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UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-K

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

For the fiscal year ended December 31, 2013

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 000-50549

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.)
175 Toyota Plaza **7th Floor** **Memphis, Tennessee**	**38103**
(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:

Title of Each Class	Name of Each Exchange on Which Registered
Common Stock, par value $0.001 per share	The NASDAQ Stock Market, LLC

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

Indicate by check mark if the registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act. Yes o No x

Indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or Section 15(d) of the Act. Yes o No x

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 x No o

Indicate by check mark whether the registrant has submitted electronically and posted on its corporate Web site, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). Yes x 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. x

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, or a smaller reporting company. See the definitions of "large accelerated filer," "accelerated filer" and "smaller reporting company" in Rule 12b-2 of the Exchange Act:

Large accelerated filer o	Accelerated filer x
Non-accelerated filer o (Do not check if a smaller reporting company)	Smaller reporting company o

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Act). Yes o No x

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 28, 2013 as reported on The NASDAQ Global Market was $181,397,363.

There were 75,161,437 shares of registrant's common stock issued and outstanding as of March 10, 2014.

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DOCUMENTS INCORPORATED BY REFERENCE

Certain portions of the registrant's definitive proxy statement to be filed with the Securities and Exchange Commission pursuant to Regulation 14A, not later than 120 days after the end of the fiscal year covered by this Annual Report on Form 10-K, in connection with the Registrant's 2014 Annual Meeting of Stockholders are incorporated by reference into Part III of this Annual Report on Form 10-K.

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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 "Risk Factors," "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 that 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 implementation of our business strategies, including our ability to preserve or realize any significant value from our enobosarm (GTx-024) and GTx-758 (Capesaris®) programs;
- the therapeutic and commercial potential of our product candidates;
- the timing of regulatory discussions and submissions, and the anticipated timing, scope and outcome of related regulatory actions or guidance;
- our ability to establish and maintain potential new collaborative, partnering or other strategic arrangements for the development and commercialization of our product candidates;
- the anticipated progress of our clinical programs, including whether our ongoing and planned clinical trials will achieve clinically relevant results;
- the timing, scope and anticipated initiation, enrollment and completion of our ongoing and planned clinical trials and any other future clinical trials that we may conduct;
- our ability to obtain and maintain regulatory approvals of our product candidates and any related restrictions, limitations, and/or warnings in the label of an approved product candidate;
- our ability to market, commercialize and achieve market acceptance for our product candidates;

- 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, expenses, capital requirements and needs for additional financing, and our ability to obtain additional financing.

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, uncertainties and other important factors. We discuss many of these risks in this Annual Report on Form 10-K in greater detail in the section entitled "Risk Factors" under Part I, Item 1A below. Given these risks, uncertainties and other important factors, 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 incorporate by reference in and have filed as exhibits to this Annual Report on Form 10-K, 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.

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PART I

ITEM 1. BUSINESS

Overview

GTx, Inc., a Delaware corporation incorporated on September 24, 1997 and headquartered in Memphis, Tennessee, is a biopharmaceutical company dedicated to the discovery, development and commercialization of small molecules for the treatment of cancer, including treatments for prostate and breast cancer, cancer supportive care, including prevention and treatment of cancer-related muscle wasting or cachexia, and other serious medical conditions.

We are developing selective androgen receptor modulators, or SARMs, a new class of drugs with the potential to prevent and treat muscle wasting in patients with cancer and other musculoskeletal wasting or muscle loss conditions, including chronic sarcopenia (age related muscle loss), as well as the potential to be used as a hormonal therapy for the treatment of metastatic breast cancer. Our lead SARM product candidate, enobosarm (GTx-024), has to date been evaluated in fourteen completed or ongoing clinical trials enrolling approximately 1,320 subjects, including in three Phase 2 and two Phase 3 clinical trials. Enobosarm is the generic name given to the compound by the USAN Council and the World Health Organization and is the first compound to receive the SARM stem in its name, recognizing enobosarm as the first in this new class of compounds.

We announced in August 2013 that the POWER 1 (platinum plus taxane chemotherapy) and POWER 2 (platinum plus non-taxane chemotherapy) (Prevention and treatment Of muscle Wasting in patients with cancER) Phase 3 clinical trials evaluating enobosarm 3 mg for the prevention and treatment of muscle wasting in patients with advanced non-small cell lung cancer, or NSCLC, failed to meet the primary statistical criterion for the co-primary endpoints of lean body mass and physical function that were assessed statistically using responder analyses as pre-specified for the United States Food and Drug Administration, or FDA. However, efficacy data from the studies demonstrated enobosarm's consistent effect on maintaining or improving lean body mass compared to placebo and that maintenance or improvement in lean body mass is potentially associated with longer survival in patients, regardless of treatment. As for safety, enobosarm was generally well tolerated, with the occurrence of serious adverse events similar across the placebo and treated groups.

Enobosarm 3 mg demonstrated a statistically significant effect versus placebo on the primary endpoint of physical function through Day 84 in the POWER 1 Phase 3 clinical trial as assessed by continuous variable analysis, as pre-specified in our statistical analysis plan for the European Medicines Agency, or EMA. Therefore, we met with representatives from two member countries to the EMA in January of 2014 to review and discuss the results of the POWER trials to determine an appropriate path forward for submitting a marketing authorization application, or MAA, in the European Union for enobosarm 3 mg for the prevention and treatment of muscle wasting in patients with advanced NSCLC. Based on input from the two member countries, we believe data from the POWER 1 trial, as well as supporting data from the POWER 2 trial, are sufficient to support the submission of a MAA to the EMA seeking marketing authorization for enobosarm 3 mg in the more narrow indication of the prevention and treatment of muscle wasting in patients with advanced NSCLC treated with platinum plus taxane chemotherapy. We plan to initiate seven Phase 1 clinical studies that are typically required for registration purposes and develop a pediatric investigational plan, or PIP, necessary for submission of the MAA. We currently expect to submit the MAA to the EMA for enobosarm 3 mg for the prevention and treatment of muscle wasting in patients with advanced NSCLC treated with platinum plus taxane chemotherapy by the first quarter of 2015, after completion of the Phase 1 studies and acceptance of the PIP by the EMA's Pediatric Committee.

In our meeting with the FDA in February 2014 to review and discuss the results of the POWER clinical trials, we learned that since data from the two POWER trials failed to meet the primary statistical criterion pre-specified for the co-primary endpoints of lean body mass and physical function, the FDA was not willing to accept a new drug application, or NDA, for enobosarm 3 mg. However, based on input from the FDA meeting, we believe there is a regulatory path forward for enobosarm 3 mg in the United States, and we plan to meet again with the FDA to discuss a potential Phase 3 clinical program evaluating enobosarm 3 mg for an indication of muscle wasting or cachexia in patients with NSCLC. Any such Phase 3 clinical trial program would be subject to our ability to obtain additional funding and would be required to be successfully completed prior to any NDA submission to the FDA for enobosarm 3 mg.

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SARMs also have the potential to be used as a novel hormonal therapy for the treatment of metastatic breast cancer. Nonselective steroidal androgens have been used to treat breast cancer; however, the unwanted virilizing side effects have limited their widespread clinical use. We believe that enobosarm, by targeting the androgen receptor, or AR, in estrogen receptor, or ER, positive breast cancer, has the potential to provide clinical benefit to women with metastatic breast cancer by treating their disease while minimizing the unwanted masculinizing side effects associated with steroidal androgens, and unlike androgens, cannot be converted into an estrogen. In the second quarter of 2013, we initiated a Phase 2 open label study evaluating enobosarm 9 mg for the

treatment of AR positive and ER positive metastatic breast cancer in women who have previously responded to hormonal therapy for the treatment of their metastatic breast cancer. Nine clinical study sites in the U.S. have fully enrolled the study with 22 postmenopausal women with metastatic breast cancer to assess clinical benefit response after six months of enobosarm 9 mg treatment, which is defined as either those women receiving treatment who have demonstrated a complete response (disappearance of all targeted lesions), a partial response (at least a 30 percent decrease in the sum of the diameters of the targeted lesions) or stable disease (no disease progression from baseline). In February 2014, we reported that enobosarm 9 mg continues to be well tolerated by patients in the study, and that we expect to meet the pre-specified goal of demonstrating, after six months of treatment, at least three clinical benefit responses in at least 14 patients with AR positive and ER positive metastatic breast cancer. The study is ongoing and data from all patients in the study is expected late in the second quarter of 2014.

Additionally, we are developing GTx-758 (Capesaris®), an oral nonsteroidal selective ER alpha agonist, for secondary hormonal therapy in men with metastatic and nonmetastatic castration resistant prostate cancer, or CRPC, and, potentially, as a secondary hormonal treatment for advanced prostate cancer used in combination with androgen deprivation therapy, or ADT. We believe GTx-758 has the potential to reduce free testosterone without also causing certain estrogen deficiency side effects, such as bone loss and hot flashes, which are common with current androgen deprivation therapies for prostate cancer. We also believe that GTx-758 may be effective, in combination with ADT, as a secondary hormonal treatment of advanced prostate cancer by reducing free testosterone to levels lower than those attainable with ADT alone and potentially reducing the estrogen deficiency side effects caused by the use of ADT.

In May 2012, we announced that the FDA had removed its full clinical hold on our Investigational New Drug, or IND, application for GTx-758. The full clinical hold was placed on our three then ongoing Phase 2 clinical trials evaluating GTx-758 to treat men with advanced prostate cancer in February 2012 and resulted in the discontinuation of these trials. The full clinical hold followed our reports to the FDA of venous thromboembolic events (blood clots), or VTEs, in subjects treated with GTx-758 at the doses being studied in those trials (1000 mg and higher per day). Based upon feedback from the FDA in connection with the removal of the full clinical hold, we initiated in the third quarter of 2012 a Phase 2 clinical trial to evaluate the safety and efficacy of lower doses of GTx-758 as secondary hormonal therapy in men with metastatic CRPC.

GTx is currently conducting a Phase 2 open-label clinical trial in men who have developed metastatic or nonmetastatic CRPC while on ADT. GTx-758 has previously demonstrated the ability to increase the production of a protein called sex hormone binding globulin, or SHBG, that binds testosterone and thereby reduces free testosterone. By reducing free testosterone, we believe serum prostate specific antigen, or PSA, will be reduced in men with CRPC. The primary endpoint of the current Phase 2 clinical trial is the proportion of subjects with a $\geq$ 50% decline from baseline in serum PSA by Day 90. Other key endpoints include serum SHBG and total and free testosterone levels, as well as prostate cancer progression, in the study subjects. In addition, the clinical study will evaluate the ability of GTx-758 to treat certain estrogen deficiency side effects associated with luteinizing hormone releasing hormone, or LHRH, agonists such as hot flashes and bone loss. The Phase 2 clinical trial allows us to assess the safety and tolerability of GTx-758 in these subjects, including the incidence of VTEs. The original trial design provided for 75 total subjects to be enrolled in three sequential dosing arms, with the first 25 subjects in the study being enrolled in the GTx-758 125 mg dosing arm. Assuming that an acceptable incidence of VTEs was observed when the last subject enrolled in the GTx-758 125 mg dosing arm completed one 30 day cycle of therapy, enrollment of the next 25 subjects would commence in the GTx-758 250 mg dosing arm. Similarly, the GTx-758 500 mg dosing arm was to have commenced enrollment of the final 25 subjects when the last subject enrolled in the 250 mg dose arm completed one 30 day cycle of therapy, assuming an acceptable incidence of VTEs has been observed in both of the lower dosage arms and management had decided to continue testing at the next higher dose. After reviewing data collected from the GTx-758 125 mg dosing arm indicating the ability of the drug to substantially increase SHBG and lower free testosterone without any unexpected side effects occurring, the clinical trial protocol was amended in the third quarter of 2013 to eliminate the 500 mg dosing arm and to increase the number of subjects to be enrolled in the 125 mg and the 250 mg dosing arms to 38 patients per arm. Enrollment in the 125 mg cohort has been completed without any occurrence of VTEs, and after a pre-specified safety review by the independent Data Safety Monitoring Board, we are now enrolling subjects in the 250 mg arm. Depending upon

the safety and efficacy data observed in the 125 mg cohort and assuming no safety issues are observed in the first ten metastatic patients enrolled in the 250 mg cohort, enrollment of the 250 mg cohort will be opened to individuals with metastatic or nonmetastatic CRPC. The study is ongoing and data from all patients in the study is expected late in the second half of 2014.

At December 31, 2013, we had cash, cash equivalents and short-term investments of $14.7 million compared to $56.1 million at December 31, 2012. On March 6, 2014, we completed a private placement of units consisting of 11,976,048 shares of common stock and warrants to purchase 10,179,642 shares of our common stock for gross proceeds of approximately $21.3 million. If exercised in full, the warrants could result in additional gross proceeds of approximately $17.0 million.

In October 2013, we announced and implemented a plan to reduce our operating expenses, including a significant reduction in our workforce, in order to preserve capital while we evaluate feasible regulatory pathways for enobosarm 3 mg, conduct our two ongoing Phase 2 clinical trials of enobosarm 9 mg and GTx-758, and pursue discussions with potential partners. Therefore, we are no longer conducting drug discovery activities and are focusing our research and development activities on the ongoing clinical development of our current product candidates.

Scientific Background on Estrogen and Androgen Hormones and Selective Hormone Receptor Modulators

Estrogens and androgens are hormones that play critical roles in regulating the reproductive system and contributing to the homeostasis of the muscular, skeletal, cardiovascular, metabolic and central nervous systems.

Testosterone, the predominant androgen, is important for masculine physical characteristics, such as muscle size and strength and bone strength, as well as for mental well-being. Male reproductive health is dependent on testosterone for sexual interest, fertility, erectile function and normal prostate function. Testosterone is converted into a more potent androgen, dihydrotestosterone, or DHT, which acts as the primary androgen in the prostate, sebaceous glands and hair follicles, and may cause unwanted effects like benign prostatic hyperplasia, or BPH, 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, erectile dysfunction, decreased sexual interest, depression and mood changes. Moreover, in men, testosterone is converted to estradiol, the primary estrogen in men and women. Estrogens improve bone quality and reduce the risk of hot flashes and skeletal fractures.

