Table of Contents

UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-K/A

Amendment No. 3

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

For the fiscal year ended December 31, 2004

OR

o 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 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 o

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. o

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

The aggregate market value of common stock held by non-affiliates of the Registrant based on the closing sales price of the Registrant's common stock on June 30, 2005, as reported on the National Association of Securities Dealers Automated Market was $75,667,037.

There were 24,664,716 shares of Registrant's common stock issued and outstanding as of July 25, 2005.

Documents Incorporated by Reference

None

Table of Contents

EXPLANATORY NOTE

GTx, Inc. (the "Company") is filing this Amendment No. 3 to its Annual Report on Form 10-K for the fiscal year ended December 31, 2004, as filed with the Securities and Exchange Commission on March 24, 2005, and as amended by Amendment No. 1 filed on March 31, 2005 and Amendment No. 2 filed on July 28, 2005, to (1) restate the full text of the Annual Report as originally filed, (2) amend and restate the Exhibit Index in Item 15, and (3) correct an inadvertent, single word error in and refile Exhibits 31.1 and 31.2.

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 and oncology. Our lead 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 have four clinical programs. We are developing ACAPODENE® (toremifene citrate) for two clinical programs in men: (1) a pivotal Phase III clinical trial for the prevention of prostate cancer in high risk men and (2) a pivotal Phase III clinical trial for the treatment of serious side effects of androgen deprivation therapy (ADT) for advanced prostate cancer. In our third clinical program, we and our partner, Ortho Biotech Products, L.P. (Ortho Biotech), a subsidiary of Johnson & Johnson, are developing andarine, a selective androgen receptor modulator (SARM). We are working with Ortho Biotech to progress andarine to Phase II clinical testing in the second half of this year. In our fourth clinical program, we are developing our second SARM, ostarine, for andropause and other chronic conditions related to aging, including sarcopenia. We also have a marketed product, FARESTON® (toremifene citrate 60mg) tablets for the treatment of metastatic breast cancer. The active pharmaceutical ingredient in FARESTON is the same as in ACAPODENE, but a different dosage form.

In addition, we have an extensive preclinical pipeline generated from our own discovery program, which includes the specific product candidates prostarine, a SARM for benign prostatic hyperplasia (BPH), and andromustine, an anticancer drug, for hormone refractory prostate cancer. We believe our four promising clinical programs along with our discovery pipeline create for us attractive long term commercial opportunities.

Our most advanced product candidate, ACAPODENE, is being developed to prevent prostate cancer in high risk men with precancerous prostate lesions known as high grade prostatic intraepithelial neoplasia, or high grade PIN. 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. In 2004, we completed a Phase IIb clinical trial in which we enrolled 514 patients to determine the efficacy and safety of ACAPODENE in the prevention of prostate cancer in men with high grade PIN. This was the largest prospective study of the natural history and treatment of patients with high grade PIN. This well controlled study confirmed that men who have high grade PIN are at high risk, as 31% of placebo patients were diagnosed with prostate cancer by year one. The intent-to-treat analysis, defined as any patient who had at least one on-study biopsy, showed that ACAPODENE 20 mg had a 20% reduction in prostate cancer incidence. The reduction of prostate cancer incidence improved in men who received ACAPODENE 20mg for one year, with the clinical trial showing a 46% reduction in this high risk population compared to the placebo group, which is consistent with the interim analysis we conducted in 2003. For men who were diagnosed with prostate cancer, those treated with ACAPODENE had similar tumor grades to those of placebo patients, providing evidence that ACAPODENE does not affect the severity of the tumor in those patients who develop prostate cancer. ACAPODENE was well tolerated, as the number of adverse events was similar between those patients receiving ACAPODENE compared to placebo. We initiated a pivotal Phase III clinical trial in January 2005 under a Special Protocol Assessment (SPA) which we have filed with the Food and Drug Administration (FDA).

We are also developing ACAPODENE for the treatment of serious 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 required growth factor for prostate cancer. Androgen deprivation therapy, however, can have serious side effects, including: severe bone loss, or osteoporosis, leading to skeletal fractures; hot flashes; and breast pain and enlargement, or gynecomastia. There are currently 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 under an approved SPA for this indication in November 2003.

Our third clinical program, andarine, is a product candidate being developed under a collaboration agreement with Ortho Biotech, initially for the treatment of cachexia from various types of cancer, a potentially life-threatening complication of many cancers. There are currently no drugs that have been approved by the FDA for the treatment of cancer cachexia. We are planning a placebo-controlled, dose-finding Phase II clinical trial for the treatment of cancer cachexia for the second half of this year.

In our fourth clinical program, another SARM from our own discovery pipeline, ostarine, is being developed for andropause and other chronic conditions of aging. Andropause is associated with loss of muscle mass, hypogonadism, osteoporosis, high cholesterol and obesity. Ostarine has been shown to be highly selective in building muscle with favorable pharmacokinetic properties, including a long half life, that support continued clinical development for this indication.

We market FARESTON for the treatment of metastatic breast cancer. FARESTON has been commercially available for over 15 years. In January 2005, we acquired from Orion Corporation the rights to distribute FARESTON in the U.S. and a license to toremifene, the active pharmaceutical ingredient in FARESTON and ACAPODENE, for all indications worldwide except breast cancer outside of the U.S.

We have multiple product candidates that are in preclinical studies required prior to initiating clinical trials. Our current preclinical product candidates primarily focus on the treatment of other major indications in men's health, including BPH, a benign prostate enlargement that results in obstruction of the urinary tract, and hormone refractory prostate cancer.

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 the relative estrogen levels in the blood, which may promote BPH, initiate prostate cancer, and cause gynecomastia.

Testosterone, the predominant androgen in men, 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. Male reproductive health is also dependent on testosterone to maintain sexual interest, fertility, erectile function and normal prostate growth. Testosterone also stimulates sebaceous and hair glands, which can cause unwanted effects like acne and hair loss. In aging men, there is a gradual decline in testosterone levels, which contributes to a loss of muscle mass and strength, decreased 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 bind to and activate their hormone receptors located in various tissues. Once a hormone binds with its receptor, this activates a series of cellular events resulting in estrogenic or androgenic tissue effects, depending on the receptor.

Pharmaceuticals that target hormone receptors for estrogens or androgens have been medically used for over 50 years. The drugs that have been used to stimulate androgen receptors are either natural or synthetic hormones, known as steroids. Steroids activate hormone receptors in all tissue types in a non-selective manner resulting in not only beneficial effects but also in unwanted clinical effects. In men, 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 which are not only inconvenient for patients, but also in some cases, result in inconsistent blood levels of testosterone.

There are also classes of small molecules that bind to hormone receptors which are not steroids. These nonsteroidal small molecules may either stimulate or block hormone receptors depending on the type of tissue in which the receptor is found and the interaction of the molecule with the receptor. A drug that has the ability to either

block or stimulate the 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 nonsteroidal 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 mimic 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 and the breast. Examples of SERMs currently on the market include toremifene, which has been prescribed to treat advanced female 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 and muscle 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 in the brain to affect mood and sexual interest. Although no SARMs have been commercialized to date, we believe that SARMs, like testosterone, could be developed to treat a range of medical conditions without the side effects of testosterone including: (1) low testosterone conditions, such as hypogonadism and andropause (sarcopenia); (2) muscle wasting conditions of chronic diseases, such as cancer, AIDS, end stage renal disease neurodegenerative disorders, 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, male hair loss and male osteoporosis.

Marketed Product

FARESTON®

Toremifene is a selective estrogen receptor modulator compound owned and manufactured by Orion Corporation (Orion), a Finnish corporation. In December 2004, we announced an agreement with Orion to acquire the exclusive license to toremifene in the U.S. and additional rights in all other countries giving us exclusive global rights to all toremifene-based products for all indications in humans, except breast cancer outside of the U.S. Toremifene is the active pharmaceutical ingredient in ACAPODENE, our lead product currently in Phase III clinical trials for two indications, and FARESTON, which has been approved by the FDA for the treatment of metastatic breast cancer. In 2000, we in-licensed toremifene from Orion to develop ACAPODENE for certain indications in men's health. At the time the agreement was executed, Shire Pharmaceuticals had already licensed from Orion the distribution rights in the U.S. to sell toremifene as FARESTON for the treatment of metastatic breast cancer. Under the terms of our purchase agreement with Orion, we paid to Orion a license fee of approximately $4.8 million and purchased FARESTON inventory of approximately $448,000. We will continue to market FARESTON in the U.S. for the treatment of metastatic breast cancer and will pay a royalty to Orion on FARESTON sales. The royalty rate for FARESTON will be reduced after we commercialize a new toremifene based product such as ACAPODENE for men's health indications. Additionally, as part of our acquisition agreement with Orion, our license and supply agreement with Orion was amended to provide that Orion will manufacture and supply all of our needs for clinical trial and commercial grade material for toremifene-based products developed and marketed globally by us, including ACAPODENE and FARESTON.

Product Candidates

The following table summarizes key information about our product candidates:

Program	Product Candidate/Indication	Development Phase	Status
SERM	**ACAPODENE**		
	- Prevention of prostate cancer in men with high grade PIN	Pivotal Phase III clinical trial	Phase III trial initiated first quarter 2005
	- Side effects of androgen deprivation therapy	Pivotal Phase III clinical trial	Phase III trial initiated fourth quarter 2003
SARM	**Andarine**		
	- Cachexia from various types of cancer	Four Phase I clinical trials completed	Planning Phase II trial second half of 2005
	Ostarine		
	- Andropause and sarcopenia	Phase I	Phase I single ascending dose (SAD) trial completed first quarter 2005 Planning Phase I multiple ascending dose (MAD) trial second quarter 2005
	Prostarine		
	- BPH	Preclinical	Preclinical studies
Anticancer	**Andromustine**		
	- Prostate cancer that is not responsive to androgen deprivation therapy	Preclinical	Preclinical studies

ACAPODENE®

Our most advanced product candidate, ACAPODENE, is a selective estrogen receptor modulator, or SERM. ACAPODENE is being developed for a once-a-day oral dosing schedule. In January 2005, we acquired all rights to toremifene for all indications, except breast cancer outside of the U.S., including the rights to develop, market and distribute toremifene 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. 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 because of the established safety and efficacy record of toremifene in the treatment of post menopausal women and men with advanced breast cancer. Orion manufactures commercial quantities of toremifene citrate 60mg as FARESTON for us and is supplying us with ACAPODENE (toremifene citrate 20mg and 40mg) for our clinical trials under a supply agreement.

The indications for which we are developing ACAPODENE target two different patient populations: (1) patients who have been diagnosed with high grade PIN, which has a high likelihood of progressing to 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 (toremifene citrate 20mg) For The Prevention Of Prostate Cancer In Men With High Grade PIN

Scientific Overview. Patients who have an abnormal test 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 demonstrated 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 men at high risk for prostate cancer may prevent the disease.

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 approximately 400,000 new cases of prostate cancer diagnosed each year 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 through the probe into the prostate to obtain cores of tissue. Complications from this procedure include bleeding, pain, prostate infection and life-threatening blood infection (sepsis). 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.

During 2004, we entered into three separate collaboration agreements with diagnostic companies, Hybritech, Inc., a wholly owned subsidiary of Beckman Coulter, Inc., diaDexus, Inc., and Tessera, Inc., to provide clinical samples to each party from our now completed Phase IIb clinical trial of ACAPODENE. Information resulting from these collaborations will be used to evaluate whether a commercial test from blood or urine may be effectively developed to detect high grade PIN and/or prostate cancer. We believe that there now exists the opportunity to develop a test for high grade PIN and/or prostate cancer. By continuing to collaborate with leading diagnostic labs, we hope to have a urine or blood test developed to detect high grade PIN in the millions of American men who may unknowingly harbor this precancerous prostate lesion.

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 in Memphis, 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 diagnosed high grade PIN to determine the efficacy and safety of a daily dose of ACAPODENE compared to placebo treatments for one year. The trial was conducted at 64 clinical sites across the United States. On June 4, 2004, we announced positive results from our Phase IIb clinical trial for ACAPODENE. The ACAPODENE Phase IIb study was a four arm, double-blind, placebo-controlled, clinical trial in 514 men with high grade PIN who are at high risk for prostate cancer. The four arms included in this study were 20mg, 40mg and 60mg of ACAPODENE and placebo given orally once a day. There were approximately 125 patients per arm. The primary entry criterion for the study was men with biopsy proven and confirmed high grade PIN. All patients were rebiopsied at 6 and 12 months from randomization. The primary endpoint was the incidence of prostate cancer. This was the largest prospective study to determine the natural history of patients with high grade PIN and supports the previous retrospective clinical observations that high grade PIN patients have a high risk for developing prostate cancer. The study also suggests that ACAPODENE may be an effective agent in preventing prostate cancer. This Phase IIb clinical trial demonstrated that ACAPODENE 20mg can produce a clinically significant reduction of prostate cancer cumulative risk by one year with the incidence of prostate cancer being approximately 24% with ACAPODENE 20mg compared to approximately 31% with placebo. Furthermore, the data appears to suggest that the longer men with high grade PIN are treated with ACAPODENE, the greater the likelihood that their risk of prostate cancer is reduced. Patients who had a negative prostate biopsy for cancer after 6 months of treatment had approximately a 46% reduction in prostate cancer after a full 12 months of treatment with ACAPODENE. ACAPODENE was well tolerated by patients compared to placebo in this trial. Based on the positive data from our Phase IIb clinical trial, we submitted our plan for a pivotal Phase III clinical trial for ACAPODENE to the FDA in October 2004 under a Special Protocol Assessment (SPA). An SPA, which is provided for in the Food and Drug Administration Modernization Act, allows a sponsor to obtain a written agreement from the FDA on the evaluation of issues related to the adequacy of the design of proposed clinical protocols. We initiated our Phase III clinical trial in January 2005 and have refiled our revised SPA in response to the FDA's input to our initial filing.

ACAPODENE (toremifene citrate) 80mg For The Treatment Of Serious 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 required 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, Zoladex and Eligard.

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 estrogen 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 preclinical and clinical 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. Also, 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 at least 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, and second, the serum PSA test is detecting advanced prostate cancer earlier than in the past. The net 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 other 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.

