,567
Deficit accumulated during the development stage	(56,278)	(151,752)	(152,020)
Total stockholders' (deficit) equity	(55,308)	(150,231)	15,061
Total liabilities and stockholders' (deficit) equity	$ 10,030	$ 17,310	$ 17,310

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,			Cumulative period from September 24, 1997 (date of inception) to December 31, 2003
	2001	2002	2003	
Operating expenses:				
Research and development	$ 5,744	$ 9,285	$ 10,468	$ 28,879
General and administrative	2,187	2,405	3,512	9,742
Depreciation	215	332	357	1,048
Total operating expenses	8,146	12,022	14,337	39,669
Other income:				
Research and development income	—	—	—	225
Interest income	83	156	143	643
Total other income	83	156	143	868
Net loss	(8,063)	(11,866)	(14,194)	(38,801)
Accrued preferred stock dividends	(790)	(2,147)	(3,436)	(6,753)
Adjustments to preferred stock redemption value	(57)	(7,220)	(77,844)	(106,198)
Net loss attributable to common stockholders	$ (8,910)	$ (21,233)	$ (95,474)	$ (151,752)
Net loss per share attributable to common stockholders, basic and diluted	$ (1.15)	$ (2.75)	$ (12.34)	
Weighted average shares used in computing net loss per share attributable to common stockholders, basic and diluted	7,734,998	7,734,998	7,735,125	
Pro forma net loss per share attributable to common stockholders, basic and diluted (unaudited)			$ (.83)	
Shares used in computing pro forma net loss per share attributable to common stockholders, basic and diluted (unaudited)			17,018,655	

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' (DEFICIT) EQUITY
For the Period From September 24, 1997 (date of inception) To December 31, 2003
(in thousands, except share and per share data)

	Cumulative Redeemable Convertible Preferred Stock		Stockholders' (Deficit) Equity					
			Common Stock		Deferred Stock Compensation	Additional Paid-in Capital	Deficit Accumulated During the Development Stage	Total Stockholders' (Deficit) Equity
	Shares	Amount	Shares	Amount				
Balances at September 24, 1997	—	$ —	—	$ —	$ —	$ —	$ —	$ —
Issuance of common stock	—	—	7,649,998	—	—	—	—	—
Balances as of December 31, 1997	—	—	7,649,998	—	—	—	—	—
Issuance of common stock	—	—	850,000	8	—	962	—	970
Net loss	—	—	—	—	—	—	(116)	(116)
Balances as of December 31, 1998	—	—	8,499,998	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 as of December 31, 1999	200,000	1,538	8,499,998	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 as of December 31, 2000	477,500	27,912	7,734,998	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	7,734,998	8	—	962	(35,045)	(34,075)
Sale of Series D Redeemable Convertible Preferred Stock at $66.762, net of issuance costs of $43	164,765	10,957	—	—	—	—	—	—
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	7,734,998	8	—	962	(56,278)	(55,308)
Issuance of Common Stock			850	—	—	1	—	1
Sale of Series E Redeemable Convertible Preferred Stock at $60.692, net of issuance costs of $14	329,536	19,986	—	—	—	—	—	—
Preferred stock dividends	—	3,436	—	—	—	—	(3,436)	(3,436)
Preferred stock adjustment to redemption value	—	77,844	—	—	—	—	(77,844)	(77,844)
Deferred stock-based compensation	—	—	—	—	(4,055)	4,055	—	—

Amortization of stock-based compensation	—	—	—	—	550	—	—	550
Net loss	—	—	—	—	—	—	(14,194)	(14,194)
Balances at December 31, 2003	1,231,955	$165,292	7,735,848	$ 8	$ (3,505)	$ 5,018	$ (151,752)	$ (150,231)

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,			Cumulative period from September 24, 1997 (date of inception) to December 31, 2003
	2001	2002	2003	
Cash flows from operating activities:				
Net loss	$ (8,063)	$(11,866)	$(14,194)	$ (38,801)
Adjustments to reconcile net loss to net cash used in operating activities:				
Depreciation	215	332	357	1,048
Stock-based compensation expense	—	—	550	550
Changes in assets and liabilities:				
Inventory	(154)	154	(194)	(194)
Prepaid expenses	(18)	5	(20)	(61)
Accounts payable	261	340	(153)	448
Accrued expenses	(225)	482	657	1,368
Net cash used in operating activities	(7,984)	(10,553)	(12,997)	(35,642)
Cash flows from investing activities:				
Purchase of property and equipment	(792)	(313)	(108)	(1,863)
Net cash used in investing activities	(792)	(313)	(108)	(1,863)
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	—	—	1	971
Proceeds from issuance of preferred stock, net	14,943	10,957	19,986	52,341
Deferred initial public offering costs	—	—	(1,038)	(1,038)
Net cash provided by financing activities	14,943	10,957	18,949	52,274
Net increase in cash and cash equivalents	6,167	91	5,844	14,769
Cash and cash equivalents, beginning of period	2,667	8,834	8,925	—
Cash and cash equivalents, end of period	$ 8,834	$ 8,925	$ 14,769	$ 14,769
Supplemental schedule of non-cash investing and financing activities:				
Preferred stock dividends	$ 790	$ 2,147	$ 3,436	$ 6,753
Preferred stock adjustment to redemption value	$ 57	$ 7,220	$ 77,844	$ 106,198
Deferred initial public offering costs	$ —	$ —	$ 433	$ 433

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™(Toremifene Citrate) tablets, 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. In March 2004 (see Note 13), we entered into a joint collaboration and license agreement with Ortho Biotech Products L.P. for the continued clinical development of our second product candidate, andarine and specified backup SARM compounds. Andarine is the most advanced of our internally discovered portfolio of compounds designed to modulate the effects of hormones. Together with Ortho Biotech, we intend to continue to pursue the clinical development of andarine for the treatment of cachexia from various types of cancer and other chronic diseases. Cancer cachexia is a muscle wasting condition that is a potentially life-threatening complication of many cancers. 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 or outside the United States as we have done with Ortho Biotech for andarine and specified backup SARM compounds.

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 (see Note 13) 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, 2003, 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.

Unaudited Pro Forma Balance Sheet Information

The unaudited pro forma balance sheet information includes the pro forma effects of the automatic conversion into common stock of all outstanding shares of preferred stock and dividends accrued thereon through December 31, 2003 upon the closing of the Company's initial public offering as if the offering occurred on December 31, 2003 (see Notes 5 and 13).

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.