Estrogens and androgens perform their physiologic functions by binding to and activating their respective hormone receptors located in various tissues. Once a hormone binds with its receptor, this activates a series of cellular events that results in the hormone specific tissue effects.

Pharmaceuticals that target estrogen or androgen receptors have been used medically for over 50 years. The drugs that have been used to stimulate androgen receptors are either natural or synthetic hormones, known as anabolic/androgenic steroids. Steroids are generally believed to 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 lack of selectivity of testosterone and its conversion to DHT may result in unwanted side effects, such as the potential stimulation of latent into clinical prostate cancer, worsening of BPH, development or worsening of acne, or loss of hair in men. Hair growth, acne and masculinization are also of concern in women who are exposed to exogenous testosterone. To date, no orally available testosterone products have been approved for use in the United States. Those testosterone products that are available must be administered by intramuscular injections or by transdermal patches or gels that may not be convenient for patients and, in some cases, can result in inconsistent blood levels of testosterone.

There are also classes of small molecules that are not steroids that can bind to the same hormone receptors. 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 small molecule with the receptor. A drug that has the ability to either block or stimulate the hormone receptor in this manner is called a selective hormone receptor modulator. A selective hormone receptor modulator may be able to mimic the beneficial, while minimizing the unwanted, effects of natural or synthetic steroid hormones.

A SARM is a small molecule that binds to and selectively modulates androgen receptors, the primary receptor to which testosterone binds. SARMs potentially have beneficial effects in muscle and bone while avoiding testosterone's unwanted effects in the prostate in men or skin and hair in men and women. Although no SARMs

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have been commercialized to date, we believe that SARMs, without the harmful side effects of testosterone or other exogenous anabolic steroid therapies, can potentially be developed to treat a range of medical conditions, including:

- muscle loss conditions of chronic diseases, such as cancer, AIDS, chronic kidney disease, end-stage renal disease, and neurodegenerative disorders;
- muscle loss of acute conditions such as trauma, burns, and rehabilitation;
- muscle loss conditions associated with aging such as frailty and chronic sarcopenia;
- the prevention and/or treatment of osteoporosis;
- disorders of the central nervous system, such as low libido, depression and other mood disorders;
- low testosterone conditions, such as primary and secondary hypogonadism;
- disorders of male reproductive functions, such as infertility and erectile dysfunction;
- androgen receptor positive breast cancer; and
- other conditions, such as anemia.

A selective ER alpha agonist is a nonsteroidal compound with the ability to preferentially bind and activate estrogen receptor alpha as compared to estrogen receptor beta. GTx-758, a selective ER alpha agonist, has previously demonstrated the ability to increase serum concentrations of SHBG, an important serum protein that tightly binds to testosterone and regulates serum concentrations of unbound (free) testosterone. Free testosterone is the functionally active form of the hormone and is capable of passively diffusing into prostate cancer cells or is available to target tissues for androgen action. We believe that GTx-758 may have the ability to treat men with advanced prostate cancer and men with metastatic and nonmetastatic CRPC by lowering serum free testosterone concentrations and lowering the incidences of hot flashes, bone loss or other side effects related to LHRH agonists and antagonists.

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Product Candidates

The following table identifies the development phase and status for each of our clinical product candidates:

Product Candidate/ Proposed Indication	Program	Clinical Development Phase	Status
Enobosarm **3 mg** Prevention and treatment of muscle wasting in patients with advanced NSCLC	SARM	Phase 3	Pursuing a potential MAA submission in the EU for the more narrow indication of advanced NSCLC patients treated with platinum plus taxane chemotherapy and planning to meet again with FDA to discuss a potential Phase 3 program.
Enobosarm **9 mg** Treatment of women with androgen receptor positive and estrogen receptor positive metastatic breast cancer	SARM	Phase 2	Completed enrollment of the Phase 2, open-label clinical trial. Data is expected late in the second quarter of 2014.
GTx-758 Secondary hormonal therapy in men with metastatic or nonmetastatic CRPC	Selective ER alpha agonist	Phase 2	Completed enrollment of the 125 mg cohort of the Phase 2 clinical trial for secondary hormonal therapy in men with metastatic CRPC and are currently enrolling the 250

mg cohort in both metastatic and nonmetastatic CRPC.

SARMs

SARMs are a new class of drugs with the potential to prevent and treat muscle wasting in patients with cancer and other musculoskeletal wasting or muscle loss conditions, including chronic sarcopenia, as well as the potential to be used as a novel hormonal therapy for the treatment of metastatic breast cancer. Enobosarm has been evaluated in fourteen completed or ongoing clinical trials enrolling approximately 1,320 subjects, including in three Phase 2 and two Phase 3 clinical trials.

Enobosarm for the Prevention and Treatment of Muscle Wasting in Patients with Advanced NSCLC

Scientific Overview. Muscle wasting, a cancer related symptom, can begin early in the course of cancer and frequently leads to cancer cachexia, a complex metabolic condition characterized by accelerated loss of skeletal muscle and severe weight loss. Cancer cachexia is usually viewed as an end of life condition in patients with advanced or incurable malignancies. The common clinical symptoms attributed to muscle wasting include decline in physical function and impaired immune function which contribute to increased disability, fatigue, diminished quality of life, and reduced survival.

Although muscle wasting associated with cancer can be partially attributed to poor nutrition, treatment with appetite stimulants and nutritional intervention alone is not effective, likely because they do not address the underlying catabolic processes responsible for muscle wasting. Additionally, patients with severe weight loss, poor performance status, and metastatic cancer that is no longer responding to cancer treatment may be less likely to respond to single therapies designed to increase muscle mass and improve physical function. Because muscle wasting, which often leads to refractory cancer cachexia, has a significant negative impact on the patient and their family, early prevention and treatment of muscle wasting are critical.

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Enobosarm is an oral nonsteroidal SARM which means that it is similar to testosterone in activating androgen receptors in muscle, thereby potentially promoting lean body mass (muscle) and improving physical function, while avoiding other effects which have been observed with testosterone such as hair growth, stimulation of sebaceous glands, the cause of acne, or enhanced growth of the prostate, which may exacerbate BPH or stimulate prostate cancer.

Potential Market. Lung cancer accounts for more deaths than any other cancer in both men and women. Worldwide, there are an estimated 1.5 million new cases and approximately 1.3 million deaths annually. In the United States, there are approximately 225,000 new cases and 160,000 deaths attributed to lung cancer each year. Approximately 85% of all newly diagnosed lung cancers are NSCLC. Approximately 186,000 new cases of NSCLC are diagnosed each year in the top five markets of the European Union and up to 30% of these patients will be treated with a taxane as first line chemotherapy. Up to 50% of NSCLC patients have severe muscle wasting at diagnosis with the majority developing severe wasting throughout the course of their disease. Body functional limitations, such as the inability to walk up or down steps without rest, or the inability to lift 10 pounds, are present in almost 90% of lung cancer survivors.

There are currently no drugs approved for the prevention or treatment of muscle wasting in patients with advanced NSCLC. Supplemental nutritional support alone has little or no benefit in counteracting muscle wasting in cancer patients. Although there are two commercially available steroids, nandrolone and oxandrolone, that are sometimes prescribed off-label for the treatment of weight loss in cancer patients, chronic use of these drugs may result in liver toxicity or other adverse events and has limited their use. Appetite stimulants such as Megace® (megestrol acetate) and dronabinol are also used off-label for weight loss and loss of appetite in patients with cancer. Additionally, there are other companies developing drugs for the treatment of muscle wasting, appetite stimulation and cachexia. These compounds may compete with enobosarm if approved for commercial sale.

Clinical Trials. In July 2007, we initiated a Phase 2b randomized, double blind, placebo-controlled clinical trial evaluating enobosarm for the treatment of muscle wasting in 159 patients diagnosed with NSCLC, colorectal cancer, non-Hodgkin's lymphoma, chronic lymphocytic leukemia, or breast cancer. In October 2008, we announced top line results of this clinical trial. The study met its primary endpoint of absolute change in total lean body mass compared to placebo and the secondary endpoint of physical function, measured by stair climb, after 16 weeks of treatment.

We held End of Phase 2 meetings with the FDA prior to initiating our Phase 3 clinical development of enobosarm for the prevention and treatment of muscle wasting in patients with advanced NSCLC. Based upon data from our Phase 2 clinical trials and with feedback from the FDA, we designed the POWER 1 and POWER 2 Phase 3 clinical trials evaluating enobosarm for this indication. We also met with representatives from two member countries to the EMA, who confirmed that the design of the POWER 1 and POWER 2 clinical trials should be sufficient for the EMA to support registration in Europe. We conducted the POWER trials at sites in the United States, Europe, Russia and South America. Each of the placebo-controlled, double-blind clinical trials was fully enrolled during the fourth quarter of 2012, with approximately 325 patients in each clinical trial. In each of these clinical trials, patients with Stage III or IV NSCLC were randomized to placebo or enobosarm 3 mg at the time they began first line standard platinum doublet chemotherapy. The only difference in the two clinical trials was that patients enrolled in the POWER 1 trial received a platinum plus taxane chemotherapy while patients enrolled in the POWER 2 trial were treated with platinum plus non-taxane chemotherapy. The last patients completed the Phase 3 clinical trials in May 2013. The vital status (survival) of patients participating in the trials is continuing to be periodically monitored in accordance with the clinical trial protocols to assess whether the survival of enobosarm treated patients is adversely affected. This assessment will continue until there are 450 deaths among all patients participating in the two clinical trials. To date, there has been no detrimental effect on survival of enobosarm treated patients as evidenced by the trials.

The POWER trials evaluated the effect of enobosarm versus placebo on the co-primary endpoints of total lean body mass (muscle) assessed by dual x-ray absorptiometry, or DXA, and of physical function assessed by the Stair Climb Test at three months of treatment. Durability of effect of enobosarm was assessed as a secondary endpoint at five months in those patients who demonstrated a lean body mass, or LBM, response at Day 84. In the POWER trials, we failed to meet the primary statistical criterion for the co-primary endpoints of LBM and physical function assessed by responder analysis as agreed upon by the FDA. The responder analysis of the co-primary endpoints showed mixed results (for POWER 1 and POWER 2, p values at Day 84 for LBM were 0.036 and 0.113, respectively, and p values at Day 84 for stair climb power, or SCP, were 0.315 and 0.289, respectively). However, when the same endpoints were assessed using another statistical test, the continuous variable analysis, which was

pre-specified in our statistical analysis plan for the EMA, enobosarm 3 mg demonstrated significant improvement in the primary endpoint of SCP, compared to placebo, in the POWER 1 trial (p value of 0.0147) but did not show statistically significant improvement in SCP in the POWER 2 trial (p value of 0.7923). The continuous variable analysis of LBM was pre-specified as a key secondary endpoint. Enobosarm had a statistically significant improvement in LBM, compared to placebo, in both POWER trials (p values were 0.0002 and 0.0227 at Day 84 for POWER 1 and POWER 2, respectively). Across both clinical trials, enobosarm was generally well tolerated, with the occurrence of serious adverse events and overall incidence of adverse events similar across placebo and treatment groups. In POWER 1, the four most common adverse events reported (in decreasing order of incidence) were nausea, alopecia, anemia and vomiting. In POWER 2, the four most common adverse events reported were anemia, nausea, neutropenia and vomiting. In the safety analysis of survival, there has been no evidence that the survival of enobosarm treated patients had been adversely affected by the drug candidate.

Regulatory Initiatives. Based on agreement with the FDA, we pre-specified the statistical test of a responder analysis of the co-primary endpoints of LBM and SCP at Day 84. A responder analysis categorizes each subject as either a success or failure, where subjects who did not remain on the trial through the Day 84 visit are labeled as a non-responder, regardless of their reason for discontinuation, including death. However for EMA regulatory purposes, we pre-specified in our statistical analysis plan that the primary endpoint would be SCP through Day 84, with LBM through Day 84 being the key secondary endpoint, and the data would be analyzed by a statistical test called continuous variable analysis. A continuous variable analysis utilizes all available data for each subject from their previous clinical visits, so subjects without Day 84 data can still contribute to the analysis via their Day 42 assessment. Since enobosarm 3 mg demonstrated a statistically significant effect versus placebo on the primary endpoint of physical function through Day 84 in the POWER 1 trial, assessed by continuous variable analysis, as pre-specified in our statistical analysis plan for the EMA, we met with representatives from two member countries to the EMA in January of 2014 to review and discuss the results of the POWER trials to determine an appropriate path forward for submitting a MAA in the European Union for enobosarm 3 mg for the prevention and treatment of muscle wasting in patients with advanced NSCLC. We believe data from the POWER 1 trial (platinum plus taxane chemotherapy) is sufficient to support the submission of a MAA for the more narrow indication of the prevention and treatment of muscle wasting in patients with advanced NSCLC treated with platinum plus taxane chemotherapy, and that the confounding results in SCP from the POWER 2 trial can be explained by the effects of the chemotherapy toxicity on the patients receiving platinum plus non-taxane chemotherapy. We plan to initiate seven Phase 1 clinical studies that are typically required for registration purposes and develop a PIP necessary for submission of the MAA. We currently expect to submit the MAA to the EMA for enobosarm 3 mg for the prevention and treatment of muscle wasting in patients with advanced NSCLC treated with platinum plus taxane chemotherapy by the first quarter of 2015, after completion of the Phase 1 studies and acceptance of the PIP by the EMA's Pediatric Committee.

In our meeting with the FDA in February 2014 to review and discuss the results of the POWER clinical trials, we learned that since data from the two POWER trials failed to meet the primary statistical criterion pre-specified for the co-primary endpoints of lean body mass and physical function, the FDA was not willing to accept a NDA for enobosarm 3 mg. However, based on input from the FDA meeting, we believe there is a regulatory path forward for enobosarm 3 mg in the United States, and we plan to meet again with the FDA to discuss a potential Phase 3 clinical program evaluating enobosarm 3 mg for an indication of muscle wasting or cachexia in patients with NSCLC. Any such Phase 3 clinical trial program would be subject to our ability to obtain additional funding and would be required to be successfully completed prior to any NDA submission to the FDA for enobosarm 3 mg.