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 or gynecomastia. 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 orally administered ACAPODENE in patients undergoing androgen deprivation therapy for advanced, recurrent or metastatic prostate cancer under a SPA from the FDA. 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 bone mineral density, hot flashes and gynecomastia. We expect that over 100 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 6 months and who have significant existing bone loss, or are greater than 70 years of age, will be randomized to receive either a placebo or a daily dose of ACAPODENE for 24 months. The primary endpoint is the reduction in vertebral skeletal fractures. We are planning an interim analysis of the measurement of bone mineral density, a secondary endpoint, in the first 200 patients in this clinical trial in the second half of this year.

Andarine

In our third clinical program, we are developing andarine, a SARM, and it is our most advanced product candidate of our internally discovered portfolio of compounds designed to target the androgen receptor. Andarine is taken orally and is being developed for a once-a-day dosing schedule. Our strategy is to continue 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.

In March 2004, we entered into a joint collaboration and license agreement with Ortho Biotech for andarine and specified backup SARM compounds. Under the terms of the agreement, in April 2004, we received an up front licensing fee and reimbursement of development expenses of the completed Phase Id clinical trial for andarine totaling approximately $6.7 million. The up front licensing fee and reimbursement of development expenses are expected to be amortized into revenue on a straight-line basis through March 2009. Additionally, we will receive licensing fees and milestone payments of up to $82.0 million based on andarine and up to $45.0 million for each additional licensed compound achieving specific clinical development decisions or obtaining regulatory approvals. All milestone payments for andarine are based on achievements prior to its commercial launch. Johnson & Johnson Pharmaceutical Research & Development will be responsible for further clinical development and related expenses for andarine and other licensed SARM compounds. Ortho Biotech will be responsible for commercialization and related expenses for andarine and other licensed SARM compounds. If andarine is approved for commercial sale, Ortho Biotech will exclusively market andarine in the United States and in markets outside the United States. Under the agreement, we have the option to copromote andarine and the other licensed SARM compounds to urologists in the United States for indications specifically related to men's health. We will receive up to double digit base royalties on all United States and worldwide sales. For all copromoted sales generated from urologists in the United States, we will receive the base royalty plus an additional royalty in excess of 20%. Depending on the results of our initial development efforts, together with Ortho Biotech, we may also develop andarine for the treatment of 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 cancer cachexia results from the loss of lean body, or muscle weight. 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.

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 can have a number of undesirable side effects in men, such as the potential stimulation of latent prostate cancer, aggravation of existing BPH and gynecomastia, and in women, it can have masculinizing effects 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 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 more 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, which 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 established as a part of our joint collaboration with Ortho Biotech.

Ostarine

Our fourth clinical program is to develop ostarine, another SARM product candidate from our own discovery portfolio of compounds. Ostarine is being targeted for the treatment of andropause which is associated with sarcopenia, or loss of muscle mass related to aging as well as hypogonadism, osteoporosis, high cholesterol and obesity. Based on the positive preclinical data that we observed with ostarine, we are developing this product candidate for use in subjects with andropause for which there are currently no treatments available. Phase I clinical development commenced in January 2005 and will evaluate the safety, tolerability and pharmacokinetic profile of ostarine with single ascending dose and multiple ascending dose, double-blind, placebo-controlled designed studies in healthy volunteers which will include elderly subjects.

Ostarine For The Treatment Of Andropause

Scientific Overview. Andropause is associated with the loss of muscle mass associated with aging, also known as sarcopenia, osteoporosis, high cholesterol, hypogonadism and obesity. As people age they undergo hormonal and metabolic changes. Each year after age 30 people gain an average of a pound of fat every year and lose a half a pound of muscle every year. An average man may lose 40% of muscle between the ages of 30 and 90 years of age. Muscle provides strength and endurance, supports the skeletal system and helps protect the body through the immune system. Loss of muscle can cause frailty, loss of independence and worsens other conditions of aging such as osteoarthritis and osteoporosis.

Potential Market. There are approximately 17 million people over the age of 65 who have age related sarcopenia each year in the United States. There are no drugs that have been approved by the FDA for the treatment of andropause.

Clinical Trials. Our Phase I single ascending dose clinical trial to evaluate the safety, tolerability and pharmacokinetic profile of ostarine using a single ascending dose, double-blind, placebo-controlled was initiated in January 2005 in 96 healthy volunteers and elderly subjects. The Phase I clinical trial to evaluate the safety, tolerability, and pharmacokinetic profile using a multiple ascending dose, double blind, placebo-controlled design is planned for the second quarter of this year.

Prostarine

We are also developing another SARM product candidate, prostarine, for the treatment of benign prostatic hyperplasia, or BPH, which is benign prostate enlargement that results in obstruction of the urinary tract. In animal models, prostarine has the ability to shrink and prevent growth of the prostate gland. We are conducting preclinical studies required to support clinical trials.

Andromustine

First line therapy of patients who have advanced, recurrent or metastatic prostate cancer is androgen deprivation therapy. Since prostate cancer is dependent on androgens, such as testosterone, to grow, the reduction in testosterone leads 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 prostate cancer.

Building on the technology of our SARM discovery program, we designed and are developing small molecules like andromustine to specifically target androgen receptors and kill cancer cells. In one approach, the andromustine molecule has two components: (1) the SARM-like part of the molecule, which binds 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, these compounds selectively kill metastatic human prostate cancer cells. We continue to use this and other approaches to identify molecules with potent in vitro and in vivo anticancer activity. Because advanced prostate cancers, including hormone refractory prostate cancer, have more androgen receptors than the normal prostate, andromustine has been designed to bind to and to 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. Once a patient develops hormone refractory prostate cancer, his prognosis is poor. Andromustine could be second line cancer therapy for patients who develop hormone refractory prostate cancer.

Drug Discovery and Other Research & Development

Steroid hormone therapies, which include estrogen and testosterone therapies, have been used to treat humans for many years. Steroid hormones by their nature have unselective effects in various tissues. As a result, they have unintended side effects, which limit their clinical value.

SERM drugs, such as toremifene, tamoxifen and raloxifene, have achieved commercial success in treating women as nonsteroidal small molecules that modulate hormone estrogen receptors in a tissue selective way and minimize some of the side effects of the natural estrogen hormone to treat breast cancer (toremifene and tamoxifen) or to treat postmenopausal osteoporosis (raloxifene). We believe that the previous commercial and scientific 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 will allow us to sustain our clinical pipeline through the design and development of nonsteroidal small molecule drugs that modulate hormone receptors. Our 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 design and synthesize new compounds based on computer, or *in silico*, models and crystal structures of a hormone receptor's binding sites. We continually modify and improve these 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 and high throughput 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 clinical product candidates for the androgen receptor, andarine and ostarine. We also have conducted other research and development efforts focused on SERM and SARM compounds, other receptor modulator compounds and viral cytolytics.

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:

Obtain Regulatory Approval Of ACAPODENE. We are focused on completing clinical trials, obtaining regulatory approval and preparing for the potential commercial launch of ACAPODENE for two distinct indications in men's health.

Retain Commercial Rights To ACAPODENE And Establish Sales And Marketing Infrastructure. We intend to retain 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 to physicians outside of urology and medical oncology in the United States and all physicians in countries in Europe and Asia and in other countries outside of the United States.

Extend Life Cycle Of ACAPODENE. We are studying various means 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. We also intend to apply for market exclusivity and regulatory extensions of patent life under applicable European and U.S. laws, as appropriate, to protect our exclusive rights in ACAPODENE for the indications we are currently testing in clinical trials.

Develop Diagnostic Tests For High Grade PIN. We are currently collaborating with three large diagnostics companies; Hybritech, Inc., a wholly owned subsidiary of Beckman Coulter, Inc., diaDexus, Inc. and Tessera, Inc. to develop an accurate blood or urine test to detect high grade PIN. We will continue to seek additional collaborations for other companies with promising high grade PIN diagnostics. 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 blood or urine test will increase the detection of high grade PIN and thereby expand the already large potential market for ACAPODENE.

Maintain Commercial Sales Of FARESTON. We intend to devote sufficient marketing and sales efforts to maintain FARESTON at current sales levels.

Pursue Clinical Development Of Andarine. Under our joint collaboration and license agreement with Ortho Biotech for the continued clinical development of andarine and specified backup SARM compounds, we intend to continue to pursue the clinical development of andarine for the treatment of cachexia from various types of cancer. In addition, we 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 and women'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., a subsidiary of Johnson & Johnson".

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 to further the development and commercialization of our small molecule products.

Ortho Biotech Products L.P., A Subsidiary of Johnson & Johnson

Under a joint collaboration and license agreement with Ortho Biotech executed in March 2004 for andarine, our most advanced SARM compound, and specified backup SARM compounds, we received an upfront licensing fee of $6 million and reimbursement of certain development expenses of approximately $687,000. We also can receive additional licensing fees and milestone payments up to $82.0 million based on andarine and up to $45.0 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 up to double-digit 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

On December 29, 2004, we entered into an Amended and Restated License and Supply Agreement (License and Supply Agreement) with Orion Corporation granting us exclusive rights to Orion's compound, toremifene, for all products for human uses, including our product candidate, ACAPODENE, excluding, however products for breast cancer sold outside of the United States. The License and Supply Agreement, which has an effective date of January 1, 2005, replaces an earlier agreement entered into with Orion in 2000, and subsequently amended in 2001 and 2003 (Original License). Under the agreement, we paid a license fee of $4.8 million. The term of the License and Supply Agreement will survive the term of our patents, including the patents we license from University of Tennessee Research Foundation (UTRF) pertaining to ACAPODENE for the treatment and/or prevention of PIN and prostate cancer. We believe that our patents pertaining to methods of use for toremifene will survive until at least 2020.

Under the Original License, we paid Orion $400,000 which we can offset, along with clinical trial expenses, against licensing fees and milestone payments we will pay to Orion if we receive licensing fees and milestone payments on account of our sublicensing rights to third parties. The License and Supply Agreement retains these provisions and, additionally, obligates us to make future royalty payments to Orion of varying amounts for sales of FARESTON for breast cancer in the U.S. and other toremifene based products licensed to us under the agreement, including ACAPODENE to treat or prevent PIN or prostate cancer or to treat complications arising from androgen deprivation therapy.

We have agreed to achieve specified minimum sales requirements of ACAPODENE in the U.S. after commercialization of the product. If we do not do so, we must pay Orion royalties based on the amount of the shortfall below the applicable minimum sales requirement. In addition, we are required to pay up to $1.0 million if we are acquired before receiving marketing approval for the use of ACAPODENE for the prevention or treatment of high grade PIN or prostate cancer or to treat complications arising from androgen deprivation therapy. Orion may terminate its supply agreement for ACAPODENE toremifene based products if marketing approval for ACAPODENE is not granted in the U.S. by December 31, 2009.

University of Tennessee Research Foundation

In August 2002, we executed an Amended and Restated Exclusive License Agreement with UTRF granting us a worldwide exclusive license under their method of use patents relating to ACAPODENE for the prevention of prostate cancer in high risk men with PIN. Under the terms of the agreement, we are required to make annual

maintenance fee payments and future royalty payments to UTRF. We are also required to pay all expenses to file, prosecute and maintain the patents relating to ACAPODENE for the prevention of prostate cancer in high risk men with high grade PIN.

The amended license agreement superseded a 1998 license agreement with UTRF pursuant to which we reimbursed UTRF for certain patent expenses incurred by UTRF and agreed to make sublicense fee payments and future royalty payments.

In June 2002, we executed two Amended and Restated Exclusive License Agreements with UTRF granting us worldwide exclusive licenses under its composition of matter and method of use patents relating to selective androgen receptor modulator (SARM) compounds, including andarine, to market, distribute and sell licensed products. Under the terms of these license agreements, we are required to make annual maintenance fee payments and future royalty payments to UTRF. We are also required to pay all expenses to file, prosecute and maintain the patents relating to SARMs.

The amended license agreement superseded a 2000 license agreement with UTRF pursuant to which we reimbursed UTRF for certain patent expenses incurred by UTRF and agreed to make sublicense fee payments and future royalty payments.

In December 2004, UTRF and the Ohio State University (OSU), entered into an Inter-institutional Agreement to share, in some cases, ownership of SARM technology, subject to our exclusive license rights, and royalty payments received from our SARM License with UTRF. We have agreed to amend our SARM License to require us to provide the same kind of reports and notifications to OSU that we currently provide to UTRF.

We have also executed with UTRF an Amended and Restated Exclusive License Agreement granting us worldwide exclusive licenses with UTRF's composition of matter and method of use patents for some of the preclinical programs pertaining to viral cytolytics and gene therapy.

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, patients who have been diagnosed with prostate cancer have been given a single oral daily dose of ACAPODENE for up to 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 any of our SARMs, including andarine or ostarine. 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 toremifene, marketed as FARESTON and branded as ACAPODENE, from Orion under an exclusive license and supply agreement providing for Orion to supply our requirements for clinical and commercial product. Orion has agreed to supply us with, and we have agreed to purchase from Orion, our worldwide requirements of toremifene 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 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 any of the licensed fields, except breast cancer, is not granted in the United States by December 31, 2009; 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 for toremifene tablets from Orion during the term of the agreement, which extends for the life of our patent rights, 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 to us 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 our bankruptcy or a material breach of the agreement by us that is not cured, we would not have the right to manufacture toremifene for ACAPODENE until Orion's patents with respect to the composition of matter of toremifene expire.

We have entered into an agreement with EaglePicher Pharmaceutical Services, a division of EaglePicher Technologies, LLC, under which EaglePicher has agreed to manufacture andarine for us in a quantity that we believe is sufficient to supply clinical trials of andarine and initial commercialization. We also contract with EaglePicher Pharmaceutical Services and Metrics, Inc. for our clinical supply needs for ostarine. Metrics uses the material provided by EaglePicher to provide us with tablets for our ostarine clinical trials. We do not have a contract with EaglePicher or Metrics for the supply of andarine or ostarine for full-scale commercialization. The active ingredient, andarine or ostarine, 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 is being 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 opportunities will be reduced or eliminated if our competitors develop and commercialize similar 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 Prevention Of Prostate Cancer In Men With High Grade PIN

Currently, there are no drug 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 Serious 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® (aledronate sodium), a bisphosphonate marketed by Merck, and Actonel (risendronate sodium), a bisphosphonate marketed by Aventis and Procter & Gamble, are each prescribed off-label for the treatment of osteoporosis. Amgen has an investigational drug, AMG-162, in Phase III trials for the prevention of fractures in men undergoing ADT. 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 can be 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 currently 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, but has been prescribed 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.