GTx, Inc.
(a development stage company)

NOTES TO FINANCIAL STATEMENTS
(in thousands, except share and per share data)

Preferred Stock Redemption Value

Unless the preferred stock is previously converted into common stock pursuant to mandatory or optional conversion features governing the preferred stock, the preferred stock is subject to redemption on or after August 31, 2006, at the option of the preferred shareholder. The per share redemption price is equal to the greater of 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. At December 31, 2003, the per share redemption value was determined based on the estimated projected midpoint on the range of the company's initial public offering price per common share.. The changes in redemption value affect the loss attributable to common stockholders (see Notes 5 and 13).

Cash and Cash Equivalents

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

Inventory

Acapodene inventory consists of a drug that is manufactured by Orion Corporation 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.

Deferred Initial Public Offering Costs

Deferred initial public offering costs represent professional fees incurred in connection with the filing of a registration statement with the Securities and Exchange Commission for the sale of shares of the Company's common stock (see Note 13).

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, 2002 and 2003. 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.

GTx, Inc.
(a development stage company)

NOTES TO FINANCIAL STATEMENTS
(in thousands, except share and per share data)

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 $8,625 and $14,632 at December 31, 2002 and 2003, respectively.

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*

GTx, Inc.
(a development stage company)

NOTES TO FINANCIAL STATEMENTS
(in thousands, except share and per share data)

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.

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,		
	2001	2002	2003
Net loss attributable to common stockholders, as reported	$(8,910)	$(21,233)	$(95,474)
Add: Employee stock-based compensation expense included in reported net earnings	—	—	550
Deduct: Employee stock-based compensation determined under fair value method	(37)	(115)	(424)
Adjusted net loss attributable to common stockholders	$(8,947)	$(21,348)	$(95,348)
Pro forma SFAS 123 disclosure:			
Net loss attributable to common stockholders per common share:			
As reported, basic and diluted	$ (1.15)	$ (2.75)	$ (12.34)
As adjusted, basic and diluted	$ (1.16)	$ (2.76)	$ (12.33)

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.

GTx, Inc.
(a development stage company)

NOTES TO FINANCIAL STATEMENTS
(in thousands, except share and per share data)

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

	Years Ended December 31,		
	2001	2002	2003
Basic net loss per share attributable to common shareholders:			
Numerator			
Net loss attributable to common stockholders	$ (8,910)	$ (21,233)	$ (95,474)
Denominator			
Weighted average common shares outstanding	7,734,998	7,734,998	7,735,125
Net loss per share attributable to common stockholders, basic and diluted	$ (1.15)	$ (2.75)	$ (12.34)
Pro Forma			
Net loss as reported			$ (14,194)
Shares used above			7,735,125
Pro forma adjustments to reflect assumed weighted average effect of conversion of preferred stock (unaudited)			9,283,530
Shares used in computing pro forma net loss per share attributable to common stockholders, basic and diluted (unaudited)			17,018,655
Pro forma basic and diluted net loss per share (unaudited)			$ (0.83)

Pro forma net loss per share for the year ended December 31, 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 Company's initial public offering (see Note 13) as if such conversion occurred on 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 9,283,530 shares for the year ended December 31, 2003. 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.

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,		
	2001	2002	2003
Shares issuable upon exercise of stock options	328,100	363,375	828,750
Shares issuable upon conversion of convertible preferred stock	6,442,660	8,151,679	11,456,912
	6,770,760	8,515,054	12,285,662

GTx, Inc.
(a development stage company)

NOTES TO FINANCIAL STATEMENTS
(in thousands, except share and per share data)

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 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 March 15, 2004. The Company does not have any ownership in any variable interest entities as of December 31, 2003. 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 impact of SFAS No. 150 and has determined that its financial instruments (common stock and preferred stock) will not be affected.

3. Property and Equipment

Property and equipment consist of the following:

	December 31,	
	2002	**2003**
Leasehold improvements	$ 113	$ 113
Equipment	1,494	1,575
Furniture and fixtures	114	141
	1,721	1,829
Less: accumulated depreciation	657	1,014
	$1,064	$ 815

Depreciation expense for the years ended December 31, 2001, 2002 and 2003 was $215, $332 and $357, respectively, and $1,048 for the period from September 24, 1997 (date of inception) through December 31, 2003.

GTx, Inc.
(a development stage company)

NOTES TO FINANCIAL STATEMENTS
(in thousands, except share and per share data)

4. Accrued Expenses

Accrued expenses consist of the following:

	December 31,	
	2002	2003
Travel	$ —	$ 20
Professional fees	—	522
Research and development	246	667
Clinical trial	449	550
Other	16	29
	$711	$1,788

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 shares 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.

In 2003, the Company authorized 450,000 shares and issued 329,536 shares of Series E Cumulative Convertible Redeemable Preferred Stock ("Series E"). 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%. 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.

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.

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, Series D and Series E are as follows:

- Shares of Series A, Series A-2, Series B, Series B-2, Series C, Series D and Series E 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, Series D is $66.76 and Series E is $60.69, in each case, plus accrued dividends. The preferred stock per share redemption value was $57.66, $66.76 and $122.83 as of December 31, 2001, 2002 and 2003, respectively.

GTx, Inc.
(a development stage company)

NOTES TO FINANCIAL STATEMENTS
(in thousands, except share and per share data)

- Shares of Series A, Series A-2, Series B, Series B-2, Series C, Series D and Series E 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 (1) 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). The number of shares issuable upon conversion will be determined by dividing the applicable aggregate liquidation value by the applicable conversion price. The per share conversion price for shares of Series A, Series B, Series C, Series D and Series E is equal to their initial per share liquidation value. 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. The per share conversion prices for shares of preferred stock are as follow: 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, Series D and Series E 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, Series D and Series E 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, Series D for Series D and Series E for Series E 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, Series D and Series E 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, Series A-2, Series B. Series B-2, Series C, Series D or Series E 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.

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.

GTx, Inc.
(a development stage company)

NOTES TO FINANCIAL STATEMENTS
(in thousands, except share and per share data)

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

	December 31, 2003
For conversion of Series A	1,782,841
For conversion of Series B	2,569,389
For conversion of Series C	2,626,551
For conversion of Series D	1,588,080
For conversion of Series E	2,890,051
Outstanding employee stock options	828,750
Possible future issuance under stock option plans	453,050
	12,738,712

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.

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 composition of matter and Restated Exclusive License Agreements with UTRF granting the Company worldwide exclusive licenses under its composition of matter and 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 Androgen Receptor Targeting Agents ("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.