Enobosarm for the Treatment of AR Positive and ER Positive Metastatic Breast Cancer

Scientific Overview. Clinical assessment of breast cancer includes routine characterization of receptor status including the presence or absence of ER, progesterone receptor, and human epidermal growth factor receptor 2 in the tumor tissue. Receptor status is used to guide treatment decisions. Hormonal manipulation with selective ER modulators, ER antagonists, or aromatase inhibitors is the standard treatment given to patients with tumors that are positive for the ER.

The AR is the most commonly expressed steroid receptor in breast cancer with approximately 75-90% of ER positive and approximately 50% of ER negative breast cancers expressing AR. Prior studies have shown that women with metastatic breast cancer, treated with tamoxifen who then progress, subsequently respond to steroidal androgens. Although steroidal androgens have been used to treat breast cancer, unwanted virilizing side

effects such as body and facial hair growth, acne and deepening of voice, have limited their widespread clinical use. Enobosarm may provide a targeted approach by exploiting the therapeutic benefits of a selective nonsteroidal androgen therapy without concerns of masculinization or conversion to estrogen.

Potential Market. Breast cancer is the most commonly diagnosed cancer in women with one in eight women developing invasive breast cancer during their lifetime. In 2011, 2.9 million women with a history of breast cancer were living in the United States. An estimated 20-30% of women diagnosed with invasive breast cancer will have a recurrence or metastasis. In 2014, an estimated 230,000 new cases of breast cancer will be diagnosed in women in the United States. Approximately 6% of these women will already have metastatic disease at time of diagnosis.

Currently, treatment approaches to postmenopausal hormonally sensitive breast cancer include antiestrogens (tamoxifen, toremifene) and aromatase inhibitors (letrozole, anastrazole, exemestane). Fulvestrant, an injectable ER antagonist, is indicated for the treatment of hormone receptor positive metastatic breast cancer in postmenopausal women after progression on antiestrogen therapy. Enobosarm may represent a novel therapeutic option for the treatment of postmenopausal metastatic breast cancer in women who have progressed on previous hormonal therapy.

Clinical Trial. In the second quarter of 2013, we initiated a Phase 2 open label study evaluating enobosarm 9 mg for the treatment of AR positive and ER positive metastatic breast cancer in women who have previously responded to hormonal therapy for the treatment of their metastatic breast cancer. Nine clinical study sites in the U.S. have fully enrolled the study with 22 postmenopausal women with metastatic breast cancer to assess clinical benefit response after six months of enobosarm 9 mg treatment, which is defined as either those women receiving treatment who have demonstrated a complete response (disappearance of all targeted lesions), a partial response (at least a 30 percent decrease in the sum of the diameters of the targeted lesions) or stable disease (no disease progression from baseline). In February 2014, we reported that enobosarm 9 mg continues to be well tolerated by patients in the study, and that we expect to meet the pre-specified goal of demonstrating, after six months of treatment, at least three clinical benefit responses in at least 14 patients with AR positive and ER positive breast cancer. The study is ongoing and data from all patients in the study is expected late in the second quarter of 2014.

Selective ER Alpha Agonist

GTx-758 ***for the secondary hormonal treatment of CRPC***

Scientific Overview. ADT is the most common treatment for patients who have advanced prostate cancer. ADT reduces testosterone, a primary growth factor for prostate cancer, to castrate levels. ADT is currently accomplished either surgically by removal of the testes or chemically by injection with LHRH agonists or antagonists. These LHRH agents work by shutting off LH secretion by the pituitary gland thereby stopping testosterone production by the testes. The reduction in testosterone by ADT also results in very low estrogen levels in men because estrogen is derived from testosterone. Estrogen deficiency side effects associated with LHRH therapies may include bone loss and fractures, adverse lipid changes, hot flashes, gynecomastia, decreased libido, impaired cognitive function, increase in body fat composition, metabolic syndrome, diabetes and cardiovascular disease. We believe GTx-758 has the potential to reduce free testosterone without also causing certain estrogen deficiency side effects, such as bone loss and hot flashes, which are common with current androgen deprivation therapies for prostate cancer.

Potential Market. We are developing GTx-758 for secondary hormonal therapy in men with metastatic and nonmetastatic CRPC, and, potentially, as a secondary hormonal treatment for advanced prostate cancer used in combination with ADT. We believe that GTx-758 may be effective, in combination with ADT, as a secondary hormonal treatment of advanced prostate cancer by reducing free testosterone, the unbound biologically active form of testosterone, to levels lower than those attainable with ADT alone and potentially reducing the estrogen deficiency side effects caused by the use of ADT.

In the United States alone, we believe there are approximately 80,000 men who have developed resistance to LHRH therapies and therefore have CRPC but who have not received chemotherapy. We believe there are approximately 36,000 men diagnosed each year with metastatic hormone sensitive prostate cancer. Zytiga® is currently the only drug approved for the treatment of metastatic CRPC in patients who have not yet received chemotherapy, although several other drugs are in clinical development for this indication. We believe new hormonal therapies in development, if approved, will be used prior to chemotherapy as physicians and patients look for treatment options capable of delaying cancer progression and possibly prolonging survival prior to

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chemotherapy.

In the United States, there are currently approximately 750,000 men with nonmetastatic hormone sensitive prostate cancer and approximately 60,000 new cases are diagnosed each year. For hormone sensitive advanced prostate cancer, ADT is the most common treatment. There are no approved androgen deprivation therapies designed to significantly minimize estrogen deficiency side effects, including bone loss, fractures, insulin resistance and hot flashes. For many men on ADT, physicians are currently prescribing certain drugs, some of them on an off-label basis, to help ameliorate some of the specific estrogen deficiency related side effects of ADT. These drugs include the use of estrogen patches and compounds, as well as, off-label use of bisphosphonates for osteoporosis and Megace® (megestrol acetate) for hot flashes.

Our ability to develop GTx-758 as an effective secondary hormonal therapy for men with metastatic and nonmetastatic CRPC or potentially, as a secondary hormonal treatment for advanced prostate cancer used in combination with ADT, is dependent on our ability to find an appropriate dose that is both effective and safe for these patient populations.

Clinical Trials. In 2009, we evaluated GTx-758 in healthy male volunteers in two Phase 1 clinical trials, including a ten day multiple ascending dose study in 61 subjects where GTx-758 demonstrated the ability to increase serum SHBG and to reduce serum total and free testosterone. In September 2010, we announced that in a Phase 2, open label, pharmacokinetic and pharmacodynamic clinical trial in young healthy male volunteers, GTx-758 suppressed serum total testosterone to castrate levels (levels of serum total testosterone less than 50ng/dL), increased serum SHBG, and reduced serum free testosterone, the form of testosterone which is available to prostate cancer cells for growth. The percentage of treatment compliant subjects receiving 1500 mg of GTx-758 who achieved medical castration was comparable to rates of castration observed with LHRH agonists or antagonists therapies. In May 2011, we completed a Phase 1 clinical trial of GTx-758 using a tablet formulation in older healthy male volunteers. In this trial, reductions in testosterone to medical castration levels, increases in SHBG and decreases in free testosterone were observed in GTx-758 doses given orally each day.

We designed two Phase 2 clinical trials to identify an appropriate dose of GTx-758 to achieve and maintain medical castration (less than 50ng/dL) from Day 28 to Day 364 in men with advanced prostate cancer. In June 2011, we initiated the Phase 2 maintenance dose finding clinical trial evaluating GTx-758 1000 mg and 2000 mg once-a-day doses compared to Lupron Depot® (leuprolide acetate for depot suspension) in 164 men with advanced prostate cancer. We also initiated the Phase 2 loading dose finding clinical trial evaluating 1000 mg and 1500 mg doses twice-a-day to medically castrate men by Day 28 in 104 men with advanced prostate cancer. After Day 28, castrate patients were to continue treatment on one of two once-a-day doses of GTx-758, 2000 mg or 1000 mg, until Day 360. We were also conducting a second line hormonal therapy Phase 2 clinical trial evaluating GTx-758 2000 mg once-a-day dose in 25 men with CRPC. The objective of this trial was to determine the ability of GTx-758 to reduce serum PSA and the duration of this PSA reduction in men with CRPC who are currently receiving ADT. On February 21, 2012, we announced that the FDA had placed a full clinical hold on our IND application for GTx-758, effective February 17, 2012, causing us to stop all three of these clinical trials. The full clinical hold followed our reports to the FDA of VTEs (blood clots) in subjects treated with GTx-758 at the doses being studied in the trials (1000 mg and higher per day). There were two deaths in subjects treated with GTx-758 and two deaths in subjects treated with Lupron Depot®. As a result of the full clinical hold, we suspended further enrollment into these three trials and notified clinical sites to discontinue treatment of subjects with GTx-758.

In May 2012, we announced that the FDA had removed its full clinical hold on our IND for GTx-758. Based upon feedback from the FDA in connection with the removal of the full clinical hold, in the third quarter of 2012 we initiated a Phase 2 clinical trial to evaluate the safety and efficacy of three lower doses of GTx-758 as secondary hormonal therapy in men with metastatic CRPC.

GTx is currently conducting a Phase 2 open-label clinical trial in men who have developed metastatic or nonmetastatic CRPC while on ADT. GTx-758 has previously demonstrated the ability to increase the production of a protein called SHBG that binds testosterone and thereby reduces free testosterone. By reducing free testosterone, we believe serum PSA will be reduced in men with CRPC. The primary endpoint of the current Phase 2 open-label clinical trial is the proportion of subjects with a $\geq$ 50% decline from baseline in serum PSA by Day 90. Other key endpoints include serum SHBG and total and free testosterone levels, as well as prostate cancer progression, in the study subjects. In addition, the clinical study will evaluate the ability of GTx-758 to treat certain estrogen deficiency side effects associated with currently available ADT agents such as hot flashes and bone loss. The Phase 2 clinical trial will allow us to assess the safety and tolerability of GTx-758 in these subjects, including the incidence of VTEs. The original trial design provided for 75 total subjects to be enrolled in three sequential dosing arms, with

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the first 25 subjects in the study being enrolled in the GTx-758 125 mg dosing arm. Assuming that an acceptable incidence of VTEs was observed when the last subject enrolled in the GTx-758 125 mg dosing arm completed one 30 day cycle of therapy, enrollment of the next 25 subjects would commence in the GTx-758 250 mg dosing arm. Similarly, the GTx-758 500 mg dosing arm was to have commenced enrollment of the final 25 subjects when the last subject enrolled in the 250 mg dose arm completed one 30 day cycle of therapy, assuming an acceptable incidence of VTEs has been observed in both of the lower dosage arms and management had decided to continue testing at the next higher dose. After reviewing data collected from the GTx-758 125 mg dosing arm indicating the ability of the drug to substantially increase SHBG and lower free testosterone without any unexpected side effects occurring, the clinical trial protocol was amended in the third quarter of 2013 to eliminate the 500 mg dosing arm and to increase the number of subjects to be enrolled in the 125 mg and the 250 mg dosing arms to 38 patients per arm. Enrollment in the 125 mg cohort has been completed without any occurrence of VTEs, and after a pre-specified safety review by the independent Data Safety Monitoring Board, we are now enrolling subjects in the 250 mg arm. Depending upon the safety and efficacy data observed in the 125 mg cohort and assuming no safety issues are observed in the first ten metastatic patients enrolled in the 250 mg cohort, enrollment of the 250 mg cohort will be opened to individuals with metastatic or nonmetastatic CRPC. The study is ongoing and data from all patients in the study is expected late in the second half of 2014.

Our Strategy

Our objective is to discover, develop and commercialize small molecules for the treatment of cancer, including treatments for prostate and breast cancer, cancer supportive care, prevention and treatment of cancer-related muscle wasting or cachexia, and other serious medical conditions. Key elements of our strategy to achieve these objectives are to:

Pursue Clinical Development of Enobosarm and Related Regulatory Initiatives. Based upon the results of our POWER 1 clinical trial, as well as supporting data from the POWER 2 clinical trial, we plan to submit a MAA by the first quarter of 2015 to the EMA for enobosarm 3 mg for the more narrow indication of the prevention and treatment of muscle wasting in patients with advanced NSCLC treated with platinum plus taxane chemotherapy. We also plan to meet again later this year with the FDA to discuss a potential Phase 3 program for enobosarm 3 mg to prevent and treat muscle wasting or cachexia in patients with NSCLC. We will continue to evaluate whether to enter into strategic partnerships or collaborations for the development and commercialization of this product candidate or to continue to pursue development efforts in the European Union, and potentially in the United States, on our own. In any event, significant additional funding would be required for us to conduct and complete any additional Phase 3 clinical trials of enobosarm. We are also developing enobosarm for the treatment of AR positive and ER positive metastatic breast cancer in women who have previously responded to hormonal therapy for the treatment of their metastatic breast cancer. Assuming positive results are achieved from our ongoing Phase 2 clinical study, we will meet with members of our enobosarm breast cancer steering committee to seek input on the design and implementation for additional clinical studies for this product candidate, the conduct and completion of which would be subject to our ability to obtain additional funding. Additionally, subject to additional funding, we may develop enobosarm for other indications in patients who could benefit from the treatment of enobosarm.

Pursue Clinical Development of GTx-758. Assuming the receipt of positive data from our ongoing Phase 2 clinical trial to evaluate the safety and efficacy of lower doses of GTx-758 as secondary hormonal therapy in men with metastatic and nonmetastatic CRPC, we plan to initiate additional clinical trials in order to seek marketing authorization for this product candidate.

Licenses and Collaborative Relationships

In addition to our internally-developed and discovered small molecules, 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 potential commercialization of our product candidates. While we currently have no ongoing collaborations for the development and commercialization of our product candidates, our strategy includes selectively partnering or collaborating with leading pharmaceutical and biotechnology companies to assist us in furthering the development and potential commercialization of our product candidates.