Ostarine For The Treatment Of Andropause

There are currently no drugs that have been approved by the FDA for the treatment of andropause. Testosterone products have been used off-label to treat andropause. Owing to its potentially unwanted effects in the prostate, we believe testosterone products have not had much of an impact on the market for andropause.

In addition, as to ACAPODENE, andarine, and ostarine, 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.

FARESTON For The Treatment Of Breast Cancer

There are a number of drugs that have been approved by the FDA for the treatment of breast cancer. Tamoxifen, which is marketed by AstraZeneca and several generic manufacturers, has been approved by the FDA for the treatment of advanced breast cancer and the reduction of breast cancer in women at high risk for developing the disease. The aromatase inhibitors, such as anastrozole, letrozole and exemestane are used to treat breast cancer in post menopausal women. The aromatase inhibitors (AIs) are growing at the expense of SERMs due to clinical trials such as Arimidex and Tamoxifen: Alone or in Combination (ATAC) which have shown efficacy and tolerability advantages for AIs compared to tamoxifen.

Sales and Marketing

In order to commercialize any future products, we must broaden our sales and marketing infrastructure or collaborate with third parties with sales and marketing experience and personnel. 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 and FARESTON prescribers, principally medical 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 countries in Europe and Asia and for other countries outside of the United States for prostate cancer-related conditions. GTx currently markets FARESTON to approximately 1,000 medical oncologists in the United States.

In part, because marketing andarine to address the cancer cachexia market would require a large sales force and due to 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., a subsidiary of Johnson & Johnson”.

Since ostarine for the treatment of andropause may be prescribed in the United States and abroad by general practitioners, as well as specialists like urologists, we anticipate that we will seek appropriate collaboration partners at an appropriate time to market, distribute and sell ostarine in the United States and abroad, although we expect to retain rights to sell to urologists in the United States through our specialty sales force.

We intend to devote sufficient marketing and sales efforts to maintain FARESTON at current sales levels.

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. Patents and other proprietary rights are an essential element of our business.

For ACAPODENE in the United States and internationally, we have entered into an Amended Restated License and Supply Agreement with Orion Corporation granting us an exclusive license under Orion’s patents covering the composition of matter of toremifene, the active pharmaceutical ingredient in ACAPODENE, for all uses in humans in the United States, and for all human uses outside the United States other than to treat breast cancer. The patent for toremifene 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 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 the method of use patents that either have been already issued or other patents that may later be issued in respect of our owned and licensed patent applications relating to the use of ACAPODENE for the relevant indications we seek.

We have licensed from the University of Tennessee Research Foundation method of use patents in the United States and issued 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 begin expiring 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 for toremifene, the active pharmaceutical ingredient of ACAPODENE, 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 some patents issued and many more 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 issued 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.

Until January 2005, our license from Orion was 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. We have since acquired the rights from Orion to market, sell and distribute a 60mg toremifene tablet under the trademark FARESTON for the treatment of advanced breast cancer in the US and the rights to market, sell and distribute toremifene for all other indications in humans in the United States and in the rest of world except for breast cancer outside of the U.S.

For andarine and other specified SARMs licensed to Ortho Biotech in the United States and abroad, we have an exclusive license from the University of Tennessee Research Foundation (UTRF) under its patents and patent applications related to the composition of matter and formulations of, and methods of using, the active pharmaceutical ingredient in these compounds. 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 UTRF to its pending patent applications in the United States and abroad related to methods of synthesizing the active pharmaceutical ingredient in andarine and methods for treating cancer cachexia with andarine. We also have our own pending patent applications in the United States and internationally related to methods of using andarine.

For ostarine, we have a license from the UTRF 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 prostarine, we have an exclusive license from UTRF under its patent and 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 andromustine, we have an exclusive license from UTRF under its pending patent applications, as well as pending patent application of our own, in the United States and abroad 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 confidentiality agreements and our employees to execute assignment of invention agreements on commencement of their employment. Agreements with our employees also prevent them from bringing any 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 applicable regulations. Non-compliance with FDA regulations 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 efficacy. 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 an applicant's new drug 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 patients to determine if there are any side effects or other risks associated with the drug and to determine the optimal dose of the drug from the safety and efficacy profile developed from the clinical study. 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 10 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 for the drug. 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 record keeping 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 product’s 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 applicant submitting 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 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 applicant of 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 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. Currently, our only marketed product, FARESTON for the treatment of metastatic breast cancer, is eligible for coverage and reimbursement by third-party payors.

Employees

As of December 31, 2004, we had 65 employees, 19 of whom were M.D.s and/or Ph.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 http://www.sec.gov that contains the reports, proxy and information statements, and other information filed electronically. Our website address is http://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.

We will provide copies of the exhibits to this Annual Report on Form 10-K should they be requested by eligible stockholders, and we may impose a reasonable fee for providing such exhibits. Requests for copies of the exhibits to our Annual Report on Form 10-K should be mailed to: GTx, Inc., 3 North Dunlap Street, Memphis, Tennessee 38163, Attention: Corporate Secretary.

Executive Officers and Other Key Employees of the Registrant

The following table sets forth information about our directors, executive officers and other key employees as of March 24, 2005.

Name	Age	Position(s)
Directors and Executive Officers		
Mitchell S. Steiner, M.D., F.A.C.S.	44	Chief Executive Officer and Vice-Chairman of the Board of Directors
Marc S. Hanover	42	President, Chief Operating Officer and Director
Henry P. Doggrell	56	Vice President, General Counsel and Secretary
Mark E. Mosteller, CPA	42	Vice President, Chief Financial Officer and Treasurer
K. Gary Barnette, Ph.D.	37	Vice President of Clinical Research and Development
James T. Dalton, Ph.D.	42	Vice President of Preclinical Research and Development
Gregory A. Deener	43	Vice President of Marketing and Sales
Other Key Employees		
T. Gary Bird, Ph.D.	53	Director of Corporate Quality
Robert S. Boger, M.D.	58	Director of Drug Safety
Karen A. Veverka, Ph.D.	37	Director of Preclinical Development
Michael A. Whitt, Ph.D.	46	Director of Molecular Biology

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 and was appointed Vice President on January 20, 2005. 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, CPA, has served as our Chief Financial Officer since August 2001 and was appointed Vice President and Treasurer on January 20, 2005. 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.

K. Gary Barnette, Ph.D. was appointed Vice President of Clinical Research and Development on January 20, 2005 and prior to that he 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.

James T. Dalton, Ph.D. has served as Vice President of Preclinical Research and Development since January 2005. Dr. Dalton served as a scientific consultant to GTx from 1999 to 2005. Prior to joining GTx, Dr. Dalton held several academic appointments including Assistant and Associate Professor of Pharmaceutical Sciences in the College of Pharmacy at the University of Tennessee, Memphis (1992-2000) and Professor and Chair of the Division of Pharmaceutics, College of Pharmacy at The Ohio State University. SARMs were first discovered in Dr. Dalton's research laboratories, and he is co-inventor on all SARM patents. Dr. Dalton holds B.S. in Pharmacy from the University of Cincinnati and a Ph.D. in Pharmaceutics and Pharmaceutical Chemistry from The Ohio State University.

Gregory A. Deener was appointed Vice President of Marketing and Sales on January 20, 2005, and prior to that he 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.

T. Gary Bird, Ph.D. has served as our Director of Corporate Quality 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. was appointed Director of Drug Safety on January 20, 2005. Prior to that he served as our Director of Clinical Development since May 2003. From January 2002 until he joined GTx, Dr. Boger was a private consultant specializing in medicine, pharmacology and clinical research. From 1997 to January 2002, Dr. Boger was Director of Clinical Research for Transplantation and Immunology for Novartis Pharmaceuticals. From 1996 to 1997, Dr. Boger served as Director of Medical Research and Clinical Science Leader of Roche's CellCeptTransplant program. Prior to joining Roche, Dr. Boger served as both Associate Director, Clinical Research and Medical Director, Renin Inhibitor Venture for Abbott Laboratories. Dr. Boger holds a B.A. in Biophysics from Amherst College and an M.D. from Harvard Medical School. Dr. Boger is board certified in internal medicine, nephrology and clinical pharmacology.

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

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

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.

As of December 31, 2004, we had an accumulated deficit of $157.4 million, of which $96.3 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. Other than our right to distribute FARESTON in the United States, we currently have no other products approved for commercial sale, and, to date, we have not generated any product revenue other than sales of FARESTON. We have received an upfront license fee of $6.7 million from Ortho Biotech for our joint collaboration for the development and commercialization of andarine and specified backup SARM compounds, which is being amortized over 5 years. 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 additional sales and marketing expenses 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 our current cash resources, and interest on these funds and product revenue from the sale of FARESTON 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 will 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. Significant delays in clinical testing could materially impact our product development costs. We do not know whether planned clinical trials will begin on time, will need to be restructured or will be completed on schedule, if at all. 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.

Our efforts to discover, develop and commercialize some of our new product candidates are at an early stage and are subject to high risk of failure.

The process of successfully developing product candidates is very time-consuming, expensive and unpredictable. We have only recently initiated Phase I clinical trial studies of ostarine and our efforts toward the development of prostarine and andromustine, two of our early-stage product candidates, are still preclinical. We do not know whether our planned preclinical studies or clinical trials for these or other compounds in our clinical pipeline will begin on time or be completed on schedule, if at all. In addition, we do not know whether these clinical trials will result in marketable products. Typically, there is a high rate of attrition for product candidates in preclinical and clinical trials.

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 and FARESTON. In the event that Orion terminates the agreement under specified circumstances, we would not be able to manufacture ACAPODENE or FARESTON until Orion's patents with respect to the composition of matter of toremifene, the active pharmaceutical ingredient in ACAPODENE and FARESTON, expire. This could delay the development of and impair our ability to commercialize ACAPODENE and FARESTON. 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 and FARESTON, but we would be required to make arrangements with a qualified alternative supplier to do so.

In addition, we currently rely on EaglePicher Pharmaceutical Services, a division of EaglePicher Technologies, LLC, as our single supplier of andarine. We do not have a contract with EaglePicher 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. EaglePicher also is the sole supplier of ostarine, which Metrics, Inc. is packaging and supplying to our Phase I clinical trial site. We do not have a contract with EaglePicher for the supply of ostarine for full-scale commercialization.

We may not be able to maintain or renew our existing or any other third-party manufacturing arrangements on acceptable terms, if at all. If we are unable to continue relationships with Orion for ACAPODENE and EaglePicher 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 or for ostarine 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 FDA, would delay or prevent the clinical development and commercialization of these product candidates.

Our dependence on Orion Corporation could hinder our ability to distribute FARESTON and receive regulatory approval for ACAPODENE.

We rely on Orion to maintain and update the website currently in use to market FARESTON. If Orion allows this website to become non-operational or otherwise caused to be shut down, whether for legal, regulatory or technical reasons, we will lose access to an important source of advertising.

We also rely on Orion to cooperate with us in the filing and maintenance of regulatory filings with respect to FARESTON and ACAPODENE. If Orion does not cooperate with us or otherwise does not successfully file or maintain these regulatory filings, we may not be able to keep our regulatory filings current, which could impact our ability to distribute FARESTON and delay regulatory approval of ACAPODENE.

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 toremifene for third parties for sale outside the U.S. 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 for the development of andarine, subjects 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.

In March 2004, we entered into a joint collaboration and license agreement with Ortho Biotech for andarine. Pursuant to this agreement, an affiliate of Ortho Biotech will be responsible for further clinical development and related expenses for andarine. If andarine is approved for commercial sale, Ortho Biotech will exclusively market andarine in the United States and in markets outside the United States. Under the agreement, we have the option to copromote andarine and other licensed SARM compounds to urologists in the United States for indications specifically related to men's health. Ortho Biotech may terminate the development or commercialization of any compound under the agreement upon 90 days' notice, or 30 days' notice if there are safety issues. Any loss of Ortho Biotech as a collaborator in the development or commercialization of andarine, dispute over the terms of the collaboration or other adverse development in our relationship with Ortho Biotech could materially harm our business and might accelerate our need for additional capital.

Risks Related to Our Intellectual Property

Our license agreement with Orion Corporation excludes the use of toremifene in humans to treat breast cancer outside the U.S. and may limit our ability to market ACAPODENE for human uses of toremifene outside the U.S.

Our exclusive license from Orion outside the U.S. excludes the use of toremifene for the treatment of breast cancer. Orion has licensed other parties outside the U.S. to market, sell and distribute toremifene for the treatment of advanced breast cancer and could license additional parties to market, sell and distribute toremifene for this indication outside the United States.

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 those development plans could adversely affect Orion's or its other licensees' activities for toremifene for breast cancer outside the U.S. Although we do not believe that our development plans adversely affect these activities, any future modifications to our plans imposed by Orion 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 Orion's patents covering toremifene in such countries 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 us and our affiliates, 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 been issued or may be issued with respect to our owned or licensed patent applications relating to the use of ACAPODENE for the relevant indications. To date, most of our applications for method of use patents filed outside of the United States are still pending and 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 create a regulatory environment that encourages 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” 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 our sales of ACAPODENE and FARESTON 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 or marketing FARESTON.

In all countries in which we hold or have licensed rights to patents or patent applications related to ACAPODENE or FARESTON, the composition of matter patents we license from Orion will expire before our method of use patents, and in some countries, the composition of matter patents have already expired. Method of use patents may not protect ACAPODENE or FARESTON 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 or its equivalent. Such off-label uses are common across medical specialties. Any such off-label sales would adversely affect our ability to generate revenue from the sale of ACAPODENE, if approved for commercial sale, and from FARESTON.

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 in such countries 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 breast cancer outside the United States, physicians in such countries 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 are issued with respect to 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 completed Phase IIb clinical trial and our ongoing 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 ongoing 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 other than FARESTON in the U.S. for the next few years. The inability to obtain FDA approval or approval from comparable authorities in other countries for such candidates would prevent us from commercializing our product candidates in the United States or other countries. See “Government Regulation” 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.

Our only marketed product generating revenue is FARESTON. If FARESTON does not receive continued market acceptance, sales may not continue and we may not earn sufficient revenues.