GTx, Inc.
(a development stage company)

NOTES TO FINANCIAL STATEMENTS
(in thousands, except share and per share data)

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, which was amended and restated in 2001 and further amended in 2003. 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. Under the Company's agreement with Orion, it has agreed to achieve specified minimum sales requirements of Acapodene in the U.S. after commercialization of the product or it must pay Orion based on the amount of the shortfall. In addition, the Company is required to pay up to $1,000 if the Company is acquired before receiving marketing approval for the use of Acapodene in the licensed field. Orion may terminate its supply agreement if marketing approval for Acapodene is not granted in the U.S. by December 31, 2007 or upon the expiration of Orion's last valid patent for Toremifene in the U.S.

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,		
	2001	2002	2003
Deferred income tax assets:			
Net federal and state operating loss carryforwards	$ 4,906	$ 9,134	$ 14,795
Research credits	390	783	1,241
Cash basis method	84	496	778
Deferred stock compensation	—	—	215
Total deferred tax assets	5,380	10,413	17,029
Deferred income tax liabilities:			
Depreciation	31	50	35
Total deferred tax liabilities	31	50	35
Net deferred income tax assets	5,349	10,363	16,994
Valuation allowance	(5,349)	(10,363)	(16,994)
	$ —	$ —	$ —

At December 31, 2003, the Company has net federal and state operating loss carryforwards of approximately $37.7 million each, which expire for federal purposes from 2018 through 2023 and for state purposes from 2013 to 2018, and research credits of $1.2 million, which expire from 2018 through 2023. 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. The Company has reduced its deferred tax assets by a a valuation allowance after considering available evidence concerning the realizations of these assets, including the development stage nature of its operations.

GTx, Inc.
(a development stage company)

NOTES TO FINANCIAL STATEMENTS
(in thousands, except share and per share data)

10. Operating Leases

The Company leases laboratory facilities and office space pursuant to leases accounted for as operating leases. Rent expense was approximately $155, $170 and $184 for the years ended December 31, 2001, 2002 and 2003, respectively and $585 for the period from September 24, 1997 (date of inception) to December 31, 2003.

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 24,650 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. At December 31, 2002 and 2003, respectively, 34,000 and 101,269 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	533,375	6.24
Options forfeited	(67,150)	4.08
Options exercised	(850)	0.94
Balances at December 31, 2003	828,750	$ 6.18

GTx, Inc.
(a development stage company)

NOTES TO FINANCIAL STATEMENTS
(in thousands, except share and per share data)

The following table summarizes information about stock options outstanding at December 31, 2003:

Options Outstanding				Options Exercisable	
Exercise Price	Number Outstanding	Weighted Average Remaining Contractual Life (years)	Weighted Average Exercise Price	Number Exercisable	Weighted Average Exercise Price
$2.24	48,875	7.17	$ 2.24	16,269	$ 2.24
$6.24	518,500	9.00	6.24	34,000	6.24
$6.78	252,875	7.81	6.78	51,000	6.78
$7.85	8,500	8.75	7.85	—	—
	828,750	8.51	$ 6.18	101,269	$ 5.87

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 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 recorded non-cash deferred stock-based compensation expense of $4.1 million 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 $37 and $115 for the years ended December 31, 2001 and 2002, respectively, and decreased by $126 for the year ended December 31, 2003. 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 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, a weighted average risk-free interest rate of 4.76% and a weighted average expected life of 8 years for the 2002 grants and a weighted average risk-free interest rate of 4.28% and a weighted average expected life of 8 years for the 2003 grants. The weighted average grant date fair value of options granted were $1.99, $2.26 and $8.02 for the years ended December 31, 2001, 2002 and 2003, 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 a statutory limit of their pre-tax compensation which was $12,000 in calendar year 2003.. 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.

13. Subsequent Events

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

GTx, Inc.
(a development stage company)

NOTES TO FINANCIAL STATEMENTS
(in thousands, except share and per share data)

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.

2004 Option Plans

On January 14, 2004, the Company adopted its 2004 Equity Incentive Plan and 2004 Non-Employee Directors' Stock Option Plan, both of which became 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.

Initial Public Offering

On February 6, 2004, the Company completed an initial public offering ("IPO") of 5.4 million shares of common stock at a price of $14.50 per share. Concurrent with the IPO, the Company amended its Certificate of Incorporation, authorizing 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. The number of authorized shares in the accompanying balance sheets have been adjusted to reflect the 60,000,000 authorized shares of common stock. Additionally, concurrent with the IPO, all outstanding preferred stock and accrued dividends were converted into 11,521,075 shares of common stock. After the IPO, the Company has outstanding 24,656,923 shares of common stock.

Collaboration, License and Co-Promotion Agreement

In March 2004, we entered into a joint collaboration and license agreement with Ortho Biotech Products L.P., a wholly owned subsidiary of Johnson & Johnson. Under the agreement, we will receive an upfront licensing fee of $6 million, be reimbursed for development expenses of approximately $687,000 for our recently completed Phase Id clinical trial for andarine and receive additional licensing fees and milestone payments up to $82 million based on andarine and up to $45 million for each additional licensed compound achieving specific clinical development decisions or obtaining regulatory approvals. Johnson & Johnson Pharmaceutical Research & Development will be responsible for further clinical development and expenses related to andarine and other licensed SARM compounds. Ortho Biotech will be responsible for commercialization and expenses related to andarine and other licensed SARM compounds. If andarine is approved for commercial sale, Ortho Biotech will exclusively market andarine in the United States and markets outside the United States. Under the agreement, we have the option to co-promote andarine and the other licensed SARM compounds to urologists in the United States for indications specifically related to men's health. We will receive royalties on all sales, as well as an additional royalty on all co-promoted sales generated from urologists in the United States.

GTx, Inc.
(a development stage company)

NOTES TO FINANCIAL STATEMENTS
(in thousands, except share and per share data)

Note 14. Quarterly Financial Data (Unaudited)

The following is a summary of the quarterly results of operations for the years ended December 31, 2002 and 2003.