In July 2007, we and the University of Tennessee Research Foundation, or UTRF, entered into a consolidated, amended and restated license agreement, or the SARM License Agreement, to consolidate and replace our two previously existing SARM license agreements with UTRF and to modify and expand certain rights and obligations

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of each of the parties under both license agreements. Pursuant to the SARM License Agreement, we were granted exclusive worldwide rights in all existing SARM technologies owned or controlled by UTRF, including enobosarm, and certain improvements thereto, and exclusive rights to certain future SARM technology that may be developed by certain scientists at the University of Tennessee or subsequently licensed to UTRF under certain existing inter-institutional agreements with The Ohio State University. Unless terminated earlier, the term of the SARM License Agreement will continue, on a country-by-country basis, for the longer of 20 years or until the expiration of the last valid claim of any licensed patent in the particular country in which a licensed product is being sold. UTRF may terminate the SARM License Agreement for our uncured breach or upon our bankruptcy.

Under the SARM License Agreement, we paid UTRF a one-time, upfront fee of $290,000 as consideration for entering into the SARM License Agreement. We are also obligated to pay UTRF annual license maintenance fees, low single-digit royalties on net sales of products and mid-single-digit royalties on sublicense revenues. During the year ended December 31, 2007, we paid UTRF a sublicense royalty of approximately $1.9 million as a result of our previous collaboration with Merck & Co., Inc. We also agreed to pay all expenses to file, prosecute and maintain the patents relating to the licensed SARM technologies, and are obligated to use commercially reasonable efforts to develop and commercialize products based on the licensed SARM technologies. In December 2008, we and UTRF amended the SARM License Agreement, or the SARM License Amendment, to, among other things, clarify the treatment of certain payments that we may receive from our current and future sublicensees for purposes of determining sublicense fees payable to UTRF, including the treatment of payments made to us in exchange for the sale of our securities in connection with sublicensing arrangements. In consideration for the execution of the SARM License Amendment, we paid UTRF $494,000.

Manufacturing

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

There are no complicated chemistries or unusual equipment required in the manufacturing process for enobosarm. The active ingredient in enobosarm is manufactured using a five-step synthetic process that uses commercially available starting materials for each step. Enobosarm drug product is manufactured using conventional manufacturing technology without the use of novel excipients. We rely on third-party vendors for drug substance and drug product manufacturing.

There are no complicated chemistries or unusual equipment required in the manufacturing process for GTx-758. The active ingredient in GTx-758 is manufactured using a three-step synthetic process that uses commercially available starting materials for each step. GTx-758 drug product is manufactured using conventional manufacturing technology without the use of novel excipients. We rely on third-party vendors for the manufacture of GTx-758 drug substance and drug product.

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, 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. 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 and/or our collaborators may develop.

Enobosarm for the Prevention and Treatment of Muscle Wasting in Patients with Advanced NSCLC

There are currently no drugs approved for the prevention or treatment of muscle wasting in patients with advanced NSCLC. Although there are two commercially available steroids, nandrolone and oxandrolone, that

are sometimes prescribed off-label for the treatment of weight loss in cancer patients, chronic use of these drugs may result in liver toxicity or other adverse events and has limited their use. For example, oxandrolone has a black box warning for liver toxicity as well as warnings and precautions related to increased risk for prostate cancer in men and virilization in women. Oxandrolone is indicated as an adjunctive therapy to promote weight gain after weight loss following extensive surgery, chronic infections and severe trauma, and in some patients who without pathophysiologic reasons fail to maintain normal weight and to offset the protein catabolism associated with prolonged administration of corticosteroids.

Testosterone products have been used off-label to treat secondary hypogonadism. Owing to their potentially unwanted effects and inconvenient dosing, we believe that testosterone products have had a limited impact on the market for muscle wasting. There are other SARM product candidates in development that may compete with our SARM product candidates if approved, including SARMs in development from Ligand Pharmaceuticals, Inc. and GlaxoSmithKline plc. Pfizer, Inc., Eli Lilly and Company, and Amgen Inc. have previously reported myostatin inhibitors in development that may compete with enobosarm if approved for commercial sale. In addition, Cytokinetics, Inc. is developing a troponin activator with a muscle specific mechanism in Phase 2 studies with a focus on neurological muscle diseases (amyotrophic lateral sclerosis and myasthenia gravis) and Novartis AG is developing a human monoclonal antibody for various muscle indications. Moreover, there are other categories of drugs in development, including ghrelin receptor agonists, growth hormone, secretagogues, inflammatory modulators, and other agents, that may have some muscle activity. Helsinn Group is developing anamorelin, a ghrelin receptor agonist, in Phase 3 clinical trials for the treatment of cancer cachexia in patients with NSCLC. Appetite stimulants such as Megace® (megestrol acetate) and dronabinol are also used off-label for weight loss and loss of appetite in patients with cancer.

GTx-758 for the Secondary Hormonal Treatment of CRPC

There are various products approved or under clinical development to treat men with advanced prostate cancer who have metastatic CRPC which may compete with GTx-758. Dendreon Corporation markets and sells Provenge®, an autologous cellular immunotherapy, for the treatment of asymptomatic or minimally symptomatic metastatic CRPC. Zytiga®, sold by Johnson & Johnson, has been approved for the treatment of metastatic CRPC in both patients who have and have not received prior chemotherapy. Medivation, Inc. has received approval for Xtandi®, an oral androgen receptor antagonist, for the treatment of metastatic CRPC in men previously treated with docetaxel. Medivation continues to develop Xtandi® for men with metastatic CRPC prior to receiving chemotherapy. Johnson & Johnson acquired Aragon Pharmaceuticals, Inc., which developed a second generation anti-androgen (ARN-509) that is currently being evaluated in Phase 2 studies in men with progressive, advanced prostate cancer. Millennium: The Takeda Oncology Company is developing TAK-700 for the treatment of men with metastatic CRPC prior to chemotherapy. GTx-758 is being developed as a treatment for this same patient population prior to the initiation of chemotherapy.

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 enobosarm and our other SARM compounds, we have an exclusive license from UTRF under its issued patents and pending patent applications in the United States, Canada, Australia, Japan, China and other countries in Asia, before the European Patent Office designating Germany, Great Britain, Spain, France, Italy, and other European Union countries, as well as in certain other countries outside those regions, covering the composition of matter of the active pharmaceutical ingredient for pharmaceutical products, pharmaceutical compositions and methods of synthesizing the active pharmaceutical ingredients. We have also exclusively licensed from UTRF issued and pending patent applications in the United States, Canada, Australia, Japan, China and other countries in Asia, before the European Patent Office designating Germany, Great Britain, Spain, France, Italy and other European Union countries, as well as in certain

other countries outside those regions, related to methods for treating muscle wasting disorders, including cancer cachexia, and for treating sarcopenia and increasing muscle performance, muscle size and muscle strength and increasing the strength of or mass of a bone and for treating bone related disorders, including bone frailty and osteoporosis. The patents we licensed from UTRF and issued in the United States for enobosarm expire in 2024. Issued patents for our other SARM compounds in the United States will expire between 2021 and 2029, depending on the specific SARM compound. The patents we licensed from UTRF and issued outside of the United States for enobosarm expire in 2025, and with respect to other SARM

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compounds, expire in 2021, 2023, and 2027, depending on the specific SARM compound. We have pending patent applications for enobosarm and our other SARM compounds that, if issued, would expire in the United States and in countries outside the United States in 2025 and 2027, depending on the specific SARM compound. We have pending patent applications for SARMs in combination with anti-cancer agents that, if issued, would expire in the United States in 2024 and in countries outside the United States in 2028.

We have our own issued and pending patent applications in the United States, Canada, Australia, Europe, Japan, China and other countries in Asia, as well as in certain other countries outside those regions, related to solid forms of enobosarm. Issued patents covering solid forms of enobosarm in the United States will expire in 2028. Pending patent applications in countries outside of the United States will expire in 2028. We have our own pending patent applications in the United States and as an International Application related to methods of treating breast cancer using our SARM compounds. Such patent applications, if issued, would expire in 2033 in the United States and outside of the United States. We also have an allowed United States Application directed to methods of treating eye disorders using our SARM compounds and pending applications in Canada, Australia, Europe, Japan, China and other countries in Asia, as well as in certain other countries outside those regions. The issued patent in the United States related to eye disorders will expire in 2031 and patent applications outside the United States, if issued, would expire in 2031.

We have our own pending patent applications in the United States, Australia, Canada, before the European Patent office designating Germany, Great Britain, Spain, France, Italy and other European Union countries, Japan, and other jurisdictions internationally covering GTx-758 as the composition of matter of the active pharmaceutical ingredient for products developed with this compound and for pharmaceutical compositions and/or methods of treating advanced prostate cancer and treating bone loss, bone fractures, bone mineral density and osteoporosis in male subjects with prostate cancer having undergone androgen deprivation therapy. Issued patents covering composition of matter for GTx-758 in the United States will expire in 2029, and pending patent applications in the United Sates covering GTx-758 method of use will expire in 2029 and 2030. Pending patent applications in countries outside of the United States will expire between 2026 and 2030.

We cannot be certain that any of our patent applications, or those of our licensors, will result in issued patents. In addition, because the patent positions of biopharmaceutical companies are highly uncertain and involve complex legal and factual questions, the patents we own and license, or any further patents we may own or license, may not prevent other companies from developing similar or therapeutically equivalent products. Patents also will not protect our product candidates if competitors devise ways of making or using these product candidates without legally infringing our patents. In recent years, several companies have been extremely aggressive in challenging patents covering pharmaceutical products, and the challenges have often been successful. We cannot be assured that our patents will not be challenged by third parties or that we will be successful in any defense we undertake. Failure to successfully defend a patent challenge could materially and adversely affect our business.

In addition, changes in either patent laws or in interpretations of patent laws in the United States and other countries may materially diminish the value of our intellectual property or narrow the scope of our patent protection. On September 16, 2011, the Leahy-Smith America Invents Act, or the Leahy-Smith Act, was signed into law. The Leahy-Smith Act includes a number of significant changes to United States patent law. These include provisions that affect the way patent applications will be prosecuted and may also affect patent defense and enforcement. Accordingly, the Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have a material adverse effect on our business and financial condition.

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 to us 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.

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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, or NDA. 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 has authority

to 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 IND. The IND becomes effective, if not rejected by the FDA, within 30 days after the FDA receives the IND. The FDA may, either 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 Practices 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 typically considers, 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 1 clinical trials usually involve healthy human subjects. The goal of a Phase I clinical trial is to establish initial data about the safety, tolerability and pharmacokinetic properties of the product candidates in humans. In Phase 2 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 3 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 3 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 current applicable FDA Good

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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 pre-approval 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 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.

Under the Prescription Drug User Fee Act, or PDUFA, submission of a NDA with clinical data requires payment of a fee, with some exceptions. In return, the FDA assigns a goal of six or ten 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. The FDA initially determines whether a 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 fees. 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.

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 a “complete response” letter at the end of the review period. A “complete response” letter will be issued to let a company know that the review period for a drug is complete and that the application is not yet ready for approval. The letter will describe specific deficiencies and, when possible, will outline recommended actions the applicant might take to get the application ready for approval.

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.

The FDA may impose risk evaluation mitigation strategies, or REMS, on a product if the FDA believes there is a reason to monitor the safety of the drug in the marketplace. REMS could add training requirements for healthcare professionals, safety communications efforts, and limits on channels of distribution, among other things. The sponsor would be required to evaluate and monitor the various REMS activities and adjust them if need be. Whether a REMS would be imposed on a product and any resulting financial impact is uncertain at this time.

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

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approved labeling, including the addition of new warnings and contraindications, or even in some instances revocation or withdrawal of the product's approval.

Approval Outside of the United States

In order to market any product outside of the United States, we must comply with numerous and varying regulatory requirements of other countries regarding safety and efficacy and governing, among other things, clinical trials and commercial sales and distribution of our products, which broadly reflect the issues addressed by the FDA above. Approval procedures vary among countries and can involve additional product testing and additional administrative review periods. The time required to obtain approval in other countries might differ from and be longer than that required to obtain FDA approval. Marketing approval in one country does not ensure marketing approval in another, but a failure or delay in obtaining marketing approval in one country may negatively impact the regulatory process in other countries.

As in the United States, the marketing approval process in Europe and in other countries is a lengthy, challenging and inherently uncertain process. If we fail to comply with applicable foreign regulatory requirements, we may be subject to fines, suspension or withdrawal of marketing approvals, product recalls, seizure of products, operating restrictions and criminal prosecution. Generally the development and approval procedures are harmonized throughout the European Union: however, there is limited harmonization in relation to national pricing and reimbursement practices.

Under European Union regulatory systems, a company may not market a medicinal product without marketing authorization. There are three procedures for submitting a MAA in the EU: (1) the mutual recognition procedure (MRP); (2) the decentralized procedure (DCP) and (3) the centralized procedure (CP). The submission strategy for a given product will depend on the nature of the product, the target indication(s), the history of the product, and the marketing plan. The centralized procedure is compulsory for medicinal products which are produced by biotechnology processes, advanced therapy medicinal products and orphan drugs. Besides the products falling under the mandatory scope, the centralized procedure is also open for other innovative products that are new active substances or other medicinal products that constitute a significant therapeutic, scientific or technical innovation.

The centralized procedure leads to approval of the product in all 27 EU member states and in Norway, Iceland and Liechtenstein. Submission of one MAA thus leads to one assessment process and one authorization that allows access to all applicable markets within the entire EU. The process of the centralized procedure is triggered when the applicant sends the letter announcing the intent to submit a MAA (letter of intent). The letter of intent also initiates the assignment of the Rapporteur and Co-Rapporteur, who are the two appointed members of the Committee for Human Medicinal Products, or CHMP, representing two EU member states.

When using the MRP or DCP, the applicant must select which and how many EU member states in which to seek approval. In the case of an MRP, the applicant must initially receive national approval in one EU member state. This will be the so-called reference member state (RMS) for the MRP. Then, the applicant seeks approval for the product in other EU member states, the so-called concerned member states (CMS) in a second step: the mutual recognition process. For the DCP, the applicant will approach all chosen member states at the same time. To do so, the applicant will identify the RMS that will assess the submitted MAA and provide the other selected member states with the conclusions and results of the assessment.

When the application for marketing authorization is made, the competent authority responsible for granting a marketing authorization must verify whether the application complies with the relevant requirements, including compliance with the agreed PIP. Assuming it does, the marketing authorization may be granted and the relevant results are included in the summary of product characteristics (SmPC) for the product, along with a statement indicating compliance with the agreed PIP. It is not necessary for the product actually to be indicated for use in the pediatric population (for example, if the results show that that would not be appropriate).