FARESTON is currently our only marketed product generating sales. We cannot be certain that FARESTON will continue to be accepted in its markets. Specifically, the following factors, among others, could affect the level of market acceptance of FARESTON:

- the perception of the healthcare community of FARESTON’s safety and efficacy, both in an absolute sense and relative to that of competing products
- the effectiveness of our sales and marketing efforts;
- any unfavorable publicity regarding FARESTON or competitive products;
- the price of FARESTON relative to other competing drugs or treatments;
- any changes in government and other third-party payor reimbursement policies and practices; and
- regulatory developments affecting the manufacture, marketing or use of FARESTON.

If the acceptance of FARESTON does not continue, it will reduce our revenues, which may impact the success of our business and the price of our common stock.

Our customer base is highly concentrated and a loss of a customer could materially impact our FARESTON sales.

Our principal customers for FARESTON are wholesale drug distributors. These customers comprise a significant part of the distribution network for pharmaceutical products in the United States. Four large wholesale distributors control a significant share of our market. These wholesaler customers, Cardinal Health, Inc., McKesson Corporation, AmerisourceBergen Corporation and Luis Garraton, in the aggregate, accounted for approximately 70% of the FARESTON business. The loss or bankruptcy of any of these customers could materially and adversely affect our results of operations and financial condition.

If we are unable to expand our 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 from such candidates.

We have limited experience as a company in the sales and distribution of pharmaceutical products. There are risks involved with building our own sales and marketing capabilities, as well as entering into arrangements with third parties to perform these services. For example, building a sales force is expensive and time-consuming and could delay any launch of a product candidate. 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 products we sell at acceptable prices, our revenues and prospects for profitability will suffer.

Many patients will not be capable of paying for FARESTON or any products themselves 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 or for FARESTON or for other products that we may sell, 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 FARESTON or 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 or products we sell. Cost-control initiatives could decrease the price we might establish for products that we may develop or that we sell, 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 or for FARESTON or other products that we may sell, 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 commercial sale of FARESTON and the testing of our product candidates in human clinical trials and will face an even greater risk if we commercially sell any product that we may develop. If we cannot successfully defend ourselves against claims that our product candidates or products caused injuries, we will incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for any product candidates or products;
- 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 for which we obtain or hold approvals.

We have product liability insurance that covers our clinical trials and commercial products up to a $20 million annual aggregate limit. 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 opportunities 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.

Tamoxifen, which is marketed by AstraZeneca, has been approved by the FDA for the treatment of advanced breast cancer and the reduction of breast cancer in women at high risk for developing the disease. In addition, aromatase inhibitors (AIs) have demonstrated efficacy and tolerability advantages compared to tamoxifen. Physicians are increasing their prescribing of AIs at the expense of tamoxifen, FARESTON and other SERMS. If this trend accelerates, it will negatively impact FARESTON sales. AIs are currently marketed by large pharmaceutical companies, which include Novartis and AstraZeneca.

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.

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 or products, 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.

As of March 1, 2005, our officers, directors and holders of 5% or more of our outstanding common stock beneficially owned approximately 79% 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 1, 2005, we had 24,664,716 shares of common stock outstanding, of which approximately 5.6 million may be resold in the public market immediately based upon the information available to us. The remaining 19.1 million shares, or 77.2% of our outstanding shares, are currently restricted as a result of securities laws, but are eligible for sale in the public market under Rules 144, 144(k) and 701, subject in some cases to volume and other limitations.

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 approximately 11.1 million 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 are 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

We sublease approximately 53,000 square feet of laboratory and office space in Memphis, Tennessee, under an operating lease through September 2005. The lease is terminable by either party upon ninety days' notice. We are currently negotiating a new sublease with a term of two years and an option to extend the sublease for an additional three years.

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

Not applicable.

PART II

ITEM 5. MARKET FOR THE REGISTRANT'S COMMON EQUITY AND RELATED STOCKHOLDER MATTERS AND ISSUER PURCHASES OF EQUITY SECURITIES

Description of our Securities

Our common stock began trading on the Nasdaq National Market ("NASDAQ") under the symbol "GTXI" on February 3, 2004. Prior to that date, there was no established public trading market for our common stock. The following table presents, for the periods indicated, the high and low closing sales prices per share of our common stock reported on the NASDAQ National Market.

	Common Stock 2004	
	High	Low
First Quarter	$ 12.90	$ 9.67
Second Quarter	14.14	10.41
Third Quarter	11.66	8.51
Fourth Quarter	14.86	11.15

On March 3, 2005 the closing price of our common stock on NASDAQ was $10.10 per share and there were approximately 49 holders of record and approximately 2,000 beneficial holders of our 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, 2004:

Plan Category	Number of securities to be issued upon exercise of outstanding options(1)(2) (a)	Weighted-average exercise price of outstanding options (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	1,143,207	$ 7.66	1,830,800
Equity compensation plans not approved by security holders	—	—	—
Total	1,143,207	$ 7.66	1,830,800

(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 may 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. By an action of the Company's Board of Directors on December 29, 2004, the Board elected not to increase for 2005 the number of shares available under the 2004 Equity Incentive Plan. 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. On January 1, 2005, the options available for issuance under the 2004 Non-Employee Directors' Stock Option Plan increased to a total of 250,000. 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.

(2) At December 31, 2004, we had no outstanding warrants or rights.

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

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 ("IPO") of our common stock. Goldman Sachs & Co., SG Cowen Securities Corporation, and Lazard Frères & Co. LLC acted as the underwriters for the offering.

The Company's common stock began trading on The Nasdaq National Market under the trading symbol "GTXI" on February 3, 2004. The Company sold 5,400,000 shares of common stock in our IPO 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. The Company paid the underwriters a commission of $5.5 million and incurred offering expenses of $2.4 million. The offering expenses included approximately $100,000 in lease payments paid to Pittco, Inc., a company owned by Mr. Hyde, Chairman of the Board of Directors, for the rental of Pittco, Inc.'s airplane during the IPO road show. The Company was reimbursed $39,000 of the $100,000 from the underwriters. Other than the fees paid to Pittco, Inc., none of the IPO expenses were paid directly or indirectly to our directors, officers or persons owning 10% or more of our common stock. After deducting the underwriters' commission and the offering expenses, the Company received net proceeds of $70.4 million. From the time of receipt through December 31, 2004, we invested the available net proceeds in short-term securities. In addition, approximately $14.4 million of the proceeds were used to fund our operations through December 31, 2004, $1.1 million of the proceeds were used for capital expenditures and $4.8 million of the net proceeds were used to acquire a license from Orion Corporation. We plan to use the balance of 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 products, technologies or businesses, although we currently have no commitments or agreements relating to any of these types of transactions.

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.

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, 2004 from our audited financial statements.

	Year Ended December 31,				
	2004	2003	2002	2001	2000
	(in thousands, except per share data)				
Statement of Operations Data:					
Total collaboration revenue	$ 1,867	$ —	$ —	$ —	$ —
Operating expenses: (a)					
Research and development	17,950	10,778	9,569	5,921	2,743
General and administrative	7,211	3,559	2,453	2,225	1,219
Loss from operations	(23,294)	(14,337)	(12,022)	(8,146)	(3,962)
Interest income	946	143	156	83	150
Net loss	(22,348)	(14,194)	(11,866)	(8,063)	(3,812)
Accrued preferred stock dividends	(455)	(3,436)	(2,147)	(790)	(297)
Adjustment to preferred stock redemption value	17,125	(77,844)	(7,220)	(57)	(21,077)
Net loss attributable to common stockholders	$ (5,678)	$ (95,474)	$ (21,233)	$ (8,910)	$ (25,186)
Net loss per share attributable to common stockholders					
Basic	$ (0.25)	$ (12.34)	$ (2.75)	$ (1.15)	$ (3.26)
Diluted	$ (0.93)	$ (12.34)	$ (2.75)	$ (1.15)	$ (3.26)

	As of December 31,				
	2004	2003	2002	2001	2000
			(in thousands)		
Balance Sheet Data:					
Cash and cash equivalents	$ 64,528	$ 14,769	$ 8,925	$ 8,834	$ 2,667
Working capital	61,298	12,775	7,654	8,544	2,241
Total assets	73,082	17,310	10,030	10,117	3,201
Cumulative redeemable convertible preferred stock	—	165,292	64,026	43,702	27,912
Accumulated deficit	(157,430)	(151,752)	(56,278)	(35,045)	(26,135)
Total stockholders' equity(deficit)	63,909	(150,231)	(55,308)	(34,075)	(25,165)

(a) Depreciation expense for the years ended December 31, 2003, 2002, 2001 and 2000 have been reclassified to research and development expense and general and administrative expense to conform to our 2004 presentation.

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.

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 and oncology. Our lead 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 have four clinical programs. We are developing ACAPODENE for two clinical programs in men: (1) a pivotal Phase III clinical trial for the prevention of prostate

cancer in high risk men and (2) a pivotal Phase III clinical trial for the treatment of serious side effects of androgen deprivation therapy (ADT) for advanced prostate cancer. In our third clinical program, together with our partner Ortho Biotech, we are developing andarine, a SARM, which we are working with our partner to progress to Phase II clinical testing in the second half of this year. In our fourth clinical program, we are developing our second SARM, ostarine, for andropause and other chronic conditions related to aging, including sarcopenia. We have an extensive preclinical pipeline generated from our own discovery program which has focused on research and development efforts of SERM and SARM compounds, other receptor modulator compounds and viral cytolytics. Two specific product candidates developed as a result of our discovery efforts include prostarine, a SARM for BPH, and andromustine, an anti-cancer drug for hormone refractory prostate cancer. We acquired the rights to distribute FARESTON in the U.S. from Orion in December 2004. 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 and FARESTON prescribers, principally medical oncologists, in the United States and to market andarine to urologists in the United States.

On February 6, 2004, we successfully completed an IPO of 5,400,000 shares of common stock at an offering price to the public of $14.50 per share resulting in net proceeds of $70.4 million. Upon the closing of the IPO, all outstanding shares of preferred stock, and accrued dividends thereon, were converted into 11,521,075 shares of common stock.

In March 2004, we entered into a joint collaboration and license agreement with Ortho Biotech for andarine and specified backup SARM compounds. Under the terms of the agreement, we received in April 2004 an up-front licensing fee and reimbursement of development expenses of $6.7 million. The up-front licensing fee and reimbursement of development expenses are expected to be amortized into revenue on a straight-line basis through March 2009. Additionally, we will receive licensing fees and milestone payments of up to $82.0 million based on andarine and up to $45.0 million for each additional licensed compound achieving specific clinical development decisions or obtaining regulatory approvals. All milestone payments are based on achievements prior to the commercial launch of andarine. Johnson & Johnson Pharmaceutical Research & Development will be responsible for further clinical development and related expenses for andarine and other licensed SARM compounds. Ortho Biotech will be responsible for commercialization and related expenses for andarine and other licensed SARM compounds. If andarine is approved for commercial sale, Ortho Biotech will exclusively market andarine in the United States and in 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 up to double digit royalties on all United States and worldwide sales plus additional royalty payments in excess of 20% on all co-promoted sales generated from urologists in the United States.

In December 2004, we purchased from Orion all remaining rights to toremifene in the U.S. and additional rights in all other countries giving us exclusive global rights to all toremifene-based products for all indications in humans, except breast cancer outside of the U.S. Toremifene is the active component in ACAPODENE and FARESTON, which has been approved by the FDA for the treatment of metastatic breast cancer. Under the terms of our purchase agreement with Orion, we were required to pay Orion a license fee of $4.8 million and to purchase FARESTON inventory of approximately $448,000. We will continue to market FARESTON in the U.S. for the treatment of metastatic breast cancer and will pay a royalty to Orion on FARESTON sales. The royalty rate for FARESTON will be reduced after we commercialize a new toremifene based product such as ACAPODENE for men’s health indications. Additionally, as part of our acquisition agreement with Orion, our license and supply agreement with Orion has been amended to provide that Orion will manufacture and supply all of our needs for clinical trial and commercial grade material for toremifene-based product developed and marketed globally by us, including ACAPODENE and FARESTON.

Critical Accounting Policies

Our management’s discussion and analysis of our financial condition and results of operations is based on our consolidated financial statements, which have been prepared in accordance with accounting principles generally accepted in the United States of America. The preparation of these financial statements requires us to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of the financial statements as well as the reported revenues and expenses during the

reporting periods. In preparing these financial statements, management has made its best estimates and judgments of certain amounts included in the financial statements giving due consideration to materiality. On an ongoing basis, we evaluate our estimates and judgments related to revenue recognition, income taxes, intangible assets, long-term service contracts and other contingencies. We base our estimates on historical experience and on various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions.

While our significant accounting policies are more fully described in Note 2 to our financial statements appearing at the end of this annual report, we believe that the following accounting policies are most critical to aid you in fully understanding and evaluating our reported financial results.

Revenue Recognition

We use revenue recognition criteria outlined in Staff Accounting Bulletin No. 101, *"Revenue Recognition in Financial Statements"* and Emerging Issues Task Force ("EITF") Issue 00-21, *"Revenue Arrangements with Multiple Deliverables"* ("EITF 00-21"). Accordingly, revenues from licensing agreements are recognized based on the performance requirements of the agreement. Non-refundable up-front fees, where we have an ongoing involvement or performance obligation, are generally recorded as deferred revenue in the balance sheet and amortized into license fees in the statement of operations over the term of the performance obligation. We estimated the performance obligation period to be five years for the development of andarine. The factors that drive the actual development period of a pharmaceutical product are inherently uncertain, and include determining the timing and expected costs to complete the project, projecting regulatory approvals and anticipating potential delays. We use all of these factors in initially estimating the economic useful lives of our performance obligations, and we also continuously monitor these factors for indications of appropriate revisions.

Revenues derived from reimbursements of costs associated with the development of andarine are recorded in compliance with EITF Issue 99-19, *"Reporting Revenue Gross as a Principal Versus Net as an Agent"* ("EITF 99-19"). According to the criteria established by this EITF Issue, in transactions where we act as a principal, have discretion to choose suppliers, bear credit risk and perform part of the services required in the transaction, we have met the criteria to record revenue for the gross amount of the reimbursements.

Research and Development Costs

We expense 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 our behalf.

Patent Costs

We expense patent costs, including legal expenses, in the period in which they are incurred. Patent expenses are included in general and administrative expenses in our statements of operations.