	Quarters Ended Year 2002			
2002	**Mar. 31**	**June 30**	**Sept. 30**	**Dec. 31**
Operating expenses:				
Research and development	$ 1,718	$ 2,257	$ 2,433	$ 2,877
General and administrative	416	689	725	575
Depreciation	79	74	87	92
Total operating expenses	2,213	3,020	3,245	3,544
Other income	32	23	50	51
Net loss	(2,181)	(2,997)	(3,195)	(3,493)
Accrued preferred stock dividends	(429)	(429)	(608)	(681)
Adjustments to preferred stock redemption value	—	(7,036)	(111)	(73)
Net loss attributable to common stockholders	$(2,610)	$(10,462)	$(3,914)	$(4,247)
Basic and diluted net loss per share	$ (0.34)	$ (1.35)	$ (0.51)	$ (0.55)

	Quarters Ended Year 2003			
2003	**Mar. 31**	**June 30**	**Sept. 30**	**Dec. 31**
Operating expenses:				
Research and development	$ 2,113	$ 2,590	$ 2,420	$ 3,345
General and administrative	610	801	928	1,173
Depreciation	87	88	89	93
Total operating expenses	2,810	3,479	3,437	4,611
Other income	29	14	36	64
Net loss	(2,781)	(3,465)	(3,401)	(4,547)
Accrued preferred stock dividends	(683)	(683)	(934)	(1,136)
Adjustments to preferred stock redemption value	(73)	4,809	(81,402)	(1,178)
Net (loss) income attributable to common stockholders	$(3,537)	$ 661	$(85,737)	$(6,861)
Basic net (loss) income per share	$ (0.46)	$ 0.09	$ (11.08)	$ (0.89)
Diluted net loss per share	$ (0.46)	$ (0.22)	$ (11.08)	$ (0.89)

EXHIBIT 14.1

GTx, INC.

CODE OF BUSINESS CONDUCT AND ETHICS

.
.
.

GTx, INC.
CODE OF BUSINESS CONDUCT AND ETHICS

GTx, INC.
CODE OF BUSINESS CONDUCT AND ETHICS

I. INTRODUCTION

GTx, Inc. (the "Company" or "GTx") is committed to achieving high standards of business and personal ethical conduct for itself, the members of its Board of Directors and all GTx personnel. Through performance in accordance with these standards, GTx, its Directors and all of its employees will earn and

enjoy the respect of one another, the business and scientific research communities, our consultants, contractors, suppliers, scientific collaborators and the public.

It is the personal responsibility of all Directors and employees to be familiar with all legal and policy standards and restrictions applicable to their duties and responsibilities, and to conduct themselves accordingly. In addition to the strictly legal aspects involved, all Directors and employees are expected to observe high standards of business and personal ethics in the discharge of their duties. This Code of Business Conduct and Ethics (the "Code") is designed to help ensure this.

This Code applies to all Directors and employees of GTx in all places. "Employees" means an officer or employee of GTx and its affiliates, and it includes Executive Officers, unless otherwise stated. Certain parts of this Code may apply specifically to "Executive Officers," and are so indicated. "Executive Officer" means a member of GTx management so designated by resolution of the Board of Directors. All employees and Directors are required to read and understand this Code, and compliance with the policies set forth herein is required of all personnel.

This Code is intended to comply with the Nasdaq Stock Market listing standards and the Sarbanes-Oxley Act of 2002. Directors and employees are encouraged to report violations, or suspected violations, of laws, regulations, or this Code using the processes described in Article IX of this Code or as otherwise provided for by the Board of Directors. GTx will not permit retaliation against Directors or employees for reports made in good faith.

II. COMPLIANCE OFFICER

In order to help ensure compliance with this Code, GTx has appointed a Compliance Officer who is GTx's General Counsel. The Compliance Officer will have the following duties:

1. Coordinate periodic reviews and update this Code as necessary;

2. Ensure that each new employee is given a copy of this Code immediately after employment and that each such employee signs an acknowledgment that he or she has read, understands and supports this Code;

3. Maintain records related to this Code; and

4. Perform such other activities as may be reasonably related to the foregoing or are required to ensure a successful application of the program contemplated by this Code.

The Compliance Officer shall make periodic reports to GTx's Chief Executive Officer and Board of Directors concerning compliance with these requirements.

III. CONFLICTS OF INTEREST

A. INTRODUCTION

For purposes of our Code, a "conflict of interest" occurs when an individual's private interests interfere in a material way or appear from the perspective of a reasonable person to interfere in a material way with the interests of GTx as a whole. A conflict situation can arise when an employee or Director takes actions or has interests that may make it difficult to perform his or her responsibilities objectively and effectively. Ordinarily, a conflict exists when an outside interest could actually or potentially influence the judgment or actions of an individual in the conduct of GTx's business. Conflicts of interest may also arise when an employee or Director, or a member of his or her family, receives improper personal benefits as a result of his or her position at GTx.

B. GENERAL POLICY

GTx must have the confidence of its consultants, contractors, suppliers, scientific collaborators and the public. Directors and employees must avoid conflicts or the appearance of conflicts, as discussed above.

Specifically, employees should avoid any outside financial interests that might conflict with GTx's interests. Such outside interests could include, among other things:

1. Personal or family financial interests in, or indebtedness to, enterprises that have business relations with GTx, such as relatives who are employed by or own an interest in consultants or suppliers.

2. Acquiring any interest in outside entities, properties, etc., in which GTx has an interest or potential interest.

3. Conduct of any business not on behalf of GTx with any consultant, contractor, supplier, scientific collaborator or any of their officers or employees, including service as a director or officer of, or employment or retention as a consultant by, such persons.

Employees should report any material transaction or relationship that could result in a conflict of interest to GTx's Compliance Officer, or through such other processes as may be established by the Board of Directors.

C. SERVING AS A DIRECTOR, OFFICER OR EMPLOYEE OF ANOTHER BUSINESS

GTx expects its employees to devote their full energies to their work. Therefore, an employees' outside activities must not give rise to a real or apparent conflict of interest with the employee's duties with GTx. Employees must be alert to potential conflicts of interests and be aware that they may be asked to discontinue any outside activity should such a conflict arise.

GTx employees must have written approval from the Compliance Officer in advance of accepting an appointment or position to serve as a director, partner, owner, officer or employee of any non-GTx business. Employees should submit in writing any requests for approval to the Compliance Officer stating the name and address of the proposed employer, the nature of the position and the expected hours of employment. If the service is permitted, then any employee acting in this dual capacity must inform the Compliance Officer of any matter affecting this dual responsibility at any time. Under no circumstances shall a Director or employee engage in any activity that competes with GTx.

Notwithstanding the foregoing, volunteering in civic and charitable organizations is encouraged for GTx employees. To serve as a director or officer of a charitable or civic organization, an employee must obtain written approval from the Compliance Officer in advance of accepting the appointment. Participation in such activities shall not be deemed to be within an individual's scope of employment or authority as an employee, and GTx assumes no liability therefor.

Directors are not considered employees of GTx and are not limited as to their outside employment by the provisions of this Section C. Directors who accept nominations to serve as directors of other public companies shall, in cases where such nominations have not previously been disclosed, notify GTx's Board of Directors in writing.