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

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basis for the approval of abbreviated new drug applications, or ANDAs.

Patent term extension 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 a NDA, plus the time between the submission date of a NDA and the approval of that application. Patent term extensions, however, are subject to a maximum extension of five years, and the patent term extension 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 generally 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 ANDA or a NDA submitted pursuant to section 505(b)(2) of the Federal Food, Drug, and Cosmetics Act, 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 (505(b)(2) NDA), 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 ANDA or 505(b)(2) NDA, 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 a NDA, however, a competitor would be required to conduct its own clinical trials, and any use of the drug for which marketing approval is sought could not violate another NDA holder's patent claims.

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, a 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 ANDA or 505(b)(2) NDA 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 ANDAs or 505(b)(2) NDAs 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 ANDAs for generic versions of their drugs assuming the approval would not violate another NDA holder's patent claims. The ANDA 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 ANDA 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 a 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, a 505(b)(2) NDA, 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.

If a competitor submits an ANDA or 505(b)(2) NDA for a compound or use of any compound covered by another NDA holder's patent claims, the Hatch-Waxman Act requires, in some circumstances, the applicant to notify the patent owner and the holder of the approved NDA of the factual and legal basis of the applicant's opinion that the patent is not valid or will not be infringed. Upon receipt of this notice, the patent owner and the NDA holder have 45 days to bring a patent infringement suit in federal district court and obtain a 30-month stay against the company seeking to reference the NDA. The NDA holder could still file a patent suit after the 45 days, but if they miss the 45-day deadline, 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 ANDA or 505(b)(2) NDA. 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

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may approve the application. Under regulations issued by the FDA, and essentially codified under the Medicare prescription drug legislation, the patent owner and the NDA holder have the opportunity to trigger only a single 30-month stay per ANDA or 505(b)(2) NDA. Once the applicant of the ANDA or 505(b)(2) NDA 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

We currently have no marketed products. 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 products. 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.

If we obtain appropriate approval in the future to market any of our oral drug product candidates, those products could potentially be covered by various government health benefit programs as well as purchased by federal agencies. The participation in such programs or the sale of products to such agencies is subject to regulation. 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.

Medicaid is a joint federal and state program that is administered by the states for low income and disabled beneficiaries. Under the Medicaid Drug Rebate Program, manufacturers are required to pay a rebate for each unit of product reimbursed by the state Medicaid programs. The amount of the rebate for each product is set by law and may be subject to an additional discount if certain pricing increases more than inflation.

Medicare is a federal program that is administered by the federal government that covers individuals age 65 and over as well as those with certain disabilities. Oral drugs may be covered under Medicare Part D. Medicare Part D provides coverage to enrolled Medicare patients for self-administered drugs (*i.e.*, drugs that do not need to be injected or otherwise administered by a physician). Medicare Part D is administered by private prescription drug plans approved by the U.S. government and each drug plan establishes its own Medicare Part D formulary for prescription drug coverage and pricing, which the drug plan may modify from time-to-time. The prescription drug plans negotiate pricing with manufacturers and may condition formulary placement on the availability of manufacturer discounts. Since 2011, manufacturers with marketed brand name drugs have been required to provide a 50% discount on brand name prescription drugs utilized by Medicare Part D beneficiaries when those beneficiaries reach the coverage gap in their drug benefits.

Drug products are subject to discounted pricing when purchased by federal agencies via the Federal Supply Schedule (FSS). FSS participation is required for a drug product to be covered and reimbursed by certain federal agencies and for coverage under Medicaid, Medicare Part B and the PHS pharmaceutical pricing program. FSS pricing is negotiated periodically with the Department of Veterans Affairs. FSS pricing is intended not to exceed the price that a manufacturer charges its most-favored non-federal customer for its product. In addition, prices for drugs purchased by the Veterans Administration, Department of Defense (including drugs purchased by military personnel and dependents through the TRICARE retail pharmacy program), Coast Guard, and PHS are subject to a cap on pricing (known as the "federal ceiling price") and may be subject to an additional discount if pricing increases more than the rate of inflation.

To maintain coverage of drugs under the Medicaid Drug Rebate Program, manufacturers are required to extend discounts to certain purchasers under the Public Health Service (PHS) pharmaceutical pricing program. Purchasers eligible for discounts include hospitals that serve a disproportionate share of financially needy patients, community health clinics and other entities that receive health services grants from the PHS.

The United States and state governments continue to propose and pass legislation designed to reduce the cost of healthcare. In March 2010, the United States Congress enacted the Patient Protection and Affordable Care Act and the Health Care and Education Reconciliation Act which includes changes to the coverage and reimbursement of drug products under government health care programs. Adoption of other new legislation at the federal or state level could further limit reimbursement for pharmaceuticals.

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Although we currently have no products approved for commercial sale, we marketed FARESTON® through September 30, 2012 and the product was covered under various government health benefit programs as well as purchased by federal agencies. We could be subject to liability under federal laws regulating our participation in such programs or the sale of our product to such agencies if we failed to comply with applicable requirements, including reporting prices for our products or offering products for sale at certain prices.

Regulations Pertaining to Sales and Marketing

Although we currently have no products approved for commercial sale, we may be subject to various federal and state laws pertaining to health care "fraud and abuse," including anti-kickback laws and false claims laws for activities related to our previous sales of FARESTON®, which we sold to a third party in 2012, or to future sales of any of our product candidates that may in the future receive regulatory and marketing approval. Anti-kickback laws generally prohibit a prescription drug manufacturer from soliciting, offering, receiving, or paying any remuneration to generate business, including the purchase or prescription of a particular drug. Although the specific provisions of these laws vary, their scope is generally broad and there may not be regulations, guidance or court decisions that apply the laws to particular industry practices. There is therefore a possibility that our practices might be challenged under such anti-kickback laws. False claims laws prohibit anyone from knowingly and willingly presenting, or causing to be presented, any claims for payment for reimbursed drugs or services to third party payors (including Medicare and Medicaid) that are false or fraudulent. Violations of fraud and abuse laws may be punishable by criminal or civil sanctions, including fines and civil monetary penalties, and/or exclusion from federal health care programs (including Medicare and Medicaid).

Laws and regulations have been enacted by the federal government and various states to regulate the sales and marketing practices of pharmaceutical manufacturers with marketed products. The laws and regulations generally limit financial interactions between manufacturers and health care providers and/or require disclosure to the government and public of such interactions. Many of these laws and regulations contain ambiguous requirements or require administrative guidance for implementation. Given the lack of clarity in laws and their implementation, our prior reporting (when we marketed FARESTON®) or any future reporting (if we obtain approval and/or reimbursement from federal healthcare programs for our product candidates) could be subject to the penalty provisions of the pertinent laws and regulations.

Research and Development

Since our inception in 1997, we have been focused on drug discovery and development programs. Research and development expenses include, but are not limited to, our expenses for personnel associated with our research activities, screening and identification of product candidates, formulation and synthesis activities, manufacturing, preclinical studies, toxicology studies, clinical trials, regulatory and medical affairs activities, quality assurance activities and license fees. Our research and development expenses were $32.3 million for the year ended December 31, 2013, $38.9 million for the year ended December 31, 2012, and $31.9 million for the year ended December 31, 2011. As a result of the October 2013 reduction in our workforce, we are no longer conducting drug discovery activities and are focusing our research and development activities on the ongoing clinical development of our current product candidates.

Employees

As of December 31, 2013, we had 32 employees, 8 of whom were M.D.s and/or Ph.D.s. None of our employees are subject to a collective bargaining agreement. We believe that we have good relations with our employees.

Available Information

We file electronically with the U.S. Securities and Exchange Commission, or SEC, our annual reports on Form 10-K, quarterly reports on Form 10-Q, current reports on Form 8-K and amendments to those reports filed or furnished pursuant to Section 13(a) or 15(d) of the Securities Exchange Act of 1934. We make available on our Web site at www.gtxinc.com, free of charge, copies of these reports as soon as reasonably practicable after we electronically file such material with, or furnish it to, the SEC. Further, copies of these reports are located at the

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SEC's Public Reference Room at 100 F Street, NE, Washington, D.C. 20549. Information on the operation of the Public Reference Room can be obtained by calling the SEC at 1-800-SEC-0330. The SEC maintains a Web site that contains reports, proxy statements, and other information regarding our filings at www.sec.gov. The information provided on our Web site is not part of this report, and is therefore not incorporated by reference unless such information is otherwise specifically referenced elsewhere in this report.

Management

The following table sets forth information about our executive officers and other key medical, clinical and regulatory officers as of February 26, 2014.

Name	Age	Position(s)
Executive Officers		
Mitchell S. Steiner, M.D., F.A.C.S.	53	Chief Executive Officer and Vice Chairman of the Board of Directors
Marc S. Hanover	51	President, Chief Operating Officer and Acting Principal Financial Officer
James T. Dalton, Ph.D.	51	Vice President, Chief Scientific Officer
Henry P. Doggrell	65	Vice President, Chief Legal Officer and Secretary
Other Key Medical, Clinical and Regulatory Officers		
Jeffrey G. Hesselberg	55	Vice President, Regulatory Affairs
Mary Ann Johnston, PharmD	42	Vice President, Medical Affairs and Clinical Operations

Executive Officers of the Registrant

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. From 1995 to 2003, Dr. Steiner held numerous academic appointments, including Chairman and Professor of Urology, Director of Urologic Oncology and Research and the Chair of Excellence in Urologic Oncology at the University of Tennessee. Dr. Steiner holds a B.A. in Molecular Biology from Vanderbilt University and an M.D. from the University of Tennessee, and performed his surgery and urologic training at The Johns Hopkins Hospital.

Marc S. Hanover, a co-founder of GTx, has served as our President and Chief Operating Officer since our inception in September 1997, and has served as our acting principal financial officer since December 31, 2013. Mr. Hanover also served as a member of our Board of Directors until August 2011. 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 in Memphis, Tennessee. Mr. Hanover holds a B.S. in Biology from the University of Memphis and a MBA in Finance from the University of Memphis.

James T. Dalton, Ph.D., was appointed Vice President, Chief Scientific Officer on January 1, 2011, and prior to that he served as Vice President, 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 in the Division of Pharmaceutics, College of Pharmacy at The Ohio State University (2000-2007). SARMs were first discovered in Dr. Dalton's research laboratories, and he is co-inventor on all SARM and GTx-758 patents. Dr. Dalton holds a B.S. in Pharmacy from the University of Cincinnati and a Ph.D. in Pharmaceutics and Pharmaceutical Chemistry from The Ohio State University.

Henry P. Doggrell currently serves as our Vice President, Chief Legal Officer and Secretary, after joining GTx in October 2001 as General Counsel and Secretary. 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

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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 JD from Vanderbilt University.

Other Key Medical, Clinical and Regulatory Officers of the Registrant

Jeffrey G. Hesselberg was appointed Vice President, Regulatory Affairs in May 2007. He joined GTx from ICOS Corporation, where from 1996 to May 2007 he served as Manager, Associate Director, and then Director of Regulatory Affairs. Most recently, Mr. Hesselberg worked on the successful development, launch and commercialization of Cialis® (tadalafil) for the treatment of erectile dysfunction. From 1984 to 1996, Mr. Hesselberg worked for Immunex Corporation and the Puget Sound Blood Center. Mr. Hesselberg holds a B.S. in Molecular Biology from the University of Wisconsin—Madison and a MBA from the University of Washington.

Mary Ann Johnston, PharmD, was appointed Vice President, Medical Affairs and Clinical Operations in January 2014. Previously, she served as the Vice President, Medical Affairs since November 2012. Before that, she served as Director, Medical Affairs and Team Leader, Medical Science Liaisons, heading up the field-based medical organization since 2009. Prior to joining GTx, Dr. Johnston was Director, Medical Science Liaisons and Managed Markets at Actelion Pharmaceuticals specializing in pulmonary arterial hypertension. Before joining the pharmaceutical industry, Dr. Johnston practiced as a clinical specialist at the University of Texas Medical Branch in Galveston where she served as an adjunct professor for the University of Houston and

University of Texas schools of pharmacy with a clinical practice focused in cardiology and critical care. Dr. Johnston holds a Doctor of Pharmacy degree from Samford University McWhorter School of Pharmacy and completed a postdoctoral residency at the Department of Veterans Affairs Medical Center in Tuscaloosa, Alabama.

ITEM 1A. RISK FACTORS

We have identified the following additional risks and uncertainties that may have a material adverse effect on our business, financial condition or results of operations. Investors should carefully consider the risks described below before making an investment decision. Our business faces significant risks and the risks described below may not be the only risks we face. Additional risks not presently known to us or that we currently believe are immaterial may also significantly impair our business operations. If any of these risks occur, our business, results of operations or financial condition could suffer, the market price of our common stock could decline and you could lose all or part of your investment in our common stock.

Risks Related to Our Financial Condition and Need for Additional Financing

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

As of December 31, 2013, we had an accumulated deficit of $455.4 million. Our net loss for the year ended December 31, 2013 was $42.1 million. We expect to incur significant operating losses for the foreseeable future as we continue our clinical development activities and potentially seek regulatory approval of our product candidates. These losses, among other things, have had and will continue to have an adverse effect on our stockholders' equity and working capital.