Preferred Stock Redemption Value

Our preferred stock was recorded at its redemption value. The per share redemption price was equal to the greater of liquidation value, which included accrued dividends, or the fair value calculated on an as-if converted to common stock basis. We determine a 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 our initial public offering price per common share. The changes in redemption value affect the loss attributable to common stockholders.

Deferred Stock Compensation

In anticipation of our IPO on February 6, 2004, 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 from June 30, 2003 to December 31, 2003. Accordingly, we recorded non-cash deferred stock-based compensation expense of $4.1 million in 2003, and are amortizing the related expense over the service period, which is generally five years. Deferred stock compensation for options granted to employees has been determined as the difference between the deemed fair value of our common stock for financial reporting purposes on the date such options were granted and the applicable exercise price. Such amount is included as a reduction of stockholders' equity and is being amortized on the straight-line basis.

Income Taxes

We account 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 basis of assets and liabilities using enacted tax rates in effect for the year in which the differences are expected to reverse. A valuation allowance is provided when it is more likely than not that some portion or all of the deferred tax assets will not be realized. This valuation allowance is estimated by management based on our projected future taxable income. The estimate of future taxable income is highly subjective. We have incurred losses since inception and anticipate that we will incur continued losses for the foreseeable future. However, these assumptions may be inaccurate, and unanticipated events and circumstances may occur in the future. To the extent actual results differ from these estimates, our future results of operations may be affected. At December 31, 2004, net of the valuation allowance, the net deferred tax assets were reduced to zero.

Purchased Intangible Assets

We account for our purchased intangible assets in accordance with Statement of Financial Accounting Standards ("SFAS") No. 142, *Goodwill and Other Intangible Assets*, which requires that purchased intangible assets with finite lives be amortized over their estimated economic lives. Our purchased intangible asset, license fee, represents a license fee paid to Orion in connection with entering into an Amended and Restated License and Supply Agreement. The license fee is being amortized on a straight-line basis over the term of the agreement which we estimate to be 16 years. Other purchased intangible assets represent the costs incurred to acquire software used by us. We amortize the cost of purchased software on a straight-line basis over the estimated useful lives of the software, generally three years. We use a discounted cash flow model to value our license fee. The discounted cash flow model requires assumptions about the timing and amount of future cash inflows and outflows, risk and the cost of capital. Each of these factors can significantly affect the value of the license fee. We review our license fee for impairment on a periodic basis using an undiscounted net cash flows approach. If the undiscounted cash flows of our license fee are less than its carrying value, it is written down to the discounted cash flow value. If we are unsuccessful in obtaining regulatory approval for ACAPODENE, we may not be able to recover the carrying amount of our license fee.

Recent Accounting Pronouncements

In December 2004, the Financial Accounting Standards Board ("FASB") issued SFAS No. 123 (revised 2004), *Share-Based Payment* ("SFAS 123R"), which replaces SFAS No. 123, *Accounting for Stock-Based Compensation* ("SFAS 123"), and supercedes APB Opinion No. 25, *Accounting for Stock Issued to Employees.* SFAS 123R requires all share-based payments to employees, including grants of employee stock options, to be recognized in the financial statements based on their fair values in the first interim or annual period beginning after June 15, 2005, with early adoption encouraged. The pro forma disclosures previously permitted under SFAS 123 no longer will be an alternative to financial statement recognition. We are required to adopt SFAS 123R in the third quarter of fiscal 2005, beginning July 1, 2005. Under SFAS 123R, we must determine the appropriate fair value model to be used for valuing share-based payments, the amortization method for compensation cost and the transition method to be used at date of adoption. The transition methods include prospective and retroactive adoption options. Under the retroactive option, prior periods may be restated either as of the beginning of the year of adoption or for all periods

presented. The prospective method requires that compensation expense be recorded for all unvested stock options and restricted stock at the beginning of the first quarter of adoption of SFAS 123R, while the retroactive methods would record compensation expense for all unvested stock options and restricted stock beginning with the first period restated. We are evaluating the requirements of SFAS 123R. We have not yet determined the method of adoption or the effect of adopting SFAS 123R, and we have not determined whether the adoption will result in amounts that are similar to the current pro forma disclosures under SFAS 123.

Research and Development

Since our inception, we have been focused on drug discovery and development programs. Research and development expenses represented approximately 71% of our total operating expenses for year ended December 31, 2004 and 75% of our total operating expenses for the year ended December 31, 2003. 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 product candidates, the development phase, the status, and research and development expense for each product candidate as well as information pertaining to our other research and development efforts for each of the periods presented. Research and development spending for past periods is not indicative of spending in future periods.

Research & Development Expense

Program	Product Candidate/ Indication	Development Phase	Status	Year Ended December 31, 2004	2003	2002
				(in thousands)		
SERM	**ACAPODENE** - Prevention of prostate cancer in men with high grade PIN	Pivotal Phase III clinical trial	Phase III trial initiated first quarter 2005	$ 2,247	$ 2,833	$3,168
	Side effects of androgen deprivation therapy	Pivotal Phase III clinical trial	Phase III trial initiated fourth quarter 2003	6,484	1,625	807
SARM	**Andarine** - Cachexia from various types of cancer	Four Phase I clinical trials completed	Planning Phase II trial second half of 2005	2,212	5,305	4,330
	Ostarine - Andropause and sarcopenia	Phase I	Phase I single ascending dose (SAD) trial completed first quarter 2005	4,011	—	—
			Planning Phase I multiple ascending dose (MAD) trial second quarter of 2005			
Other research and Development		Preclinical	Preclinical studies	2,996	1,015	1,264
Total research and development expense				$ 17,950	$10,778	$9,569

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 not 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, public relations and marketing functions. Other costs include facility costs not otherwise included in research and development expense and professional fees for legal, accounting, public relations and marketing services. We expect that our general and administrative expenses will increase in future periods as we add personnel, facilities (including our office space expansion), and infrastructure to support the planned growth of our business as well as additional expenses associated with operating as a public company. In addition, we plan to expand our sales and marketing efforts which will result in increased sales and marketing expenses in future years.

Results of Operations

Comparison of Years Ended December 31, 2004 and December 31, 2003

Collaboration Revenue. We recognized collaboration revenue for the year ended December 31, 2004 of $1.9 million. Collaboration revenue included $1.1 million from the amortization of the up-front license fee received in April 2004 in connection with our collaboration and license agreement with Ortho Biotech Products, L.P. In addition, collaboration revenue included $812,000 from the reimbursement of andarine development costs under our collaboration and license agreement. No revenue was recognized for the year ended December 31, 2003.

Research and Development Expenses. Research and development expenses increased 66.5% to $18.0 million for the year ended December 31, 2004 from $10.8 million for the year ended December 31, 2003. The $7.2 million increase in research and development expenses included increased expenditures of approximately $4.9 million related to 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 $4.0 million related to the preclinical development of ostarine and approximately $2.0 million on other research and development efforts. These increases were offset by a reduction in clinical trial expenses for the Phase IIb clinical trial of ACAPODENE for the prevention of prostate cancer in men with high grade PIN which was completed in the second quarter of 2004 of approximately $586,000 and a reduction in research and development spending on andarine of approximately $3.1 million. Research and development expenses included amortization of non-cash stock based compensation expense of $530,000.

We expect that research and development expenditures will continue to increase substantially in future years due to (1) the continuation of the pivotal Phase III clinical trial of ACAPODENE for the treatment of serious side effects of androgen deprivation therapy for advanced prostate cancer, (2) the continuation of a pivotal Phase III clinical trial of ACAPODENE for the prevention of prostate cancer in men with high grade PIN, (3) the continued clinical and preclinical development of other product candidates in the Company's SARM program that are not included in our collaboration with Ortho Biotech, including ostarine and prostarine, (4) the continued preclinical development of other product candidates including andromustine, (5) the increase in research and development personnel, (6) the planned expansion of office and lab space in our corporate headquarters and (7) prelaunch educational activities. 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 Expenses. General and administrative expenses increased 102.6% to $7.2 million for the year ended December 31, 2004 from $3.6 million for the year ended December 31, 2003. The increase of $3.6 million was primarily due to an increase in personnel related expenses, insurance costs, intellectual property related expenses, marketing and investor relations costs, professional fees and other administrative costs to support the planned growth of our business, as well as additional expenses associated with operating as a public company.

Interest Income. Interest income increased to approximately $946,000 for the year ended December 31, 2004 from approximately $143,000 for the year ended December 31, 2003. The increase was the result of higher average cash and cash equivalents balances during the year ended December 31, 2004 as compared to the prior year as a result of the IPO net proceeds of approximately $70.4 million and $6.7 million received as an up-front milestone payment from Ortho Biotech.

Adjustment to Preferred Stock Redemption Value. Our preferred stock was recorded at its redemption value. The per share redemption price was equal to the greater of liquidation value, which included accrued dividends, or the fair value calculated on an as-if converted to common stock basis. At December 31, 2003, the per share redemption value was determined based on the estimated projected midpoint of the range of our initial public offering price per common share of approximately $14.50 per share. At February 6, 2004, the date of the closing of the Company's IPO and automatic conversion of all outstanding preferred stock, and accrued dividends thereon, into common stock, the market price for our common stock was $12.90 per share. Prior to conversion into common stock, the carrying value of the preferred stock and accrued dividends was adjusted to reflect the per share redemption value on the date of conversion resulting in a decrease in the carrying value of preferred stock of $17.1 million and an offsetting decrease in net loss attributable to common stockholders. The adjustment to the preferred stock redemption value for the year ended December 31, 2003 was an increase of $77.8 million with an offsetting increase to net loss attributable to common stockholders. The increase in the redemption value for the year ended December 31, 2003 was the result of the achievement of significant milestones and clinical trials, general market conditions, and was made in connection with our anticipated initial public offering.

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

Research and Development. Research and development expenses increased 12.6% to $10.8 million for the year ended December 31, 2003 from $9.6 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 $975,000 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 $249,000.

General and Administrative. General and administrative costs increased 45.0% to $3.6 million for the year ended December 31, 2003 from $2.5 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."

Liquidity and Capital Resources

Through December 31, 2004, we have financed our operations and internal growth almost exclusively through private placements of preferred stock and with the proceeds of our IPO. We 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, 2004, we had an accumulated deficit of $157.4 million, of which $96.3 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 prevention of prostate cancer in men with high grade PIN, including our Phase IIb clinical trial and an ongoing pivotal Phase III clinical trial; ACAPODENE for the treatment of side effects of androgen deprivation therapy, including two Phase II clinical trials and an ongoing pivotal Phase III clinical trial; andarine for the treatment of cachexia from various forms of cancer, including preclinical development, manufacturing and formulation, and four Phase I clinical trials; preclinical development of ostarine which is being developed for the treatment of andropause; and our other research and development efforts;

- general and administrative expenses; and

- non-cash dividends and adjustments to the preferred stock redemption value of $96.3 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, expand our sales and marketing capabilities and grow our operations.

At December 31, 2004, we had cash and cash equivalents of $64.5 million, compared to $14.8 million at December 31, 2003. On February 6, 2004, we successfully completed an initial public offering of 5,400,000 shares of common stock at an offering price to the public of $14.50 per share, resulting in net proceeds of approximately $70.4 million.

Net cash used in operating activities was $15.7 million, $13.0 million and $10.6 million for the years ended December 31, 2004, 2003 and 2002, respectively. The use of cash in all periods resulted primarily from funding our

net losses. Net cash used in operating activities for the year ended December 31, 2004 was reduced by the up-front license fee and reimbursement of development expenses in connection with our collaboration with Ortho Biotech which is being amortized as income over the development period. Cash requirements for operating activities are expected to increase in future periods, due in part to significant costs related to the continuation of two pivotal Phase III clinical trials for ACAPODENE as well as the clinical and preclinical development of our other product candidates.

Net cash used in investing activities was $6.0 million, $108,000 and $313,000 for the years ended December 31, 2004, 2003 and 2002, respectively. The use of cash in all periods was primarily for the purchase of research and development equipment and office equipment and the purchase of an intangible asset (license fee) in 2004. We currently expect to make expenditures for capital equipment and leasehold improvements of up to $2.7 million for the year ended December 31, 2005.

Net cash provided by financing activities, was $71.4 million excluding approximately $1.0 million of IPO related expenses paid in 2003, $18.9 million and $11.0 million for the years ended December 31, 2004, 2003 and 2002, respectively. Net cash provided by financing activities for the year ended December 31, 2004 reflected net proceeds from the Company's IPO, which closed February 6, 2004, less underwriters' commission and offering expenses paid during the period. Net cash provided by financing activities for the years ended December 31, 2003 and 2002 reflected the net proceeds received from the issuance of preferred stock.

We believe that our current cash resources, interest on these funds and product revenue from the sale of FARESTON, 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. 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.

Until we can generate a sufficient amount of product revenue, 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 and revenues from the sale of FARESTON. 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, 2004, we had contractual obligations as follows:

	Payment Due by Period (in thousands)				
	Total	Less than 1 year	1-3 years	5 years	More than 5 years
Capital lease obligations	$ 24	$ 4	$ 15	$ 5	$ —
Operating lease obligations	182	182	—	—	—
Purchase obligations	135	135	—	—	—
Total	$ 341	$ 321	$ 15	$ 5	$ —

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.

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. The effect of a hypothetical 20% change in all interest rates on our investments would result in a change in interest income of $247 for the year ended December 31, 2004.

We operate primarily in the United States. Through December 31, 2004, we have not had any material exposure to foreign currency rate fluctuations. Our exposure to foreign currency rate fluctuations will increase because we are obligated to pay Orion Corporation, our supplier of ACAPODENE and FARESTON, in Euros; however such exposure is not expected to be material.

ITEM 8. FINANCIAL STATEMENTS AND SUPPLEMENTARY DATA

Our financial statements and the report of our independent registered public accounting firm are included in this Annual Report on Form 10-K beginning on page F-1. The index to this report and the financial statements are included in Item 15 below.

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

Not applicable.

ITEM 9A. 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.

We have carried out an evaluation, under the supervision and with the participation of our management, including, our Chief Executive Officer and Chief Financial Officer of the effectiveness of the design and operation of our disclosure controls and procedures (as defined in Rule 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended) 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.

There have been no significant changes in internal control over financial reporting during the fourth quarter of 2004 that have materially affected, or are reasonably likely to materially affect, the Company's internal control over financial reporting.

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.