D. ACCEPTANCE OF GIFTS AND OTHER FAVORS

The general purpose of gifts and favors in a business context is to create goodwill. If they do more than that, and appear to have the potential to unduly influence judgment or create a feeling of obligation, employees should not accept them. Employees may not solicit any kind of gift or personal benefit from present or potential consultants, contractors, suppliers or scientific collaborators. Employees are prohibited from accepting gifts of money (or monetary equivalents) or gifts that would be viewed as expensive or extraordinary by a reasonable person, whether solicited or unsolicited, from consultants, contractors, suppliers or scientific collaborators. Notwithstanding the foregoing, the following transactions are permitted and shall be considered an exception to the general prohibition against accepting things of value:

1. Acceptance of gifts, gratuities, amenities or favors based on obvious family or personal relationships (such as those with parents, children or spouse) when the circumstances make it clear that it is those relationships, rather than the business

of GTx, that are the motivating factors;

2. Acceptance of meals, refreshments, travel arrangements or accommodations, or entertainment, all of reasonable value, in the course of a meeting or other occasion, the purpose of which is to hold bona fide business discussions or to foster better business relations, provided that the expense would be paid for by GTx as a reasonable business expense if not paid for by another party;

3. Acceptance of advertising or promotional material of reasonable value such as pens, pencils, note pads, key chains, calendars and similar items;

4. Acceptance of gifts of reasonable value related to commonly recognized events or occasions, such as a promotion, new job, wedding, retirement or holiday; or

5. Acceptance of civic, charitable, education, or religious organizational awards for recognition of service and accomplishment.

If there is any doubt regarding acceptability, the item should be refused or returned. In the case of a perishable gift, it may be contributed to a charitable organization in the donor's name. Also, the donor should receive written notification of the return or disposal of the gift and a reminder of GTx's policies, and GTx's Compliance Officer should be copied on such correspondence. If you encounter situations in which you are not sure of your obligations, you should consult GTx's Compliance Officer.

Conversely, GTx will not tolerate any employee giving any gift, bribe, kickback, favor or any other item to anyone doing business with, or anyone who may do business with, GTx with the intent of influencing that party in a transaction or potential transaction with GTx.

It is inevitable and desirable that you may have individual business and personal relationships with GTx's consultants, contractors, suppliers, scientific collaborators and others who do business with GTx even though such individual business and personal relationship is not connected with GTx's business. This policy is not intended to discourage such relationships. Any such business relationship should be on customary terms and for proper and usual purposes.

E. POTENTIAL CONFLICTS BY FAMILY AND FRIENDS

There may be situations where the actions of family members and close personal friends may cause an employee a conflict of interest. For example, gifts or other benefits offered to an employee's family member by contractors or suppliers or potential contractors or suppliers are considered business gifts and are treated the same as if they were given to the employee. If an employee's spouse or relative is directly involved in a business that would like to provide products or services to GTx, the employee cannot use his or her position at GTx to influence the bidding process or negotiation in any way. If an employee's spouse or relative is a competitor of GTx, or is employed by one, you must disclose the situation to the Compliance Officer so GTx may assess the nature and extent of any conflict and how it can be resolved.

F. POLITICAL ACTIVITIES

It is GTx's policy to comply with all laws relating to elections, voting and the political process. No employee of GTx, acting on GTx's behalf, may contribute or loan money or items of value to any foreign, federal, state or local political candidates or parties. Employees may, however, participate in and/or contribute to the political process as concerned individuals, through means which would include voting and the contribution of their own time and money, and participate in or make contributions to political action committees.

G. BRIBERY

Federal law and the laws of most states prohibit bribery, which is the act of giving anything of value to public officials with the corrupt intent of influencing an official act. These laws also prohibit unlawful gratuities, which is the act of giving or promising something of value to a public official because of an official act, either before or after the act has been done. Employees should clearly avoid even the appearance of such "quid pro quo" arrangements. Employees also shall observe all applicable United States and foreign laws, including the Anti-Kickback Act and the Foreign Corrupt Practices Act. No gifts or business entertainment of any kind may be given to any government employee, whether or not there is an intent to influence, without the prior approval of GTx's Compliance Officer.

IV. PROHIBITION ON TAKING CORPORATE OPPORTUNITIES OF GTx

Directors and employees of GTx owe a fiduciary duty to GTx and must advance its legitimate interests when possible. It is a breach of this duty for any such person to take advantage of a business or investment opportunity for his or her own or another person's personal profit or benefit when the opportunity is within the corporate powers of GTx and when the opportunity is of present or potential practical advantage to GTx. If such a person so appropriates a GTx corporate opportunity, GTx may claim the benefit of the transaction or business and such person exposes himself or herself to liability. It is GTx's policy that no Director or employee take a GTx corporate opportunity without the consent of the Board of Directors.

V. BUSINESS CONDUCT AND FAIR DEALING

A. GENERAL POLICY

GTx expects that all Directors and employees will perform their duties in a professional manner, in good faith using prudent judgment and in the best interests of GTx. Each GTx employee and Director must endeavor to deal fairly with GTx's consultants, contractors, suppliers, scientific collaborators, competitors and other employees. No employee or Director shall take unfair advantage of anyone through manipulation, concealment, abuse of privileged or confidential information, misrepresentation of a material fact or any other unfair-dealing practice.

B. DEALINGS WITH COMPETITORS

GTx is committed to fair competition. GTx seeks competitive advantages through superior performance, never through unethical or illegal business practices, stealing proprietary information, possessing or utilizing trade secret information that was obtained without the owner's consent or inducing such disclosures by past or present employees of other companies. The most important laws governing competitive practices in the United States are the federal anti-trust laws, which are designed to protect economic freedoms and promote competition. It is GTx's policy to fully comply with the anti-trust laws.

C. DEALINGS WITH CONSULTANTS, CONTRACTORS, SUPPLIERS, SCIENTIFIC COLLABORATORS AND OTHER PARTIES DOING BUSINESS WITH GTx

1. Transactions with consultants, contractors, suppliers and scientific collaborators shall always be conducted at "arm's length."

2. No employee shall misrepresent, circumvent, or conceal the nature of any material aspect of any transaction when dealing with a party doing business with GTx.

3. If a relationship between an employee and a party doing business with GTx or a party that might do business with GTx exists, which potentially creates a conflict of interest, that employee shall remove himself/herself from all dealings with that party.