Our current product candidates, enobosarm (GTx-024) and GTx-758 (Capesaris®), will require significant additional clinical development and financial resources in order to obtain necessary regulatory approvals for these product candidates and to develop them into commercially viable products. A substantial portion of our efforts and expenditures have been devoted to enobosarm 3 mg, which was the subject of our POWER 1 and POWER 2 Phase 3 clinical trials for the prevention and treatment of muscle wasting in patients with advanced non-small cell lung cancer, or NSCLC, and we are substantially dependent on the successful development, regulatory approval and commercialization of enobosarm 3 mg. The failure of the POWER trials to meet the primary statistical criterion for the co-primary endpoints agreed upon with the FDA significantly depressed our stock price and harmed our future prospects. While we expect to submit a marketing authorization application, or MAA, to the European Medicines Agency, or EMA, by the first quarter of 2015 seeking marketing approval of enobosarm 3 mg in the European Union for the more narrow indication of the prevention and treatment of muscle wasting in patients with advanced

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NSCLC treated with platinum plus taxane chemotherapy, the EMA must determine that the safety and efficacy data from the POWER 1 trial are sufficient to support approval of the MAA. However, the EMA may determine that the safety and efficacy data from the POWER 1 trial, as supported by data from the POWER 2 trial, are insufficient to support approval of the planned MAA submission and that one or more additional Phase 3 clinical trials of enobosarm 3 mg would be required to be successfully conducted by us in order to support any such approval. If we are required to successfully conduct and complete any additional Phase 3 clinical trials of enobosarm 3 mg in order to support potential approval of enobosarm 3 mg in the European Union, we would be required to obtain substantial additional capital, and given the uncertainties inherent in the clinical development process, there can be no assurances that we would be successful in obtaining the additional funding necessary to support additional Phase 3 clinical trials of enobosarm 3 mg. In such event, we may be required to cease further development of our enobosarm program and forego any return on our investment from our enobosarm program. Based on recent input from the United States Food and Drug Administration, or FDA, we plan to meet again with the FDA later in the year to discuss a potential Phase 3 clinical program for enobosarm 3 mg for the prevention and treatment of muscle wasting or cachexia in patients with NSCLC. There can be no assurance that we will reach agreement with the FDA on any Phase 3 program for enobosarm 3 mg. In addition, we would be required to obtain substantial additional capital in order to conduct any Phase 3 clinical trials of enobosarm 3 mg to support potential approval in the United States and there can be no assurances that we would be successful in obtaining the additional funding necessary to support additional Phase 3 clinical trials of enobosarm 3 mg. In any event, we do not expect to obtain FDA or EMA approval, or any other regulatory approvals, to market any of our product candidates, including enobosarm, for the foreseeable future, and it is possible that none of our product candidates will ever receive any regulatory approvals.

Because of the numerous risks and uncertainties associated with developing and commercializing small molecule drugs, we are unable to predict the extent of any future losses or when we will become profitable, if at all. We have funded our operations primarily through public offerings and private placement of our common stock, as well as payments from our former collaborators. We also previously recognized product revenue from the sale of FARESTON®, the rights to which we sold to a third party in the third quarter of 2012. Currently, we have no ongoing collaborations for the development and commercialization of our product candidates, and as a result of the sale of our rights and certain assets related to FARESTON®, we also currently have no sources of revenue.

If we and/or any potential collaborators are unable to develop and commercialize enobosarm or GTx-758, if development is further delayed or is eliminated, or if sales revenue from enobosarm or GTx-758 upon receiving marketing approval, if ever, is insufficient, we may never become profitable and we will not be successful.

We will need to raise substantial additional capital 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 substantial additional capital to:

- fund our operations and conduct clinical trials, including to conduct and complete any additional Phase 3 clinical trials of enobosarm 3 mg that may be required to support approval of enobosarm 3 mg in the European Union or to pursue regulatory approval of enobosarm 3 mg in the United States;
- continue our research and development;
- seek regulatory approval for our product candidates; and
- commercialize our product candidates, if any such product candidates receive regulatory approval for commercial sale.

As of the date of this Annual Report on Form 10-K, we estimate that our current cash, cash equivalents and short-term investments, together with interest thereon, will be sufficient to meet our projected operating requirements for at least the next twelve months. We have based this estimate on our current business plan and assumptions that may prove to be wrong. We could utilize our available capital resources sooner than we currently expect, and we could need additional funding sooner than currently anticipated. In any event, to complete the development of and seek regulatory approval for enobosarm and GTx-758, we will need to obtain substantial additional funding. Based on our current business plan and assumptions, we will need to obtain substantial additional funding to conduct any additional clinical development of our product candidates beyond the Phase 2 clinical trials we are currently conducting, the Phase 1 clinical trials we plan to conduct to support a potential MAA submission to the EMA for enobosarm 3 mg or any additional Phase 3 clinical trial we may have to conduct to seek approval from any regulatory authorities for enobosarm 3 mg. Our future funding requirements will depend on many factors, including:

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- the scope, rate of progress and cost of our clinical development programs, including our ongoing and any future clinical trials of enobosarm and GTx-758;
- the terms and timing of any potential collaborative, licensing and other strategic arrangements that we may establish;
- the amount and timing of any licensing fees, milestone payments and royalty payments from potential collaborators, if any;
- future clinical trial results;
- the cost and timing of regulatory filings and/or approvals to commercialize our product candidates and any related restrictions, limitations, and/or warnings in the label of an approved product candidate;
- 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, and the cost of defending any other litigation claims.

We do not currently have any commitments for future external funding nor do we currently have any sources of revenue. Until we can generate a sufficient amount of product revenue, which we may never do, we expect to finance future cash needs through public or private equity offerings, debt financings, or collaboration and licensing arrangements, or a combination of the above, as well as through interest income earned on the investment of our cash balances and short-term investments. In October 2013, following our announcement that the POWER trials failed to achieve the results required by the FDA for us to file a new drug application, or NDA, for enobosarm 3 mg, we announced a workforce reduction of approximately 60%. If we are unable to raise additional funds when needed, we may need to further reduce our expenditures, perhaps significantly, to preserve our cash. Cost-cutting measures that we may take in the future may not be sufficient to enable use to meet our cash requirements, and they may negatively affect our business and growth prospects.

To the extent that we raise additional funds through potential collaboration, partnering or other strategic arrangements, it may be necessary to relinquish rights to some of our technologies or product candidates, or grant licenses on terms that are not favorable to us, any of which could result in the stockholders of GTx having little or no continuing interest in our enobosarm and/or GTx-758 programs as stockholders or otherwise. To the extent we raise additional funds by issuing equity securities, our stockholders may experience significant dilution, particularly given our currently depressed stock price, and debt financing, if available, may involve restrictive covenants. For example, we announced in March 2014 that we completed a private placement of common stock and warrants to purchase additional common stock for gross proceeds of approximately $21.3 million, which financing was substantially dilutive, and our stockholders may experience additional, perhaps substantial, dilution should we again raise additional funds by issuing equity securities. Any debt financing or additional equity that we raise may contain terms that are not favorable to us or our stockholders. Our ability to raise additional funds and the terms upon which we are able to raise such funds have been severely harmed by the failure of the two enobosarm 3 mg Phase 3 clinical trials to meet both of the co-primary endpoints agreed upon with the FDA, and may in the future be adversely impacted if we are unable to obtain approval of enobosarm 3 mg in the European Union or if we are required to conduct additional Phase 3 development of enobosarm 3 mg to obtain any such approval. As a result of these and other factors, we cannot be certain that additional funding will be available on acceptable terms, or at all. If adequate funds are not available when we need them, we may be required to delay, reduce the scope of or eliminate one or more of our research or development programs, including our enobosarm and GTx-758 programs, or conduct additional workforce or other expense reductions, any of which could have a material adverse effect on our business and our prospects.

Risks Related to Development of Product Candidates

We are substantially dependent on the success of enobosarm and our failure to obtain regulatory approval of our product candidates from the EMA or FDA may harm our prospects.

A substantial portion of our efforts and expenditures have been devoted to enobosarm 3 mg, which was the subject of our POWER 1 and POWER 2 Phase 3 clinical trials evaluating enobosarm 3 mg for the prevention and treatment of muscle wasting in patients with advanced NSCLC, and we are substantially dependent on the successful development, regulatory approval and commercialization of enobosarm 3 mg. We announced in August 2013 that these two Phase 3 clinical trials failed to meet the co-primary endpoints of lean body mass and physical function that

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were assessed statistically using responder analyses as required by the FDA. The failure of the POWER trials to meet each of the co-primary endpoints significantly depressed our stock price and harmed our future prospects. While we expect to submit a MAA to the EMA for the marketing approval of enobosarm 3 mg in the European Union for the more narrow indication of the prevention and treatment of muscle wasting in patients with advanced NSCLC

treated with platinum plus taxane chemotherapy by the first quarter of 2015, the EMA may determine that the safety and efficacy data from the POWER 1 trial are insufficient to support approval of the planned MAA submission and that one or more additional Phase 3 clinical trials of enobosarm 3 mg would be required to be successfully conducted by us in order to support any such approval, which would require us to obtain substantial additional funding to conduct and complete any additional Phase 3 clinical trials of enobosarm 3 mg. Given the uncertainties inherent in the clinical development process, there can be no assurances that we would be successful in obtaining the additional funding necessary to support additional Phase 3 clinical trials of enobosarm 3 mg. In such event, we may be required to cease further development of our enobosarm program and forego any return on our investment from our enobosarm program and we could be required to cease operations. Based on recent input from the FDA, we plan to meet again with the FDA later in the year to discuss a potential Phase 3 clinical program for enobosarm 3 mg for the prevention and treatment of muscle wasting or cachexia in patients with NSCLC. There can be no assurance that we will reach agreement with the FDA on any Phase 3 program for enobosarm 3 mg. In addition, we would be required to obtain substantial additional capital in order to conduct any Phase 3 clinical trials of enobosarm 3 mg to support potential approval in the United States and there can be no assurances that we would be successful in obtaining the additional funding necessary to support additional Phase 3 clinical trials of enobosarm 3 mg.

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

Significant additional clinical development and financial resources will be required to obtain necessary regulatory approvals for our product candidates and to develop them into commercially viable products. Preclinical and clinical testing is expensive, can take many years to complete 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. Typically, the failure rate for development candidates is high. If a product candidate fails at any stage of development, we will not have the anticipated revenues from that product candidate to fund our operations, and we will not receive any return on our investment in that product candidate. For example, we announced in August 2013 that our POWER 1 and POWER 2 Phase 3 clinical trials evaluating enobosarm 3 mg for the prevention and treatment of muscle wasting in patients with advanced NSCLC failed to meet the co-primary endpoints of lean body mass and physical function that were assessed statistically using responder analyses as agreed upon with the FDA. Although, based on our recent meetings with European regulators, we believe we expect to submit a MAA to the EMA for the more narrow indication of enobosarm 3 mg to prevent and treat muscle wasting in advanced NSCLC patients treated with platinum plus taxane chemotherapy, there is no guarantee that the EMA will approve our MAA, which could result in the requirement that we conduct additional clinical studies or our ceasing further development of enobosarm program. Based on recent input from the United States Food and Drug Administration, or FDA, we plan to meet again with the FDA later in the year to discuss a potential Phase 3 clinical program for enobosarm 3 mg for the prevention and treatment of muscle wasting or cachexia in patients with NSCLC. There can be no assurance that we and the FDA will agree on a Phase 3 clinical program for enobosarm 3 mg for the prevention and treatment of muscle wasting or cachexia in patients with NSCLC. Even if we reach agreement with the FDA to conduct additional Phase 3 clinical trials of enobosarm 3 mg and we believe the results from any trial we conduct to be positive, the efficacy and/or safety results from the trials still may be found to be insufficient to support the submission of a NDA to the FDA or if submitted, the approval of the NDA by the FDA. For example, we received a Complete Response Letter in October 2009 from the FDA regarding our NDA for toremifene 80 mg to reduce fractures in men with prostate cancer on androgen deprivation therapy, or ADT, notifying us that the FDA would not approve the NDA. We have since discontinued our toremifene development programs.

Significant delays in clinical testing could materially impact our product development costs. We do not know whether potential clinical trials will begin on time, or whether ongoing clinical trials will need to be restructured or will be completed on schedule, if at all. We or any potential collaborators may experience numerous unforeseen and/or adverse events during, or as a result of, preclinical testing and the clinical trial process that could delay or prevent our or our potential collaborators' ability to commercialize our product candidates, including:

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- regulators or institutional review boards may not authorize us or any potential collaborators to commence a clinical trial or conduct a clinical trial at a prospective trial site, or we may experience substantial delays in obtaining these authorizations;
- preclinical or clinical trials may produce negative or inconclusive results, which may require us or any potential collaborators to conduct additional preclinical or clinical testing or to abandon projects that we expect to be promising;
- even if preclinical or clinical trial results are positive, the FDA or foreign regulatory authorities could nonetheless require us to conduct unanticipated additional clinical trials;
- registration or enrollment in clinical trials may be slower than we anticipate, resulting in significant delays or study terminations;
- we or any potential collaborators may suspend or terminate 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 or any potential collaborators have significant delays in or termination of clinical trials, our costs could increase and our ability to generate revenue could be impaired, which would materially and adversely impact our business, financial condition and growth prospects.

If we or any potential collaborators observe serious or other adverse events during the time our product candidates are in development or after our products are approved and on the market, we or any potential collaborators may be required to perform lengthy additional clinical trials, may be required to cease further development of such product candidates, may be denied regulatory approval of such products, may be forced to change the labeling of such products or may be required to withdraw any such products from the market, any of which would hinder or preclude our ability to generate revenues.

In three Phase 2 clinical trials of GTx-758, which we discontinued in February 2012, we observed venous thromboembolic events, or blood clots, in subjects treated with GTx-758 at the doses then being studied in these clinical trials (1000 mg and higher per day) and reported those events to the FDA.

There were two deaths in subjects treated with GTx-758 and two deaths in subjects treated with Lupron Depot®. In February 2012, the FDA placed all of our then ongoing clinical studies of GTx-758 on full clinical hold, and we suspended further enrollment into these studies and notified clinical sites to discontinue treatment of subjects with GTx-758. In May 2012, the FDA notified us that it had removed the full clinical hold on GTx-758. In the third quarter of 2012, we initiated a Phase 2 clinical trial to evaluate GTx-758, at doses lower than those which were previously being tested in our discontinued Phase 2 clinical trials, as secondary hormonal therapy in men with metastatic castration resistant prostate cancer, or CRPC. Although our current Phase 2 clinical trial is evaluating GTx-758 at doses lower than those which were previously being tested in our discontinued Phase 2 clinical trials, we cannot be confident that we will not observe an unacceptable incidence of venous thromboembolic events or other adverse events in the current Phase 2 clinical trial. Our ability to develop GTx-758 as an effective secondary hormonal therapy for men with metastatic CRPC or, potentially, as a secondary hormonal treatment for advanced prostate cancer used in combination with ADT, is dependent on our ability to find an appropriate dose that is both effective and safe for these patient populations. If an unacceptable incidence of venous thromboembolic events or other adverse events are observed in our current Phase 2 clinical trial of GTx-758, we may be required to abandon our development of GTx-758, in which case, we would not receive any return on our investment in that product candidate.