As indicated on the cover page of this report, we are not an "accelerated filer" within the meaning of Rule 12b-2 under the Exchange Act. We must reassess our status of non-accelerated filer on June 30, 2005, based on our market capitalization as of that date. We will be required to include in our periodic filings under the Exchange Act the disclosures contemplated by Section 404 of the Sarbanes-Oxley Act beginning with our Annual Report on Form 10-K for the year ending December 31, 2005 if we are an accelerated filer as of June 30, 2005 or for the year ending December 31, 2006 if we are not an accelerated filer as of June 30, 2005. Section 404 will require us to include an internal control report of management in our Annual Report on Form 10-K. The internal control report must contain (1) a statement of management's responsibility for establishing and maintaining adequate internal control over financial reporting, (2) a statement identifying the framework used by management to conduct the required evaluation of the effectiveness of our internal control over financial reporting, (3) management's assessment of the effectiveness of our internal control over financial reporting as of the end of our most recent fiscal year, including a statement as to whether or not internal control over financial reporting is effective, and (4) a statement that our registered public accounting firm has issued an attestation report on management's assessment of internal control over financial reporting.

ITEM 9B. OTHER INFORMATION

Not applicable.

PART III

ITEM 10. DIRECTORS AND EXECUTIVE OFFICERS OF THE REGISTRANT

(a) The information required by this Item concerning our directors, code of ethics, audit committee, audit committee financial expert, and Section 16(a) beneficial ownership reporting compliance is incorporated by reference to our definitive proxy statement for the 2005 Annual Meeting of Stockholders to be filed with the Securities and Exchange Commission not later than 120 days after the end of the fiscal year covered by this Annual Report on Form 10-K (the "2005 Proxy Statement").

(b) The information required by this Item concerning our executive officers is set forth in the section entitled "*Executive Officers and Other Key Employees of Registrant"* in Part I of this Form 10-K and is incorporated by reference into this section.

ITEM 11. EXECUTIVE COMPENSATION

The information required by this Item is incorporated by reference to our 2005 Proxy Statement.

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

The information required by this Item regarding security ownership of certain beneficial owners and management, as well as equity compensation plans, is incorporated by reference to the information set forth in the sections "Security Ownership of Certain Beneficial Owners and Management" and "Executive Compensation" in our 2005 Proxy Statement.

ITEM 13. TRANSACTIONS WITH RELATED AND CERTAIN OTHER PARTIES

The information required by this Item is incorporated by reference to our 2005 Proxy Statement.

ITEM 14. PRINCIPAL ACCOUNTANT FEES AND SERVICES

The information required by this Item is incorporated by reference to our 2005 Proxy Statement.

PART IV

ITEM 15. EXHIBITS AND FINANCIAL STATEMENT SCHEDULES

(a)(1) Index to Financial Statements

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

(a)(3) See 15(b) below.

(b) 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)
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)

Number	Description
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)
10.24†	Joint Collaboration and License Agreement dated March 16, 2004, between Registrant and Ortho Biotech, L.P. (3)
10.25†	Purchase Agreement dated December 13, 2004, between Registrant and Orion Corporation (4)
10.26†	Amended and Restated License and Supply Agreement effective January 1, 2005, between Registrant and Orion Corporation (4)
13.1*	Annual Report to Shareholders
14.1	Code of Ethics (2)
23.1*	Consent of Ernst & Young LLP
24.1*	Power of Attorney
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.

* Previously filed.

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

(2) Incorporated by reference to the same exhibit filed with GTx's Annual Report on Form 10-K for the year ended December 31, 2003.

(3) Incorporated by reference to the same exhibit filed with GTx's Form 10-Q for the period ended March 31, 2004, filed on May 7, 2004.

(4) Incorporated by reference to Exhibits 10.1 and 10.2 filed with GTx's Current Report on Form 8-K/A, filed on March 7, 2005.

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
(Prinicpal Executive Officer)
Date: August 3, 2005

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
*	Chairman of the Board of Directors	August 3, 2005
J. R. Hyde, III		
*	Chief Executive Officer, Vice Chairman and Director (Principal Executive Officer)	August 3, 2005
Mitchell S. Steiner, M.D. F.A.C.S.		
*	Vice President, Chief Financial Officer and Treasurer (Principal Financial and Accounting Officer)	August 3, 2005
Mark E. Mosteller, CPA		
*	President, Chief Operating Officer and Director	August 3, 2005
Marc S. Hanover		
*	Director	August 3, 2005
Andrew M. Clarkson		
*	Director	August 3, 2005
J. Kenneth Glass		
*	Director	August 3, 2005
Robert Karr		
*	Director	August 3, 2005
Rosemary Mazanet, M.D., Ph.D.		
*	Director	August 3, 2005
John H. Pontius		
*	Director	August 3, 2005
Timothy R. G. Sear		
* By: /s/ Mark E. Mosteller		August 3, 2005
Mark E. Mosteller, as Attorney-in-Fact		

Exhibit Index

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)
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)
10.24†	Joint Collaboration and License Agreement dated March 16, 2005, between Registrant and Ortho Biotech, L.P. (3)

Number	Description
10.25†	Purchase Agreement dated December 13, 2004, between Registrant and Orion Corporation (4)
10.26†	Amended and Restated License and Supply Agreement effective January 1, 2005, between Registrant and Orion Corporation (4)
13.1*	Annual Report to Shareholders
14.1	Code of Ethics (2)
23.1*	Consent of Ernst & Young LLP
24.1*	Power of Attorney
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.

* Previously filed.

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

(2) Incorporated by reference to the same exhibit filed with GTx's Annual Report on Form 10-K for the year ended December 31, 2003.

(3) Incorporated by reference to the same exhibit filed with GTx's Form 10-Q for the period ended March 31, 2004, filed on May 7, 2004.

(4) Incorporated by reference to Exhibits 10.1 and 10.2 filed with GTx's Current Report on Form 8-K/A, filed on March 7, 2005.

GTx, Inc.

INDEX TO FINANCIAL STATEMENTS

REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

Board of Directors and Stockholders
GTx, Inc.

We have audited the accompanying balance sheets of GTx, Inc. as of December 31, 2004 and 2003, and the related statements of operations, cumulative redeemable convertible preferred stock and stockholders' equity (deficit) and cash flows for each of the three years in the period ended December 31, 2004. These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on these financial statements based on our audits.

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

In our opinion, the financial statements referred to above present fairly, in all material respects, the financial position of GTx, Inc. as of December 31, 2004 and 2003, and the results of its operations and its cash flows for each of the three years in the period ended December 31, 2004, in conformity with U.S. generally accepted accounting principles.

/s/ Ernst & Young LLP

Memphis, Tennessee
February 11, 2005

GTx, Inc.
BALANCE SHEETS
(in thousands, except share data)

	December 31, 2004	December 31, 2003
ASSETS		
Current assets:		
Cash and cash equivalents	$ 64,528	$ 14,769
Inventory	448	—
Prepaid expenses and other current assets	1,176	255
Total current assets	66,152	15,024
Property and equipment, net	1,537	793
Purchased intangible assets:		
License fee	4,826	—
Other	117	22
Other assets	450	—
Deferred initial public offering costs	—	1,471
Total assets	$ 73,082	$ 17,310
LIABILITIES, CUMULATIVE CONVERTIBLE PREFERRED STOCK AND STOCKHOLDERS' EQUITY (DEFICIT)		
Current liabilities:		
Accounts payable	$ 900	$ 461
Accrued expenses	2,617	1,788
Deferred revenue	1,337	—
Total current liabilities	4,854	2,249
Deferred revenue	4,295	—
Capital lease obligation	24	—
8% Cumulative Redeemable Convertible Preferred Stock, at redemption value $0.001 par value 1,677,500 shares authorized, 1,231,955 shares issued and outstanding at December 31, 2003; liquidation value of $59,209 at December 31, 2003	—	165,292
Commitments and contingencies		
Stockholders' equity (deficit):		
Common stock, $0.001 par value: 60,000,000 shares authorized; 24,664,716 shares issued and outstanding at December 31, 2004 and 7,735,848 shares issued and outstanding at December 31, 2003	25	8
Deferred stock compensation	(2,701)	(3,505)
Additional paid-in capital	224,015	5,018
Accumulated deficit	(157,430)	(151,752)
Total stockholders' equity (deficit)	63,909	(150,231)
Total liabilities and stockholders' equity (deficit)	$ 73,082	$ 17,310

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

GTx, Inc.
STATEMENTS OF OPERATIONS
(in thousands, except share and per share data)

	Year Ended December 31,		
	2004	2003	2002
Collaboration revenue:			
License fees	$ 1,055	$ —	$ —
Reimbursement of development costs	812	—	—
Total collaboration revenue	1,867	—	—
Operating expenses:			
Research and development	17,950	10,778	9,569
General and administrative	7,211	3,559	2,453
Total operating expenses	25,161	14,337	12,022
Loss from operations	(23,294)	(14,337)	(12,022)
Interest income	946	143	156
Net loss	(22,348)	(14,194)	(11,866)
Accrued preferred stock dividends	(455)	(3,436)	(2,147)
Adjustments to preferred stock redemption value	17,125	(77,844)	(7,220)
Net loss attributable to common stockholders	$ (5,678)	$ (95,474)	$ (21,233)
Net loss per share attributable to common stockholders:			
Basic	$ (0.25)	$ (12.34)	$ (2.75)
Diluted	$ (0.93)	$ (12.34)	$ (2.75)
Weighted average shares used in computing net loss per share attributable to common stockholders:			
Basic	22,993,221	7,735,125	7,734,998
Diluted	24,062,271	7,735,125	7,734,998

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

GTx, Inc.
STATEMENTS OF CUMULATIVE REDEEMABLE CONVERTIBLE PREFERRED STOCK AND STOCKHOLDERS' EQUITY (DEFICIT)
For the Period From January 1, 2002 to December 31, 2004
(in thousands, except share and per share data)

	Cumulative Redeemable Convertible Preferred Stock		Stockholders' Equity (Deficit)					
			Common Stock		Deferred Stock Compensation	Additional Paid-in Capital	Accumulated Deficit	Total Stockholders' Equity (Deficit)
	Shares	Amount	Shares	Amount				
Balances at January 1, 2002	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)
Preferred stock dividends	—	455	—	—	—	—	(455)	(455)
Preferred stock adjustment to redemption value	—	(17,125)	—	—	—	—	17,125	17,125
Conversion of preferred stock to common stock	(1,231,955)	(148,622)	11,521,075	12	—	148,610	—	148,622
Issuance of common stock	—	—	5,400,000	5	—	70,360	—	70,365
Amortization of stock-based compensation	—	—	—	—	804	—	—	804
Exercise of employee stock options	—	—	7,793	—	—	27	—	27
Net loss	—	—	—	—	—	—	(22,348)	(22,348)
Balances at December 31, 2004	—	$ —	24,664,716	$ 25	$ (2,701)	$ 224,015	$ (157,430)	$ 63,909

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

GTx, Inc.
STATEMENTS OF CASH FLOWS
(in thousands)

	Year Ended December 31,		
	2004	2003	2002
Cash flows from operating activities:			
Net loss	$ (22,348)	$ (14,194)	$ (11,866)
Adjustments to reconcile net loss to net cash used in operating activities:			
Depreciation and amortization	475	357	332
Stock-based compensation expense	804	550	—
License fee amortization	(1,055)	—	—
Changes in assets and liabilities:			
Inventory	(448)	—	—
Prepaid expenses and other current assets	(921)	(214)	159
Other assets	(567)	—	—
Accounts payable	439	(153)	340
Accrued expenses	1,263	657	482
Deferred revenue	6,687	—	—
Net cash used in operating activities	(15,671)	(12,997)	(10,553)
Cash flows from investing activities:			
Purchase of property and equipment	(1,174)	(108)	(313)
Purchase of intangible asset	(4,826)	—	—
Net cash used in investing activities	(6,000)	(108)	(313)
Cash flows from financing activities:			
Proceeds from issuance of common stock	71,836	1	—
Proceeds from issuance of preferred stock, net	—	19,986	10,957
Deferred initial public offering costs	(433)	(1,038)	—
Proceeds from exercise of employee stock options	27	—	—
Net cash provided by financing activities	71,430	18,949	10,957
Net increase in cash and cash equivalents	49,759	5,844	91
Cash and cash equivalents, beginning of year	14,769	8,925	8,834
Cash and cash equivalents, end of year	$ 64,528	$ 14,769	$ 8,925
Supplemental schedule of non-cash investing and financing activities:			
Preferred stock dividends	$ 455	$ 3,436	$ 2,147
Preferred stock adjustment to redemption value	$ (17,125)	$ 77,844	$ 7,220
Deferred initial public offering costs in accrued expenses	$ —	$ 433	$ —
Capital lease	$ 24	$ —	$ —
Transfer of deferred IPO costs to stockholders' equity (deficit)	$ 1,471	$ —	$ —

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

GTx, Inc.
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 and oncology. Our lead drug discovery and development programs are focused on small molecules that selectively modulate the effects of estrogens and androgens, two essential classes of hormones.

GTx has four clinical programs. The company is developing ACAPODENE® (toremifene citrate) for two clinical programs in men: (1) a pivotal Phase III clinical trial for the prevention of prostate cancer in high risk men and (2) a pivotal Phase III clinical trial for the treatment of serious side effects of androgen deprivation therapy (ADT) for advanced prostate cancer. In its third clinical program, GTx and its partner, Ortho Biotech Products, L.P., a subsidiary of Johnson & Johnson (Ortho Biotech), are developing andarine, a selective androgen receptor modulator (SARM), which we anticipate progressing to Phase II clinical testing in the second half of 2005. In its fourth clinical program, GTx is developing its second SARM, ostarine, for andropause and other chronic conditions related to aging, including sarcopenia. GTx also has a marketed product, FARESTON® (toremifene citrate 60mg) tablets for the treatment of metastatic breast cancer. The active compound in FARESTON is the same as in ACAPODENE.

GTx also has an extensive preclinical pipeline generated from its own discovery program, which includes the specific product candidates prostarine, a SARM for benign prostatic hyperplasia (BPH), and andromustine, an anticancer drug, for hormone refractory prostate cancer. We believe the four promising clinical programs along with the company's discovery pipeline create attractive long term commercial opportunities for GTx.

Stock Split and Initial Public Offering

On January 14, 2004, the Company effected an 8.5-for-1 stock split of its common stock in the form of a stock dividend. All common stock share and per share amounts in these financial statements have been adjusted retroactively to reflect the stock split.