VI. CONFIDENTIAL INFORMATION AND PRESERVATION OF RECORDS

Much of the information developed by GTx, especially in research, is original, and its protection is essential to the continued success of GTx. Employees frequently have access to confidential information concerning GTx's business and the business of those entities who do business with GTx. Confidential information includes all non-public information, including trade

secrets and other proprietary information, that might be of use to competitors or harmful to GTx or its affiliates if disclosed. Safeguarding confidential information is essential to the conduct of the business of GTx. Caution and discretion must be exercised in the use of such information, which should be shared only with those who have a clear and legitimate need and right to know.

Employees shall maintain the confidentiality of GTx's business information, proprietary information and information relating to GTx's consultants, contractors, suppliers and scientific collaborators. Employees shall not use such information except for uses that are appropriate for GTx's business. Information regarding a consultant, contractor, supplier or scientific collaborator may not be released to third parties or government or other organizations, without the written consent of the consultant, contractor, supplier or scientific collaborator, unless required or permitted by law.

It is GTx's policy to cooperate with all reasonable requests from government authorities. Whenever an employee becomes aware of an investigation which affects GTx or an entity doing business with GTx, or receives a request for information from a government authority, other than routine items requested in the ordinary course of business, he or she shall immediately notify GTx's Compliance Officer. Notwithstanding any GTx records retention guidelines, under no circumstances shall any records known to be the subject of or germane to any anticipated, threatened or pending lawsuit or governmental or regulatory investigation or case filed in bankruptcy be removed, concealed, altered or destroyed. For purposes of this section, "records" means any hard copy, paper documents and electronic records, including, but not limited to, e-mail, voicemail and the contents of hard drives.

Furthermore, all audit and audit review work papers shall be retained as required, in accordance with the rules promulgated by the Securities and Exchange Commission (the "SEC") under the Sarbanes-Oxley Act of 2002.

VII. PROTECTION AND USE OF GTX PROPERTY

A. GTx PROPERTY

Employees and Directors have a duty to protect and conserve GTx property and to insure its continued use for proper purposes. All GTx assets shall be used for legitimate business purposes and not for personal gain. Employees of GTx are to take care and responsibility to safeguard the property of GTx within reason. GTx property includes, but is not limited to: (i) all physical property of GTx whether leased or owned by GTx and includes all fixtures; (ii) all books and records in possession of GTx; (iii) all marketing studies, advertising or promotional materials, logs, reports or any other forms or surveys that are in GTx's possession; and (iv) all proprietary software and technology.

B. USE OF ELECTRONIC SYSTEMS

Electronic mail and e-mail systems (including electronic bulletin boards) are property of GTx and must be used primarily for business purposes. The use of e-mail must conform to the policies and values of GTx. Among other things, messages which violate any of GTx's policies or invite participation in illegal activities, such as gambling or the use and sale of controlled substances, are prohibited. Statements or images which, if made in any other forum, would violate any of GTx's policies, including, without limitation, policies against harassment or discrimination and the misuse of confidential information, are prohibited to the same extent in an e-mail message. E-mail systems may be used to transmit confidential or proprietary information only when such information is adequately protected. Subject to applicable laws and regulations, GTx reserves the right to monitor and review e-mail and voicemail as it deems appropriate.

The Internet is an efficient and valuable business tool and is to be used primarily for business purposes. GTx reserves the right to access all information on Company computers, including but not limited to e-mail and history of internet usage, even where personal passwords have been assigned. If you have questions about the use of your computer, the Internet, e-mail or voice mail, please see the Compliance Officer.

VIII. COMPLIANCE WITH LAWS, RULES AND REGULATIONS

A. GENERAL

Directors and employees must comply fully with applicable laws, rules and regulations at all times. In particular, Directors and employees should take note of laws, rules and regulations regarding the integrity of GTx's records, financial reporting, insider trading, health care laws and regulations and fair employment practices.

B. GTx BUSINESS RECORDS

Accuracy, reliability and timeliness in the preparation of all business records, financial statements, reports to regulatory and other government agencies and other public communications is of critical importance to the corporate decision-making process and to the proper discharge of GTx's financial, legal and reporting obligations. All GTx business transactions shall be carried out in accordance with management's general or specific directives and with the highest standard of care. To this end, GTx shall:

- comply with United States Generally Accepted Accounting Principles at all times;
- maintain a system of internal accounting controls that will provide reasonable assurances to management that all transactions are properly recorded;
- maintain books and records that accurately and fairly reflect GTx's transactions, assets, liabilities, revenues and expenses;
- prohibit the establishment of any undisclosed or unrecorded funds or assets; and
- maintain a system of internal controls that will provide reasonable assurances to management that material information about GTx is made known to management on a timely basis, particularly during the periods in which GTx's periodic reports are being prepared.

False or misleading entries are prohibited. For example, no payment shall be requested, approved or made with the intention or understanding that it will be used for any purpose other than that described in the documentation supporting the payment. Compliance with accounting procedures and internal control procedures is required at all times. It is the responsibility of all employees to ensure that corporate accounting and internal control procedures are strictly adhered to at all times. If you suspect that any records or financial information are not being properly kept or are being falsified, immediately contact Compliance Officer.

Only authorized officials of GTx are allowed to respond to inquiries for Company information from the media, investors, the financial community and others, and employees are to promptly refer all such inquiries to the authorized officials.

In accordance with the rules promulgated by the SEC under the Sarbanes-Oxley Act of 2002, it shall be unlawful and a violation of this Code for any officer or Director of GTx or any other person acting under the direction thereof, to take any action to fraudulently influence, coerce, manipulate, or mislead any independent or certified accountant engaged in the performance of an audit of GTx's financial statements for the purposes of rendering such financial statements materially misleading.

C. SPECIFIC POLICIES GOVERNING CEO AND SENIOR FINANCIAL OFFICERS

The CEO and all senior financial officers are responsible for full, fair, accurate, timely and understandable disclosure in the periodic reports required to be filed by GTx with the SEC. Accordingly, it is the responsibility of the CEO and each senior financial officer promptly to bring to the attention of the Disclosure Controls Committee any material information of which he or she may become aware that affects the disclosures made by GTx in its public filings or otherwise assist the Disclosure Committee in fulfilling its responsibilities as required by rules promulgated by the SEC.

The CEO and each senior financial officer shall promptly bring to the attention of the Disclosure Committee and the Audit Committee any information he

or she may have concerning

(a) significant deficiencies in the design or operation of internal controls, which could adversely affect GTx's ability to record, process, summarize and report financial data or (b) any fraud, whether or not material, that involves management or other employees who have a significant role in GTx's financial reporting, disclosures or internal controls.