In our Phase 2 clinical trials for enobosarm for the treatment of muscle wasting in patients with cancer and healthy older males and postmenopausal females, we observed mild elevations of hepatic enzymes, which in certain circumstances may lead to liver failure, in a few patients in both the placebo and enobosarm treated groups. Reductions in high-density lipoproteins have also been observed in subjects treated with enobosarm. Lower levels of high-density lipoproteins could lead to increased risk of adverse cardiovascular events.

If the incidence of serious or other adverse events related to our product candidates increases in number or severity, if a regulatory authority believes that these or other events constitute an adverse effect caused by the drug, or if other effects are identified during clinical trials that we or any potential collaborators may conduct in the future or after any of our product candidates are approved and marketed:

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- we or any potential collaborators may be required to conduct additional preclinical or clinical trials, make changes in the labeling of any such approved products, reformulate any such products, or implement changes to or obtain new approvals of our contractors' manufacturing facilities;
- regulatory authorities may be unwilling to approve our product candidates or may withdraw approval of our products;
- we may experience a significant drop in the sales of the affected products;
- our reputation in the marketplace may suffer; and
- we may become the target of lawsuits, including class action suits.

Any of these events could prevent approval or harm sales of the affected product candidates or products, or could substantially increase the costs and expenses of commercializing and marketing any such products.

Risks Related to Our Dependence on Third Parties

If we do not establish collaborations for our product candidates or otherwise raise substantial additional capital, we will likely need to alter, delay or abandon our development and any commercialization plans.

Our strategy includes selectively partnering or collaborating with leading pharmaceutical and biotechnology companies to assist us in furthering development and potential commercialization of our product candidates. We face significant competition in seeking appropriate collaborators, and collaborations are complex and time consuming to negotiate and document. We may not be successful in entering into new collaborations with third parties on acceptable terms, or at all. In addition, we are unable to predict when, if ever, we will enter into any additional collaborative arrangements because of the numerous risks and uncertainties associated with establishing such arrangements. If we are unable to negotiate new collaborations, we may have to curtail the development of a particular product candidate, reduce, delay, or terminate its development or one or more of our other development programs, delay its potential commercialization or reduce the scope of our sales or marketing activities or increase our expenditures and undertake development or commercialization activities at our own expense. For example, we may have to cease further development of our enobosarm program if we are unable to raise sufficient funding for any additional clinical development of enobosarm through a new partnership, collaboration or financing. In this regard, we do not have sufficient funds to conduct any additional clinical development of our product candidates beyond the Phase 2 clinical trials we are currently conducting, the Phase 1 clinical trials we plan to conduct to support a potential MAA submission for enobosarm 3 mg, and any additional Phase 3 clinical trial we may have to conduct to seek approval from any regulatory authority for enobosarm 3 mg is subject to our ability to raise additional funds. There can be no assurances that we will be successful in obtaining additional funding in any event. If we do not have sufficient funds, we will not be able to bring our product candidates to market and generate product revenues.

Any collaborative arrangements that we establish in the future may not be successful or we may otherwise not realize the anticipated benefits from these collaborations. In addition, any future collaboration arrangements may place the development and commercialization of our product candidates outside our control, may require us to relinquish important rights or may otherwise be on terms unfavorable to us.

We have in the past established and intend to continue to establish collaborations with third parties to develop and commercialize some of our current and future product candidates, and these collaborations may not be successful or we may otherwise not realize the anticipated benefits from these collaborations. For example, in March 2011, we and Ipsen Biopharm Limited, or Ipsen, mutually agreed to terminate our collaboration for the development and commercialization of our toremifene-based product candidate, and, as a result, we will not receive any additional milestone payments from Ipsen on account of our collaboration with Ipsen. As of the date of this report, we have no ongoing collaborations for the development and commercialization of our product candidates. We may not be able to locate third-party collaborators to develop and market our product candidates, and we lack the capital and resources necessary to develop our product candidates alone.

Dependence on collaborative arrangements subjects us to a number of risks, including:

- we may not be able to control the amount and timing of resources that our potential collaborators may devote to our product candidates;
- potential collaborations may experience financial difficulties or changes in business focus;

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- 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 the 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;
- under certain circumstances, a collaborator could move forward with a competing product candidate developed either independently or in collaboration with others, including our competitors; and
- collaborative arrangements are often terminated or allowed to expire, which could delay the development and may increase the cost of developing our product candidates.

If third parties do not manufacture our product candidates in sufficient quantities, in the required timeframe, at an acceptable cost, and with appropriate quality control, clinical development and commercialization of our product candidates would be delayed.

We do not currently own or operate manufacturing facilities, and we 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, if any, and our ability to develop product candidates and commercialize any product candidates on a timely and competitive basis.

We rely on third-party vendors for the manufacture of enobosarm drug substance. If the contract manufacturers that we are currently utilizing to meet our supply needs for enobosarm or any future SARM product candidates prove incapable or unwilling to continue to meet our supply needs, we could experience a delay in conducting any additional clinical trials of enobosarm or any future SARM product candidates. In addition, we rely on third-party contractors for the manufacture of GTx-758 drug substance. 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 our suppliers fail to meet our requirements for GTx-758, enobosarm or any future product candidates for any reason, we would be required to obtain alternate suppliers. Any inability to obtain alternate suppliers, including an inability to obtain approval from the FDA of an alternate supplier, would delay or prevent the clinical development and commercialization of these product candidates.

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

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;
- the possible termination or non-renewal of the agreement by the third party, based on its own business priorities, at a time that is costly or inconvenient for us; and
- drug product supplies not meeting the requisite requirements for clinical trial use.

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 and/or our potential collaborators may develop may compete with other product candidates and products for access to manufacturing facilities.

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

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obtain regulatory approval for or successfully 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.

Risks Related to Our Intellectual Property

If we lose our license from the University of Tennessee Research Foundation, or UTRF, we may be unable to continue a substantial part of our business.

We have licensed intellectual property rights and technology from UTRF used in a substantial part of our business. This license agreement, under which we were granted rights to SARM compounds and technologies, including enobosarm, may be terminated by UTRF if we are in breach of our obligations under, or fail to perform any terms of, the agreement and fail to cure that breach. If this agreement is terminated, then we may lose our rights to utilize the SARM technology and intellectual property covered by that agreement to market, distribute and sell licensed products, including enobosarm, which may prevent us from continuing a substantial part of our business and may result in a material and serious adverse effect on our financial condition, results of operations and any prospects for growth.

If some or all of our or our licensors' patents expire or are invalidated or are found to be unenforceable, or if some or all of our patent applications do not result in issued patents or result in patents with narrow, overbroad, or unenforceable claims, or claims that are not supported in regard to written description or enablement by the specification, or if we are prevented from asserting that the claims of an issued patent cover a product of a third party, we may be subject to competition from third parties with products in the same class of products as our product candidates or products with the same active pharmaceutical ingredients as our product candidates, including in those jurisdictions in which we have no patent protection.

Our commercial success will depend in part on obtaining and maintaining patent and trade secret protection for our product candidates, as well as the methods for treating patients in the product indications using these product candidates. We will be able to protect our product candidates and the methods for treating patients in the product indications using these product candidates from unauthorized use by third parties only to the extent that we or our exclusive licensors own or control such valid and enforceable patents or trade secrets.

Our rights to certain patents and patent applications relating to SARM compounds that we have licensed from UTRF are subject to the terms of UTRF's inter-institutional agreements with The Ohio State University, or OSU, and our rights to future related improvements in some instances are subject to UTRF's exercise of exclusive options under its agreements with OSU for such improvements.

Even if our product candidates and the methods for treating patients for prescribed indications using these product candidates are covered by valid and enforceable patents and have claims with sufficient scope, disclosure and support in the specification, the patents will provide protection only for a limited amount of time. Our and our licensors' ability to obtain patents can be highly uncertain and involve complex and in some cases unsettled legal issues and factual questions. Furthermore, different countries have different procedures for obtaining patents, and patents issued in different countries provide different degrees of protection against the use of a patented invention by others. Therefore, if the issuance to us or our licensors, in a given country, of a patent covering an invention is not followed by the issuance, in other countries, of patents covering the same invention, or if any judicial interpretation of the validity, enforceability, or scope of the claims in, or the written description or enablement in, a patent issued in one country is not similar to the interpretation given to the corresponding patent issued in another country, our ability to protect our intellectual property in those countries may be limited. Changes in either patent laws or in interpretations of patent laws in the United States and other countries may materially diminish the value of our intellectual property or narrow the scope of our patent protection.

We may be subject to competition from third parties with products in the same class of products as our product candidates or products with the same active pharmaceutical ingredients as our product candidates in those jurisdictions in which we have no patent protection. Even if patents are issued to us or our licensors regarding our

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product candidates or methods of using them, those patents can be challenged by our competitors who can argue such patents are invalid or unenforceable, lack of utility, lack sufficient written description or enablement, or that the claims of the issued patents should be limited or narrowly construed. Patents also will not protect our product candidates if competitors devise ways of making or using 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 companies to challenge branded drug patents or to create non-infringing versions of a patented product in order to facilitate the approval of abbreviated new drug applications for generic substitutes. These same types of incentives encourage competitors to submit new drug applications that rely on literature and clinical data not prepared for or by the drug sponsor, providing another less burdensome pathway to approval.

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.

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 development and manufacturing 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/or 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 or our licensors, which may later result in issued patents that cover the production, manufacture, synthesis, commercialization, formulation or use of our product candidates. In addition, the production, manufacture, synthesis, commercialization, formulation 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 and/or any potential collaborators may develop unless the patent holder licenses the patent to us, which the patent holder is not required to do;
- be required to pay substantial royalties or other amounts, or grant a cross license to our patents to another patent holder; or
- be required to redesign the formulation of a product candidate so that it does not infringe, which may not be possible or could require substantial funds and time.

Risks Related to Regulatory Approval of Our Product Candidates

If we or any potential collaborators are not able to obtain required regulatory approvals, we or such collaborators 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, other regulatory agencies in the United States and by comparable authorities in other countries, including the EMA. Failure to obtain regulatory approval for a product candidate will prevent us or any potential collaborator from commercializing the product candidate. We have not received regulatory approval to market any of our product candidates in any jurisdiction, and we do not expect to obtain FDA, EMA or any other regulatory approvals to market any of our product candidates for the foreseeable future, if at all. 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 or the EMA 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. Any FDA approval may also impose risk evaluation mitigation strategies, or REMS, on a product if the FDA believes there is a reason to monitor the safety of the drug in the market place. REMS may include requirements for additional training for health care professionals, safety communication efforts and limits on channels of distribution, among other things. The sponsor would be required to evaluate and monitor the various REMS activities and adjust them if need be. The FDA and EMA 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, the EMA and other foreign regulatory authorities have substantial discretion in the approval process and may refuse to accept any application or may decide that our data are insufficient for approval and require additional preclinical, clinical or other studies. For example, in October 2009, we received a Complete Response Letter from the FDA regarding our NDA for toremifene 80 mg to reduce fractures in men with prostate cancer on ADT notifying us that the FDA would not approve our NDA as a result of certain clinical deficiencies identified in the Complete Response Letter. We have since discontinued our toremifene 80 mg development program, as well as our other toremifene-based products and terminated our license and supply agreement with Orion for toremifene products. While we expect to submit a MAA to the EMA for the marketing approval of enobosarm 3 mg in the European Union for the more narrow indication of the prevention and treatment of muscle wasting in patients with advanced NSCLC treated with platinum plus taxane chemotherapy by the first quarter of 2015, the EMA may determine that the safety and efficacy data from the POWER 1 trial are insufficient to support approval of the planned MAA submission and that one or more additional Phase 3 clinical trials of enobosarm 3 mg would be required to be successfully conducted by us in order to support any such approval. Based on recent input from the FDA, we plan to meet again with the FDA later in the year to discuss a potential Phase 3 clinical program for enobosarm 3 mg for the prevention and treatment of muscle wasting or cachexia in patients with NSCLC. There can be no assurance that we will reach agreement with the FDA on any Phase 3 program for enobosarm 3 mg. Additionally, there can be no assurance that the FDA will determine that the data from our ongoing clinical trial or future clinical trials of GTx-758 will be sufficient for approval of this product candidate in any indication. For example, we may observe an unacceptable incidence of adverse events in our ongoing, planned or potential clinical trials of enobosarm or GTx-758, which could require us to abandon the development of the affected product candidate.

In addition, varying interpretations of the data obtained from preclinical and clinical testing could delay, limit, or prevent regulatory approval of a product candidate. Even if we submit an application to the FDA, the EMA and other foreign regulatory authorities for marketing approval of a product candidate, it may not result in any marketing approval.

We do not expect to receive regulatory approval for the commercial sale of any of our product candidates that are in development for the foreseeable future, if at all. The inability to obtain approval from the FDA, the EMA and other foreign regulatory authorities for our product candidates would prevent us or any potential collaborators from commercializing these product candidates in the United States, the European Union, or other countries. See the section entitled "Business — Government Regulation" under Part 1, Item 1 of this Annual Report on Form 10-K for additional information regarding risks associated with marketing approval, as well as risks related to potential post-approval requirements.

Risks Related to Commercialization

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

Based upon our recent meetings with European regulators, we expect to submit a MAA to the EMA by the first quarter of 2015 for the more narrow indication of the prevention and treatment of muscle wasting in patients with advanced NSCLC treated with platinum plus taxane chemotherapy. We anticipate that the commercial prospects for the drug product candidate could be diminished as a result of this more limited product indication. Additionally, any

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products that we and/or any potential collaborators may develop, including enobosarm 3 mg, may not gain market acceptance for its stated indication 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 or receive royalties to the extent we currently anticipate, 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:

- efficacy and safety results in clinical trials;
- the prevalence and severity of any side effects;
- potential advantages over alternative treatments;
- whether the products we commercialize remain a preferred course of treatment;
- the ability to offer our product candidates for sale at competitive prices;
- relative convenience and ease of administration;
- the strength of marketing and distribution support; and
- sufficient third-party coverage or reimbursement.