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 to authorize 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.

2. Significant Accounting Policies

Basis of Presentation

Prior to March 2004, the Company operated as a development stage company and did not generate any revenue. Effective March 2004, the Company exited the development stage when it entered into a joint collaboration and license agreement with Ortho Biotech. The Company operates as one business segment.

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

Cash and Cash Equivalents

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

Inventory

Inventory consists of FARESTON tablets that are manufactured by Orion Corporation and delivered to the Company as finished goods. Inventory is stated at the lower of cost (first-in, first-out method) or market.

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 1). Such amounts were reclassified to additional paid-in capital upon completion of the Company's IPO.

Property and Equipment

Property and equipment is stated at cost. Depreciation is computed using the straight-line method over the estimated useful lives of the assets which range from three to five years. Amortization of leasehold improvements is recognized over the shorter of the estimated useful life of the leasehold improvement or the lease term.

Impairment of Long-Lived Assets

The Company accounts for long-lived assets in accordance with 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, 2004, 2003, and 2002. Should there be impairment in the future, the Company would recognize the amount of the impairment based on the expected future cash flows from the impaired assets. The cash flow estimates would be based on management's best estimates, using appropriate and customary assumptions and projections at the time.

Purchased Intangible Assets

The Company accounts for its purchased intangible assets in accordance with Statement of Financial Accounting Standards ("SFAS") No. 142, *Goodwill and Other Intangible Assets*, which requires that purchased intangible assets with finite lives be amortized over their estimated economic lives. The Company's purchased intangible asset, license fee, represents the value of a license and supply agreement purchased by the Company as described in Note 5. The license fee is being amortized on a straight-line basis over the term of the agreement which the Company estimates to be 16 years. Other purchased intangible assets represent the costs incurred to acquire software used by the Company. The Company amortizes the cost of purchased software on a straight-line basis over the estimated useful lives of the software, generally three years. Management analyzed the license fee in accordance with SFAS No. 144 and determined that there was no impairment as of December 31, 2004.

Fair Value of Financial Instruments

The carrying amounts of the Company's financial instruments, which include cash and cash equivalents, accounts payable and preferred stock approximate their fair values.

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

Concentration of Risk

Financial instruments that potentially subject the Company to a concentration of credit risk consist of cash and cash equivalents. The Company has established guidelines relating to diversification and maturities which allow the Company to manage risk.

Revenue Recognition

Revenues associated with the Company's collaboration and license agreement discussed in Note 8 consist of non-refundable, up-front license fees and reimbursement of development expenses. Through December 31, 2004, the Company has not generated any product revenue.

The Company uses revenue recognition criteria outlined in Staff Accounting Bulletin No. 101, *"Revenue Recognition in Financial Statements"* and Emerging Issues Task Force ("EITF") Issue 00-21, *"Revenue Arrangements with Multiple Deliverables"*. Accordingly, revenues from licensing agreements are recognized based on the performance requirements of the agreement. Non-refundable up-front fees, where the Company has an ongoing involvement or performance obligation, are generally recorded as deferred revenue in the balance sheet and amortized into license fees in the statements of operations over the term of the performance obligation.

Revenues derived from reimbursements of costs associated with the development of andarine are recorded in compliance with EITF Issue 99-19, *"Reporting Revenue Gross as a Principal Versus Net as an Agent"*. According to the criteria established by this EITF Issue, in transactions where the Company acts as a principal, has discretion to choose suppliers, bears credit risk and performs part of the services required in the transaction, the Company has met the criteria to record revenue for the gross amount of the reimbursements.

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 trials 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 basis of assets and liabilities using enacted tax rates in effect for the year in which the differences are expected to reverse. A valuation allowance is provided when it is more likely than not that some portion or all of the deferred tax assets will not be realized.

Reclassification

Depreciation expense for the years ended December 31, 2003 and 2002 have been reclassified to research and development expense and general and administrative expense to conform to the 2004 presentation.

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

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 SFAS No. 123, *Accounting for Stock-Based Compensation (*"SFAS No. 123"), as amended by SFAS No. 148, *Accounting for Stock-Based Compensation, Transition and Disclosure*. Under APB No. 25, unearned stock compensation is based on the difference, if any, on the date of grant, between the fair value of the Company's common stock and the exercise price.

SFAS No. 123 requires pro forma disclosure of net loss attributable to common stockholders, assuming all stock options were valued on the date of grant using the minimum value option pricing model for stock options granted prior to the Company's initial public offering in February 2004 and using the Black-Scholes option-pricing model for stock options granted after the IPO. The following weighted average assumptions were used for 2004, 2003 and 2002, respectively: risk free interest rates of 3.9%, 4.28% and 4.76%, expected volatility of 59.7%, 0.0% and 0.0%, no expected dividend yield, and expected option life of 6 years, 8 years and 8 years. If compensation cost for stock-based compensation plans had been determined under SFAS No. 123, the Company's net income (loss) attributable to common stockholders would have been the pro forma amounts indicated as follows:

	Year Ended December 31,		
	2004	2003	2002
Net loss attributable to common stockholders, as reported	$ (5,678)	$ (95,474)	$ (21,233)
Add: Deferred compensation amortization included in reported net loss	804	550	—
Deduct: Stock-based employee compensation determined under fair value based method for all awards	(1,319)	(424)	(115)
Pro forma net loss attributable to common stockholders	$ (6,193)	$ (95,348)	$ (21,348)
Pro forma SFAS No. 123 disclosure:			
Net loss per share attributable to common stockholders as reported:			
Basic	$ (0.25)	$ (12.34)	$ (2.75)
Diluted	$ (0.93)	$ (12.34)	$ (2.75)
Net loss per share attributable to common stockholders pro forma:			
Basic	$ (0.27)	$ (12.33)	$ (2.76)
Diluted	$ (0.95)	$ (12.33)	$ (2.76)

Deferred Stock Compensation

In anticipation of the Company's IPO on February 6, 2004, 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 from June 30, 2003 to December 31, 2003. Accordingly, the Company recorded non-cash deferred stock-based compensation of $4,055 as a reduction of stockholders' equity in 2003, and is amortizing the related expense over the service period, which is generally five years on the straight-line basis. Deferred stock compensation for options granted to employees has been determined as the difference between the deemed fair value of the Company's common stock for financial reporting purposes on the date such options were granted and the applicable exercise price. The Company recorded amortization of deferred stock compensation of approximately $804 and $550 for years ended December 31, 2004 and 2003, respectively. Of these amounts, $530 and $472 for the respective periods were included in research and development expenses and $274 and $78, respectively, were

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

included in general and administrative expenses in the statements of operations. At December 31, 2004, the Company had approximately $2,701 to be amortized over the remaining vesting periods of the stock options.

Basic And Diluted Net Loss Per Share

The Company computed net loss per common share according to Statement of Financial Accounting Standards No. 128, *"Earnings per Share,"* which requires disclosure of basic and diluted earnings (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 gives effect to the dilutive effect of potential common stock consisting of stock options and convertible preferred stock.

The following tables set forth the computation of the Company's basic and diluted net loss per common share attributable to common stockholders for the years ended December 31, 2004, 2003, and 2002:

	Year Ended December 31,		
	2004	2003	2002
Basic net loss per share			
Numerator:			
Net loss attributable to common stockholders	$ (5,678)	$ (95,474)	$ (21,233)
Denominator:			
Common stock outstanding at beginning of period	7,735,848	7,734,998	7,734,998
Conversion of preferred stock to common stock	10,387,855	—	—
Issuance of common stock in initial public offering	4,868,852	—	—
Other share activity	666	127	—
Weighted average shares used in computing basic net loss per share	22,993,221(1)	7,735,125	7,734,998
Basic net loss per share attributable to common stockholders	$ (0.25)	$ (12.34)	$ (2.75)

(1) The weighted average shares used in computing basic net loss per share attributable to common stockholders for the year ended December 31, 2004 include 4,868,852 shares, which represent the weighted average effect during the period of the issuance of 5,400,000 shares of common stock for the Company's IPO on February 6, 2004, and 10,387,855 shares, which represent the weighted average effect during the year of the issuance of 11,521,075 shares for the conversion of all preferred stock, and accrued dividends thereon, into common stock at the closing of the IPO. At December 31, 2004, the Company had outstanding 24,664,716 shares of common stock.

	Year Ended December 31,		
	2004	2003	2002
Diluted net loss per share			
Numerator:			
Net loss	$ (22,348)(2)	$ (14,194)(3)	$ (11,866)(3)
Denominator:			
Common stock outstanding at beginning of period	7,735,848	7,734,998	7,734,998
Conversion of preferred stock to common stock	11,456,905	—	
Issuance of common stock in initial public offering	4,868,852	—	—
Other share activity	666	127	—
Weighted average shares used in computing diluted net loss per share	24,062,271	7,735,125	7,734,998
Diluted net loss per share attributable to common stockholders	$ (0.93)	$ (12.34)	$ (2.75)

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

(2) Diluted net loss per share attributable to common stockholders is calculated as if the conversion of all preferred stock, and accrued dividends thereon, into shares of common stock occurred as of the beginning of the period. As a result, the diluted net loss per share attributable to common stockholders does not include accrued preferred stock dividends or the adjustments to preferred stock redemption value.

(3) Diluted net loss per share attributable to common stockholders is not calculated as if the conversion of all preferred stock, and accrued dividends thereon, into shares of common stock occurred as of the beginning of the year because their inclusion would have an anti-dilutive effect on the net loss for the year.

Outstanding options to purchase shares of common stock of 1,143,207, 828,750 and 363,375 were excluded from the calculation of diluted loss per share attributable to common stockholders for the periods ended December 31, 2004, 2003 and 2002, respectively, as inclusion of the options would have an anti-dilutive effect on the net loss per share for the periods. Of the 1,143,207 options outstanding at December 31, 2004, 1,103,207 had an exercise price less than the market price of the common stock at December 31, 2004.

Adjustment To Preferred Stock Redemption Value

The Company's preferred stock was recorded at its redemption value. The per share redemption price was equal to the greater of liquidation value, which included 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 Note 6).

Comprehensive Loss

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

Recent Accounting Pronouncements

In December 2004, the Financial Accounting Standards Board ("FASB") issued SFAS No. 123 (revised 2004), *Share-Based Payment* ("SFAS 123R"), which replaces SFAS No. 123, *Accounting for Stock-Based Compensation* ("SFAS 123"), and supercedes APB Opinion No. 25, *Accounting for Stock Issued to Employees.* SFAS 123R requires all share-based payments to employees, including grants of employee stock options, to be recognized in the financial statements based on their fair values in the first interim or annual period beginning after June 15, 2005, with early adoption encouraged. The pro forma disclosures previously permitted under SFAS 123 no longer will be an alternative to financial statement recognition. The Company is required to adopt SFAS 123R in the third quarter of fiscal 2005, beginning July 1, 2005. Under SFAS 123R, the Company must determine the appropriate fair value model to be used for valuing share-based payments, the amortization method for compensation cost and the transition method to be used at date of adoption. The transition methods include prospective and retroactive adoption options. Under the retroactive option, prior periods may be restated either as of the beginning of the year of adoption or for all periods presented. The prospective method requires that compensation expense be recorded for all unvested stock options and restricted stock at the beginning of the first quarter of adoption of SFAS 123R, while the retroactive methods would record compensation expense for all unvested stock options and restricted stock beginning with the first period restated. The Company is evaluating the requirements of SFAS 123R. The Company has not yet determined the method of adoption or the effect of adopting SFAS 123R, and it has not determined whether the adoption will result in amounts that are similar to the current pro forma disclosures under SFAS 123.

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

3. Property and Equipment

Property and equipment consist of the following:

	December 31, 2004	December 31, 2003
Leasehold improvements	$ 144	$ 113
Equipment	2,640	1,530
Furniture and fixtures	198	141
	2,982	1,784
Less: accumulated depreciation	1,445	991
	$ 1,537	$ 793

Depreciation expense for the years ended December 31, 2004, 2003 and 2002 was $454, $346 and $325, respectively.

4. Accrued Expenses

Accrued expenses consist of the following:

	December 31, 2004	December 31, 2003
Travel	$ 10	$ 20
Professional fees	60	522
Research and development	1,465	667
Clinical trial	849	550
Other	233	29
	$ 2,617	$ 1,788

5. Purchased Intangible Assets

Purchased intangible assets consist of the following:

	December 31, 2004	December 31, 2003
License fee	$ 4,826	$ —
Other purchased intangible assets	$ 161	$ 45
Less: accumulated amortization	(44)	(23)
	$ 117	$ 22

In accordance with the terms of the Amended and Restated License and Supply Agreement with Orion Corporation, the Company was required to pay a license fee of $4,826. This license fee is being amortized on a straight-line basis over the term of the agreement which the Company estimates to be 16 years (see Note 8). Other purchased intangible assets consist of software which is being amortized on a straight-line basis over its estimated useful life of 3 years. Amortization expense for the years ended December 31, 2004, 2003 and 2002 was $21, $11, and $7, respectively.

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

Estimated future amortization expense for purchased intangible assets at December 31, 2004 is as follows:

Year Ended December 31,	
2005	$ 349
2006	343
2007	332
2008	302
2009	302
Thereafter	3,315
Total	$ 4,943

6. Cumulative Redeemable Convertible Preferred Stock

The Company issued 8% Cumulative Redeemable Convertible Preferred Stock as follows:

		Shares Outstanding December 31,		Liquidation Value December 31,		Redemption Value December 31,	
Series	Date Issued	2004	2003	2004	2003	2004	2003
A	May 1999	—	200,000	—	$ 2,017	—	$ 25,763
B	July 2000	—	277,500	—	6,429	—	37,129
C	October 2001	—	260,154	—	17,817	—	37,955
D	July 2002	—	164,765	—	12,310	—	22,681
E	August 2003	—	329,536	—	20,636	—	41,764
		—	1,231,955	—	$ 59,209	—	$ 165,292

Dividends on the preferred stock compounded annually, were cumulative at the annual rate of 8% of the respective liquidation value and were payable at such time as such shares were converted or redeemed. The preferred stock was convertible 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. Concurrent with the Company's IPO, all outstanding preferred stock and accrued dividends were converted into 11,521,075 shares of common stock.