D. INSIDER INFORMATION AND SECURITIES TRADING

The information contained below is a summary of GTx's Securities Trading Policy (the "Policy"), and employees are encouraged to consult the Policy for a complete description of the laws regulating stock transactions. Severe civil and criminal penalties can be imposed on individuals and corporations convicted of violations.

1. Employees who know any "material" fact about GTx, which has not been disclosed to the public ("inside information") may not buy or sell GTx's stock until a reasonable amount of time has passed after the information has been disclosed to the public. "Material" information means facts that would be likely to cause the value of the stock to go up or down or that a reasonable shareholder would consider important in deciding whether to buy or sell. Examples include knowledge of new products or discoveries; unpublished clinical trial results; earnings or dividend figures; new contracts with consultants, contractors, suppliers or scientific collaborators; tender offers; acquisitions; mergers; and sales of businesses.

2. In addition, employees can be legally liable if someone outside GTx trades in GTx stock based on a "tip" of inside information given by an employee. Company policy forbids giving confidential information about GTx to outsiders except under limited circumstances approved by the Compliance Officer.

3. Specific additional legal restrictions on GTx stock trading apply to Executive Officers and Directors, who have been furnished with detailed explanations of these restrictions.

E. HEALTH CARE LAWS AND REGULATIONS

The pharmaceutical business, especially the approval and sale of pharmaceutical products, is subject to extensive governmental regulation. Many of these regulations are complex in nature, and employees must be aware of the requirements and take necessary steps to comply with them. The "Anti-Kickback Law" is one of several special health care laws that prohibits offering any inducement to a person intended to influence that person to recommend or purchase a health care product (including prescription medications) that may be reimbursed by Medicare or Medicaid.

F. FAIR EMPLOYMENT PRACTICES

Race, Color, Religion, National Origin, Sex, Age, Covered Veteran Status and Disability. Employees at GTx are recruited, selected and hired on the basis of individual merit and ability with respect to the position filled. As a business comprised of talented and diverse team

members, GTx is committed to the fair and effective utilization of all employees without regard to race, color, religion, national origin, sex, age, covered veteran status, disability or any other category protected by federal, state or local laws. Employees must remember that equal employment opportunity is critical in every aspect of the employment relationship. The relationship covers origin, training, working conditions, benefits, compensation practices, employment functions (including promotion, demotion, discipline, transfer, termination and reduction in force) and Company sponsored educational, social and recreational programs. GTx expects all of its employees to treat each other, regardless of title or position, with the fairness and respect necessary to

maintain a place of employment that encourages each person to contribute to her or his fullest potential.

Harassment. Every person conducting Company business, whether or not employed by GTx, must refrain from engaging in any verbal or physical conduct that could be construed as harassment. Such conduct may consist of making unwelcome sexual advances, or engaging in coercive behavior that is sexual in nature when the rejection of or submission to such conduct affects, either implicitly or explicitly, an employee's status of employment (e.g., pay, promotion, assignment, termination, etc.) or the business relationship of a consultant, contractor, supplier or scientific collaborator. In addition to offending, if not injuring, the victim of such conduct, sexual harassment is counterproductive to sound business policy.

IX. COMPLIANCE WITH AND ENFORCEMENT OF THIS CODE OF BUSINESS CONDUCT AND ETHICS

A. GENERAL

All employees are required to read, understand and refer to this Code. Compliance with the conduct policies set forth in this Code is required of all personnel. Enforcement is the direct responsibility of every supervisor. Supervisors may be sanctioned for failure to instruct their subordinates adequately or for failing to detect non-compliance, where reasonable diligence on the part of the supervisor would have led to the discovery of any problems or violations and given GTx the opportunity to correct them earlier.

Employees should immediately disassociate themselves from taking part in any discussions, activities or other situations that they recognize to be potentially illegal or unethical. No supervisor may direct a subordinate to violate this Code. If an employee becomes aware of any illegal or unethical conduct or behavior in violation of this Code by anyone working for or on behalf of GTx, that employee should report it promptly, fully and objectively to the Compliance Officer or such other point of contact established by the Board of Directors. GTx will attempt to treat such reports confidentially and to protect the identity of the employee who has made the request to the maximum extent and as may be permitted under applicable law. All reports will be investigated. Upon receipt of credible reports of suspected violations or irregularities, the investigative party shall see that corrective action takes place appropriately.

THIS CODE SETS FORTH GENERAL GUIDELINES ONLY AND MAY NOT INCLUDE ALL CIRCUMSTANCES THAT WOULD FALL WITHIN THE INTENT OF THE CODE AND BE CONSIDERED A VIOLATION THAT SHOULD BE REPORTED. EMPLOYEES

SHOULD REPORT ALL SUSPECTED DISHONEST OR ILLEGAL ACTIVITIES WHETHER OR NOT THEY ARE SPECIFICALLY ADDRESSED IN THE CODE.

B. QUESTIONS REGARDING CODE

General questions regarding this Code or the application of this Code to particular situations may be directed to GTx's Compliance Officer. Questions from Directors and Executive Officers may also be discussed with the Chairman of the Board of Directors or the Chairman of the Audit Committee.

C. DETERMINATION OF VIOLATIONS

Determinations regarding whether a violation of this Code has occurred shall be made as follows:

(a) If the alleged violation under consideration concerns an Executive Officer or Director, the determination of the existence of any violation shall be made by the Audit Committee in consultation with the General Counsel and/or such external legal counsel as the Audit Committee deems appropriate.

(b) If the situation under consideration concerns any other employee, the determination of the existence of a violation shall be made by the person to whom the employee ultimately reports, in consultation with the General Counsel.

(c) Whoever makes the decision as to whether a violation has occurred shall document the decision and forward the documentation to the

Compliance Officer, or such other point of contact established by the Board of Directors, for filing and retention. These files shall be available to the Internal Audit and Human Resources Departments.

(d) In determining whether a violation of this Code has occurred, the committee or person making such determination may take into account to what extent the violations were intentional; the qualitative and quantitative materiality of such violation from the perspective of either the detriment to GTx or the benefit to the Director, Executive Officer or employee, the policy behind the provision violated and such other facts and circumstances as they shall deem advisable under all the facts and circumstances.

Acts or omissions that have been determined to be violations of this Code other than by the Audit Committee, under the process set forth above, shall be promptly reported by the Compliance Officer to the Audit Committee and by the Audit Committee to the Board of Directors.

The Board of Directors shall determine, or designate appropriate persons to determine, appropriate actions to be taken if this Code has been violated. Such actions shall be reasonably designed to deter wrongdoing and to promote accountability for adherence to the Code, and shall include written notices to the individual involved that the Board of Directors has determined that there has been a violation, censure by the Board of Directors, demotion or re-assignment of the individual involved, suspension with or without pay or benefits (as determined by the Board of Directors) and termination of the individual's employment.

D. REQUEST FOR WAIVERS

A waiver of a provision of this Code shall be requested whenever there is a reasonable likelihood that a contemplated action will violate the Code. Waivers will only be granted under extraordinary circumstances.

1. Process:

(a) If the request under consideration relates to an Executive Officer or Director, the determination with respect to the waiver shall be made by the Audit Committee, in consultation with the General Counsel and/or such external legal counsel as the Audit Committee deems appropriate and submitted to the Board of Directors for ratification.

(b) If the request under consideration relates to any other employee, the determination shall be made by the person to whom the employee ultimately reports, in consultation with the General Counsel unless such request is quantitatively or qualitatively material or outside the ordinary course of business, in which case such determination shall be made by the Audit Committee.

(c) The decision with respect to the waiver requested shall be documented and forwarded to the Compliance Officer for filing and retention. These files shall be available to the Internal Audit and Human Resources Departments.

2. All waivers of this Code (other than those approved by the Audit Committee) shall be promptly reported by the Compliance Officer to the Audit Committee and by the Audit Committee to the Board of Directors.

3. Waivers shall be publicly disclosed on a timely basis, to the extent determined to be required or appropriate by GTx's Board of Directors in consultation with the General Counsel and/or external legal counsel, as the Audit Committee deems appropriate.

E. GOOD FAITH REPORTING OF WRONGDOING

1. Employees of GTx are protected, to the extent provided by law, against retaliation by GTx when they provide information or assist in an investigation by federal regulators, law enforcement, Congress, or GTx itself, regarding conduct which the employee reasonably believes relates to fraud against GTx's shareholders.

2. An employee or Director shall report such concerns to the General Counsel or the CEO and to the Audit Committee. The General Counsel or

CEO may then arrange a meeting with the employee or Director to allow the employee or Director to present a personal and complete description of the situation. Alternatively, good faith reports of wrongdoing may be reported to such other point of contact as may from time to time be established by the Board of Directors.

(a) "Good faith report" shall mean a report of conduct defined as wrongdoing, which the person making the report has reasonable cause to believe is true and which is made without malice or consideration of personal benefit.

(b) "Wrongdoing" shall mean a violation which is not of a merely technical or minimal nature of a federal or state statute or regulation or of this Code designed to protect the interest of the public or GTx.

(c) All good faith reports and resulting investigations will be kept confidential to the extent required by law.

3. The Sarbanes-Oxley Act of 2002 requires that the GTx Audit Committee establish procedures for confidential, anonymous submission of employee concerns regarding questionable accounting or auditing matters. Employee complaints and reports of this nature shall be handled under the procedures established by the Audit Committee. Information regarding these procedures will be made available on GTx's website.

Any employee who violates a provision of this Code is subject to applicable disciplinary action up to and including termination, and, where appropriate, the filing of a civil or criminal complaint. Directors who violate a provision of this Code are subject to such sanctions as the Board of Directors shall impose. Notwithstanding the foregoing, GTx also preserves and reserves its other rights and remedies against any individual who violates any provision of this Code, both at law and in equity.

X. DISCLAIMER OF EMPLOYMENT CONTRACT

This Code is neither an employment contract nor any guaranty of continued employment. The employment relationship between GTx and its employees is "at will." GTx's policies, guidelines and related procedures are subject to unilateral change by GTx at any time. A fuller discussion of these matters appears in the GTx Employee Manual.

XI. RESERVATION OF RIGHTS

GTx reserves the right to amend this Code, in whole or in part, at any time and solely at its discretion. Any amendments, to the extent determined to be required or appropriate by the Board of Directors in consultation with the General Counsel and/or any other legal counsel as the Audit Committee deems appropriate, shall be publicly disclosed on a timely basis.

XII. CERTIFICATION

Each Director and Executive Officer will be required to read or review this Code each year and certify, in writing, that he or she understands his or her responsibilities to comply with the guidelines and provisions set forth herein.

EXHIBIT 23.1

Consent of Independent Auditors

We consent to the incorporation by reference in the Registration Statement (Form S-8 No. 333-112576) pertaining to the 2004 Equity Incentive Plan, 2004 Non-Employee Directors' Stock Option Plan, 2002 Stock Option Plan, 2001 Stock Option Plan, 2000 Stock Option Plan and 1999 Stock Option Plan of GTx, Inc. of our report dated January 30, 2004, except for Note 13 as to which the date is March 16, 2004, with respect to the financial statements of GTx, Inc. included in the Annual Report (Form 10-K) for the year ended December 31, 2003.

/s/ Ernst & Young LLP

Memphis, Tennessee
March 23, 2004

EXHIBIT 31.1

Chief Executive Officer Certification

I, Mitchell S. Steiner, certify that:

1. I have reviewed this annual report on Form 10-K of GTx, Inc.;

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

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

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

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

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

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

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

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

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

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

Date: March 26, 2004

/s/ Mitchell S. Steiner

Mitchell S. Steiner
Chief Executive Officer

EXHIBIT 31.2

Chief Financial Officer Certification

I, Mark E. Mosteller, certify that:

1. I have reviewed this annual report on Form 10-K of GTx, Inc.;

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

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

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

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

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

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

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

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

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

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

Date: March 26, 2004

/s/ Mark E. Mosteller

Mark E. Mosteller
Chief Financial Officer

EXHIBIT 32.1

CERTIFICATION PURSUANT TO
18 U. S. C. SECTION 1350,
AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Annual Report of GTx, Inc. (the "Company") on Form 10-K for the period ending December 31, 2003, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I, Mitchell S. Steiner, Chief Executive Officer of the Company certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that:

1. The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

2. The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

/s/ Mitchell S. Steiner

Mitchell S. Steiner
Chief Executive Officer

March 26, 2004

EXHIBIT 32.2

CERTIFICATION PURSUANT TO
18 U. S. C. SECTION 1350,
AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Annual Report of GTx, Inc. (the "Company") on Form 10-K for the period ending December 31, 2003, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I, Mark E. Mosteller, Chief Financial Officer of the Company certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that:

1. The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

2. The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

/s/ Mark E. Mosteller

Mark E. Mosteller
Chief Financial Officer

March 26, 2004