The Company's preferred stock was recorded at its redemption value. The per share redemption price was equal to the greater of liquidation value, which included accrued dividends, or the fair value calculated on an as-if converted to common stock basis. At December 31, 2003, the per share redemption value was determined based on the estimated projected midpoint of the range of the Company's initial public offering price per common share of approximately $14.50 per share. At February 6, 2004, the date of the closing of the Company's IPO and automatic conversion of all outstanding preferred stock, and accrued dividends thereon, into common stock, the market price for the Company's common stock was $12.90 per share. Prior to conversion into common stock, the carrying value of the preferred stock and accrued dividends was adjusted to reflect the per share redemption value on the date of conversion resulting in a decrease in the carrying value of preferred stock of $17,125 and an offsetting increase in stockholders' equity (deficit). The changes in redemption value affected the loss attributable to common stockholders. The adjustments to the preferred stock redemption value of preferred stock for the years ended December 31, 2003 and 2002 were increases of $77,844 and $7,220, respectively.

7. Common and Preferred Stock

The Company's certificate of incorporation authorizes the Company to issue 60,000,000 shares of common stock with $0.001 par value per share and 5,000,000 shares of Preferred Stock, par value $0.001.

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

8. License and Collaboration Agreements

University of Tennessee Research Foundation License Agreement

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 for the treatment and/or prevention of prostate cancer and premalignant lesions ("PIN") that may develop into prostate cancer. 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 superseded a 1998 license agreement with UTRF pursuant to which the Company reimbursed UTRF for certain patent expenses incurred by UTRF and agreed to make sublicense fee payments and future royalty payments.

In June 2002, the Company executed two Amended and Restated Exclusive License Agreements with UTRF granting the Company worldwide exclusive licenses under its composition of matter and method of use patents relating to SARM compounds, including andarine, to market, distribute and sell licensed 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 superseded a 2000 license agreement with UTRF pursuant to which the Company reimbursed UTRF for certain patent expenses incurred by UTRF and agreed to make sublicense fee payments and future royalty payments.

The Company also has executed with UTRF an Amended and Restated Exclusive License Agreement granting the Company worldwide exclusive licenses with UTRF's composition of matter and method of use patents for some of the Company's preclinical programs pertaining to viral cytolytics and gene therapy.

Orion Corporation License and Supply Agreement

On December 29, 2004, the Company entered into an Amended and Restated License and Supply Agreement ("License and Supply Agreement") with Orion Corporation ("Orion") granting the Company exclusive rights to Orion's compound, toremifene, for all products for human uses, including the Company's product candidate, ACAPODENE, excluding, however products for breast cancer sold outside of the United States. The License and Supply Agreement, which has an effective date of January 1, 2005, replaces an earlier agreement entered into with Orion in 2000, and subsequently amended in 2001 and 2003 ("Original License"). Under the agreement, the Company was required to pay a license fee of $4,826. The term of the agreement will survive for the term of the Company's patents, including the patents it licenses from UTRF pertaining to ACAPODENE for the treatment and/or prevention of PIN and prostate cancer. The Company believes that its patents pertaining to methods of use for toremifene will survive until at least 2020.

Under the Original License, the Company paid Orion $400, which it is allowed to offset along with clinical trial expenses against licensing fees and milestone payments it will pay to Orion if the Company sublicenses rights to its patents to third parties. The License and Supply Agreement retains these provisions and obligates the Company to make future royalty payments of varying amounts for toremifene based products for breast cancer in the US (FARESTON) or ACAPODENE to treat or prevent PIN or prostate cancer or to treat complications arising from androgen deprivation therapy.

The Company has agreed to achieve specified minimum sales requirements of ACAPODENE in the U.S. after commercialization of the product or it must pay Orion royalties 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 for the prevention or treatment of PIN or prostate cancer or to treat complications arising from

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

androgen deprivation therapy. Orion may terminate its supply Agreement if marketing approval for ACAPODENE is not granted in the U.S. by December 31, 2009.

Ortho Biotech Collaboration, License and Co-Promotion Agreement

In March 2004, the Company entered into a joint collaboration and license agreement with Ortho Biotech for andarine, its most advanced SARM compound, and specified backup SARM compounds. Under the terms of the agreement, the Company received in April 2004 an up-front licensing fee and reimbursement of development expenses of the completed Phase Id clinical trial for andarine totaling $6,687. Additionally, the Company will receive licensing fees and milestone payments of up to $82,000 based on andarine and up to $45,000 for each additional licensed compound achieving specific clinical development decisions or obtaining regulatory approvals. All milestone payments are based on achievements prior to the commercial launch of andarine. Johnson & Johnson Pharmaceutical Research & Development will be responsible for further clinical development and related expenses for andarine and other licensed SARM compounds. Ortho Biotech will be responsible for commercialization and related expenses for andarine and other licensed SARM compounds. If andarine is approved for commercial sale, Ortho Biotech will exclusively market andarine in the United States and in markets outside the United States. Under the agreement, the Company has 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. The Company will receive up to double digit royalties on all United States and worldwide sales plus additional royalty payments in excess of 20% on all co-promoted sales generated from urologists in the United States.

The up-front licensing fee and reimbursement of Phase Id clinical trial expenses for andarine totaling $6,687 are expected to be amortized into revenue on a straight-line basis through March 2009. The Company recognized revenue of $1,055 for the year ended December 31, 2004 from the amortization of the up-front license fee and expense reimbursement. Additionally, the Company recognized revenue of $812 for the year ended December 31, 2004 from the reimbursement of andarine development costs in accordance with this collaboration and license agreement. The reimbursement amount approximated the Company's actual expenses of which $514 and $298 were incurred in 2004 and 2003, respectively.

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

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,	
	2004	2003
Deferred income tax assets:		
Net federal and state operating loss carryforwards	$ 20,437	$ 14,795
Research credits	1,961	1,241
Cash basis method	528	778
Deferred stock compensation	786	215
Deferred revenue	2,196	—
Total deferred tax assets	25,908	17,029
Deferred income tax liabilities:		
Depreciation	49	35
Total deferred tax liabilities	49	35
Net deferred income tax assets	25,859	16,994
Valuation allowance	(25,859)	(16,994)
	$ —	$ —

At December 31, 2004, the Company had net federal operating loss carryforwards of approximately $52.5 million, which expire from 2018 through 2024 as follows: 2018 $0.5 million, 2019 $1.8 million, 2020 $7.8 million, 2021 $2.5 million, 2022 $10.8 million, 2023 $12.8 million and 2024 $16.3 million. The Company had state operating loss carryforwards of approximately $52.5 million , which expire from 2013 through 2019 as follows: 2013 $0.5 million, 2014 $1.8 million, 2015 $7.8 million, 2016 $2.5 million, 2017 $10.8 million, 2018 $12.8 million and 2019 $16.3 million. The Company also had research and development credits of $2.0 million, which expire from 2018 through 2024. Utilization of the net operating loss carryforwards and credits may be subject to a substantial annual limitation if certain events occur which result in an ownership change as defined 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 valuation allowance after considering available evidence concerning the realizations of these assets. At December 31, 2004, net of the valuation allowance, the net deferred tax assets were reduced to zero.

10. Operating Leases

The Company leases laboratory facilities and office space pursuant to leases accounted for as operating leases. Rent expense was approximately $219, $184 and $170 for the years ended December 31, 2004, 2003 and 2002, respectively.

11. Stock Option Plans

In 1999, 2000, 2001 and 2002, the Company adopted the Genotherapeutics, Inc. Stock Option Plan ("1999 Plan"), the GTx, Inc. 2000 Stock Option Plan ("2000 Plan"), the GTx, Inc. 2001 Stock Option Plan ("2001 Plan") and the GTx, Inc. 2002 Stock Option Plan ("2002 Plan"). 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, which amount may be increased annually on January 1st of each year from 2005 until 2013, by the lesser of five percent of the number of shares of

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

common stock outstanding on such date or an amount designated by the Company's Board of Directors. By an action of the Company Board of Directors on December 29, 2004, the Board elected not to increase for 2005 the number of shares available under the 2004 Equity Incentive Plan. The Company may issue options for up to 200,000 shares of common stock under the 2004 Non-Employee Directors' Stock Option Plan, which may be increased annually January 1st of each year, from 2005 until 2013, by the lesser of the number of shares of options granted during the prior calendar year or such amount designated by the Company's Board of Directors. On January 1, 2005, the options available for issuance under the plan increased to a total of 250,000. The Company's stock option plans allow 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. At December 31, 2004, 1,830,800 stock options were available for future issuance under the Company's equity compensation plans. The employee stock 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. The non-employee directors' stock options vest one-third on the first anniversary, one-third on the second anniversary and one-third on the third anniversary. 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 issued under the 1999 Plan, the 2000 Plan, the 2001 Plan, the 2002 Plan and 2004 Non-Employee Directors' Stock Option Plan will become fully vested and be converted to cash, options or stock of equivalent value. At December 31, 2004 and 2003, respectively, 184,172 and 101,269 of the Company's stock options were exercisable.

The following is a summary of stock option transactions for the three years ended December 31, 2004:

	Options	Weighted Average Exercise Price Per Share
Balances at January 1, 2002	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
Options granted	323,250	11.35
Options forfeited	(1,000)	8.90
Options exercised	(7,793)	3.48
Balances at December 31, 2004	1,143,207	$ 7.66

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

The following table summarizes information about stock options outstanding at December 31, 2004:

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 - $2.24	43,208	5.89	$ 2.24	32,585	$ 2.24
$6.24 - $8.90	894,999	8.29	6.75	151,587	6.60
$10.26 - $14.50	205,000	9.38	12.77	—	—
	1,143,207	8.59	$ 7.66	184,172	$ 5.83

The Company accounts for its stock-based compensation 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 $4,100 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 $515 and $115 for the years ended December 31, 2004 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. For stock options granted prior to the Company's IPO, 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. For stock options granted after the Company's IPO, the weighted average fair value of options granted was determined using the Black-Scholes option-pricing model assuming a weighted average risk free interest rate of 3.9%, weighted average volatility of 59.7%, no expected dividend yield and an expected option life of 6 years. The weighted average grant date fair value of options granted were $6.58, $8.02 and $2.26 for the years ended December 31, 2004, 2003 and 2002, respectively.

12. Employee Benefit Plan

The Company maintains 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 $13 for employees under age 50 and $16 for employees 50 and older in calendar year 2004. 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. Related Party Transactions

The Company purchased directors' and officers' liability insurance policies through an insurance agent who is the brother-in-law of an officer of the Company. Premiums paid to the insurance agency for the policies were approximately $825 for the year ended December 31, 2004. The Company retained the consulting services of the brother of an officer of the Company to provide graphic art, graphic design, and website design services. The

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

Company made payments for the year ended December 31, 2004 related to such consulting services of approximately $73, which included the reimbursement of third party printing services of $23. The Company reimbursed a company owned by the Company's Chairman of the Board of Directors for the use of its aircraft during the "road show" period of the Company's IPO. The amount reimbursed during the year ended December 31, 2004 was $100, of which $39 was reimbursed by the Company's IPO underwriters. GTx maintains investments in money market accounts, which were purchased through a brokerage account at a financial institution. GTx also has its checking account with the same financial institution. A member of our Board of Directors is the Chairman, President and Chief Executive Officer of that financial institution.

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

14. Quarterly Financial Data (Unaudited)

The following is a summary of the quarterly results of operations for the years ended December 31, 2004 and 2003.

	Quarters Ended Year 2004			
	March 31	June 30	September 30	December 31
Collaboration revenue:				
License fees	$ 52	$ 334	$ 335	$ 334
Reimbursement of development costs	—	760	42	10
Total collaboration revenue	52	1,094	377	344
Operating expenses: (a)				
Research and development	4,411	4,224	3,971	5,344
General and administrative	1,612	1,601	1,801	2,197
Total operating expenses	6,023	5,825	5,772	7,541
Loss from operations	(5,971)	(4,731)	(5,395)	(7,197)
Interest income	150	212	270	314
Net loss	(5,821)	(4,519)	(5,125)	(6,883)
Accrued preferred stock dividends	(455)	—	—	—
Adjustments to preferred stock redemption value	17,125	—	—	—
Net income (loss) attributable to common stockholders	$ 10,849	$ (4,519)	$ (5,125)	$ (6,883)
Net income (loss) per share attributable to common stockholders:				
Basic	$ 0.60	$ (0.18)	$ (0.21)	$ (0.28)
Diluted	$ (0.26)	$ (0.18)	$ (0.21)	$ (0.28)

	Quarters Ended Year 2003			
	March 31	June 30	September 30	December 31
Operating expenses: (a)				
Research and development	$ 2,187	$ 2,665	$ 2,499	$ 3,427
General and administrative	623	814	938	1,184
Total operating expenses	2,810	3,479	3,437	4,611
Interest 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)
Net income (loss) per share attributable to common stockholders:				
Basic	$ (0.46)	$ 0.09	$ (11.08)	$ (0.89)
Diluted	$ (0.46)	$ (0.22)	$ (11.08)	$ (0.89)

(a) Depreciation expense for the 2003 and 2004 quarters have been reclassified to research and development expense and general and administrative expense to conform to the 2004 fourth quarter presentation.

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)) 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) 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; and

   (c) 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 the 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, process, 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: August 3, 2005

/s/ MITCHELL S. STEINER
Mitchell S. Steiner, M.D., F.A.C.S.
Chief Executive Officer and Vice-Chairman of the Board of Directors

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)) 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) 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; and

   (c) 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 the 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, process, 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: August 3, 2005

/s/ MARK E. MOSTELLER
Mark E. Mosteller, CPA
Vice President and Chief Financial Officer

Exhibit 32.1

CERTIFICATION PURSUANT TO
18 U. S. C. SECTION 1350,
AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES -0XLEY ACT OF 2002

In connection with the Annual Report of GTx, Inc. (the "Company") on Form 10-K/A for the period ending December 31, 2004, 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, M.D., FACS
Chief Executive Officer

August 3, 2005

Exhibit 32.2

CERTIFICATION PURSUANT TO
18 U. S. C. SECTION 1350,
AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES -0XLEY ACT OF 2002

In connection with the Annual Report of GTx, Inc. (the "Company") on Form 10-K/A for the period ending December 31, 2004, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I, Mark E. Mosteller, Vice President and 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, CPA
Vice President and Chief Financial Officer

August 3, 2005