If we are unable to establish sales and marketing capabilities or establish and maintain 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, marketing and distribution of pharmaceutical products. In the event one of our product candidates is approved, we will need to establish sales and marketing capabilities or establish and maintain agreements with third parties to market and sell our product candidates. We may be unable to build our own sales and marketing capabilities, and there are risks involved with entering into arrangements with third parties to perform these services, which could delay the commercialization of any of our product candidates if approved for commercial sale. 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 and/or any potential collaborators are unable to obtain reimbursement or experience a reduction in reimbursement from third-party payors for products we sell, our revenues and prospects for profitability will suffer.

Sales of products developed by us and/or any potential collaborators are dependent on the availability and extent of reimbursement from third-party payors. Changes in the reimbursement policies of these third-party payors that reduce reimbursements for any products that we and/or any potential collaborators may develop and sell could negatively impact our future operating and financial results.

Medicare coverage and reimbursement of prescription drugs exists under Medicare Part D for oral drug products capable of self-administration by patients. Our oral drug product candidates would likely be covered by Medicare Part D (if covered by Medicare at all). In March 2010, the United States Congress enacted the Patient Protection and Affordable Care Act and the Health Care and Education Reconciliation Act. This health care reform legislation will increase the number of individuals who receive health insurance coverage and will close a gap in drug coverage under Medicare Part D. The legislation, however, also implemented cost containment and other measures that could adversely affect revenues from sales of product candidates, including an increase in drug rebates manufacturers must pay under Medicaid for brand name prescription drugs and extension of these rebates to Medicaid managed care.

Pharmaceutical manufacturers and importers of brand name prescription drugs are assessed a fee based on our proportionate share of sales of brand name prescription drugs to certain government programs, including Medicare and Medicaid, made in the preceding year if such sales exceed a defined threshold. Since 2011, manufacturers have been required to provide a 50% discount on brand name prescription drugs sold to beneficiaries who fall within a gap that exists in the Medicare Part D prescription drug program (commonly known as the “donut hole”).

The health care reform legislation has been subject to political and judicial challenge. In 2012, the Supreme Court considered the constitutionality of certain provisions of the law. The court upheld as constitutional the mandate for individuals to obtain health insurance but held that the provision allowing the federal government to withhold certain Medicaid funds to states that do not expand state Medicaid programs was unconstitutional. The

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impact of the court’s ruling remains uncertain. Political and judicial challenges to the law may continue in the wake of the court’s ruling.

Economic pressure on state budgets may result in states increasingly seeking to achieve budget savings through mechanisms that limit coverage or payment for drugs. State Medicaid programs are increasingly requesting manufacturers to pay supplemental rebates and requiring prior authorization for use of drugs where supplemental rebates are not provided. Private health insurers and managed care plans are likely to continue challenging the prices charged for medical products and services, and many of these third-party payors may 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 and/or any potential collaborators may develop or sell. These cost-control initiatives could decrease the price we might establish for products that we or any potential collaborators may develop or sell, which would result in lower product revenues or royalties payable to us.

Similar cost containment initiatives exist in countries outside of the United States, particularly in the countries of the European Union, where the pricing of prescription pharmaceuticals is subject to governmental control. In these countries, pricing negotiations with governmental authorities can extend well beyond the receipt of regulatory marketing approval for a product and may require us or any potential collaborators 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 or a potential collaborators' commercialization efforts. 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. Recently budgetary pressures in many European Union countries are also causing governments to consider or implement various cost-containment measures, such as price freezes, increased price cuts and rebates. If budget pressures continue, governments may implement additional cost containment measures. Cost-control initiatives could decrease the price we might establish for products that we or any potential collaborators may develop or sell, which would result in lower product revenues or royalties payable to us.

Another development that could affect the pricing of drugs would be if the Secretary of Health and Human Services allowed drug reimportation into the United States. The Medicare Prescription Drug, Improvement and Modernization Act of 2003 gives discretion to the Secretary of Health and Human Services to allow drug reimportation into the United States under some circumstances from foreign countries, including from countries where the drugs are sold at a lower price than in the United States. If the circumstances were met and the Secretary exercised the discretion to allow for the direct reimportation of drugs, it could decrease the price we or any potential collaborators receive for any products that we and/or any potential collaborators may develop, negatively affecting our revenues and prospects for profitability.

Health care reform measures could hinder or prevent our product candidates' commercial success.

Among policy makers and payors in the United States and elsewhere, there is significant interest in promoting health care reform, as evidenced by the enactment in the United States of the Patient Protection and Affordable Care Act and the Health Care and Education Reconciliation Act in 2010. Federal and state legislatures within the United States and foreign governments will likely continue to consider changes to existing health care legislation. These changes adopted by governments may adversely impact our business by lowering the price of health care products in the United States and elsewhere.

We operate in a highly regulated industry and new laws, regulations or judicial decisions, or new interpretations of existing laws, regulations or decisions, related to health care availability, method of delivery or payment for health care products and services, or sales, marketing and pricing practices could negatively impact our business, operations and financial condition.

If product liability lawsuits are brought against us, we may 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 our prior commercial sales of FARESTON® and the testing of our product candidates in human clinical trials, and we 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;

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- 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 marketing approvals.

We have product liability insurance that covers our clinical trials and any 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 than any products that we and/or any potential collaborators may develop, our commercial opportunity will be reduced or eliminated.

We face competition from commercial pharmaceutical and biotechnology enterprises, 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 and/or any potential collaborators may develop. Competition could result in reduced sales and pricing pressure on our product candidates, if approved, which in turn would reduce our ability to generate meaningful revenue and have a negative impact on our results of operations. In addition, significant delays in the development of our product candidates could allow our competitors to bring products to market before us and impair any ability to commercialize our product candidates.

Various products are currently marketed or used off-label for some of the diseases and conditions that we are targeting in our pipeline, and a number of companies are or may be developing new treatments. These product uses, as well as promotional efforts by competitors and/or clinical trial results of competitive products, could significantly diminish any ability to market and sell any products that we and/or any potential collaborators may develop.

With respect to our SARM program, there are other SARM product candidates in development that may compete with enobosarm and any future SARM product candidates, if approved for commercial sale, including SARMs in development from Ligand Pharmaceuticals Inc. and GlaxoSmithKline plc. Pfizer Inc., Eli Lilly and Company and Amgen Inc. have myostatin inhibitors in development that may compete with enobosarm if approved for commercial sale. In addition, Cytokinetics, Inc. is developing a troponin activator with a muscle specific mechanism in Phase 2 studies, with a focus on neurological muscle diseases (amyotrophic lateral sclerosis and myasthenia gravis) and Novartis AG is developing a human monoclonal antibody for various muscle indications. Moreover, there are other categories of drugs in development, including ghrelin receptor agonists, growth hormone, secretagogues, inflammatory modulators and other agents, that may have some muscle activity. Helsinn Group is developing anamorelin, a ghrelin receptor agonist, in Phase 3 clinical trials for treatment of cancer cachexia in patients with NSCLC. Appetite stimulants such as Megace® (megestrol acetate) and dronabinol are used off-label for the treatment of weight loss and the treatment of loss of appetite in patients with cancer.

We are developing GTx-758 for secondary hormonal therapy in men with metastatic and nonmetastatic CRPC, and, potentially, as a secondary hormonal treatment for advanced prostate cancer used in combination with androgen deprivation therapy. There are various products approved or under clinical development to treat men with advanced prostate cancer who have metastatic CRPC which may compete with GTx-758. Dendreon Corporation markets and sells Provenge®, an autologous cellular immunotherapy, for the treatment of asymptomatic or minimally symptomatic metastatic castrate resistant prostate cancer. Medivation, Inc. has received approval for Xtandi®, an oral androgen receptor antagonist, for the treatment of metastatic castration-resistant prostate cancer in men previously treated with docetaxel. Medivation continues to develop Xtandi® for men with metastatic CRPC prior to receiving chemotherapy. Zytiga®, sold by Johnson & Johnson, has been approved for the treatment of metastatic CRPC prior in patients who have received prior chemotherapy and recently received approval for the treatment of metastatic castrate resistant prostate cancer prior to chemotherapy. Johnson & Johnson acquired Aragon Pharmaceuticals, Inc., which developed a second generation anti-androgen (ARN-509) that is currently being evaluated in Phase 2 studies in men with progressive, advanced prostate cancer. Millennium: The Takeda Oncology Company is developing TAK-700 for the treatment of men with metastatic castrate resistant prostate cancer prior to chemotherapy. GTx-758 is being developed as a treatment for this same patient population prior to the initiation of chemotherapy.

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 $22.5 million of insurance covering Dr. Steiner.

In October 2013, we announced a reduction of approximately 60% of our workforce following our announcement that our POWER trials failed to achieve the results required by the FDA to file a NDA for enobosarm 3 mg for the prevention and treatment of muscle wasting in patients with advanced NSCLC. Our former Chief Financial Officer also resigned from GTx, effective December 31, 2013. Primarily as a result of our October 2013 workforce reduction, only 32 employees remained as employees of GTx as of December 31, 2013. Accordingly, we have been and are operating with a shortage of resources and may not be able to effectively conduct our operations with this limited number of employees. In addition, we announced past workforce reductions in each of December 2009 and June 2011, and our history of implementing workforce reductions, along with the potential for future workforce reductions, may negatively affect our ability to retain or attract talented employees.

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

As of December 31, 2013, we had only 32 employees, and we will need to hire experienced personnel to develop and commercialize our product candidates and to otherwise grow our business, and we will need to expand the number of our managerial, operational, financial and other employees to support that growth. Competition exists for qualified personnel in the biotechnology field.

Future growth, if any, 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 develop and 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 biotechnology companies in general have been highly volatile and may continue to be so 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:

- announcements regarding our ability to complete the prerequisites for and submit a MAA to the EMA seeking marketing approval for enobosarm 3 mg for the prevention and treatment of muscle wasting in patients with advanced NSCLC treated with platinum plus taxane chemotherapy;
- announcements regarding our ability to determine, in consultation with the FDA, a feasible pathway forward to seek marketing approval for enobosarm 3 mg for the prevention and treatment of muscle wasting or cachexia in patients with NSCLC;
- our ability to raise additional capital to carry through with our clinical development plans and current and future operations and the terms of any related financing arrangements;
- delays in the initiation, enrollment and/or completion of our ongoing and planned clinical trials of enobosarm and GTx-758, or negative, inconclusive or mixed results reported in any of our ongoing clinical trials of enobosarm and GTx-758;
- reports of unacceptable incidences of adverse events observed in any of our ongoing clinical trials of enobosarm and GTx-758;
- announcements regarding further cost-cutting initiatives or restructurings;
- our ability to enter into new collaborative, licensing or other strategic arrangements with respect to our product candidates;
- the terms and timing of any future collaborative, licensing or other arrangements that we may establish;
- the timing of achievement of, or failure to achieve, our and any potential collaborators' 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 or adverse events during 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, including regulatory actions requiring or leading to restrictions, limitations and/or warnings in the label of an approved product candidate;
- the commercial success of any product approved by the FDA or its foreign counterparts;
- introductions or announcements of technological innovations or new products by us, our potential collaborators, or our competitors, and the timing of these introductions or announcements;
- market conditions for equity investments in general, or the biotechnology or pharmaceutical industries in particular;
- announcements regarding our ability to comply with the minimum listing requirements of The NASDAQ Stock Market LLC;
- regulatory developments in the United States and foreign countries;
- changes in the structure or reimbursement policies of health care payment systems;
- any intellectual property infringement lawsuit involving us;
- actual or anticipated fluctuations in our results of operations;
- changes in financial estimates or recommendations by securities analysts;
- hedging or arbitrage trading activity that may develop regarding our common stock;
- 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. The financial markets continue to face significant uncertainty, resulting in a decline in investor confidence and concerns

about the proper functioning of the securities markets, which decline in general investor confidence has resulted in depressed stock prices for many companies notwithstanding the lack of a fundamental change in their underlying business models or prospects. 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 executive officers, directors and largest stockholders have the ability to control all matters submitted to stockholders for approval.

As of March 10, 2014, our executive officers, directors and holders of 5% or more of our outstanding common stock, including their affiliated or associated entities, held approximately 65.9% of our outstanding common stock, and our executive officers and directors alone, including their affiliated or associated entities, held approximately 39.8% of our outstanding common stock. As a result, these stockholders, acting together, have the ability 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.

If we fail to meet continued listing standards of The NASDAQ Stock Market LLC, our common stock may be delisted. Delisting could adversely affect the liquidity of our common stock and the market price of our common stock could decrease, and our ability to obtain sufficient additional capital to fund our operations and to continue as a going concern would be substantially impaired.

Our common stock is currently listed on The NASDAQ Global Market. The NASDAQ Stock Market LLC, or NASDAQ, has minimum requirements that a company must meet in order to remain listed on The NASDAQ Global Market. These requirements include maintaining a minimum closing bid price of $1.00 per share, and the closing bid price of our common stock on March 7, 2014 was $1.70 share. If the closing bid price of our common stock were to fall below $1.00 per share for 30 consecutive trading days or we do not meet other applicable listing requirements, we would fail to be in compliance with NASDAQ's listing standards. There can be no assurance that we will continue to meet the minimum bid price requirement, or any other NASDAQ continued listing requirement, in the future. If we fail to meet these requirements, including the minimum bid price requirement, NASDAQ may notify us that we have failed to meet the minimum listing requirements and initiate the delisting process. If our common stock is delisted, the liquidity of our common stock would be adversely affected and the market price of our common stock could decrease, and our ability to obtain sufficient additional capital to fund our operations and to continue as a going concern would be substantially impaired.

Our ability to use our net operating loss carryforwards and certain other tax attributes may be limited.

Under Section 382 of the Internal Revenue Code of 1986, as amended, if a corporation undergoes an "ownership change," generally defined as a greater than 50% change (by value) in its equity ownership over a three-year period, the corporation's ability to use its pre-change net operating loss carryforwards and other pre-change tax attributes (such as research tax credits) to offset its post-change taxable income or taxes may be limited. Although we have recently completed a study to determine whether any Section 382 limitations existed through December 31, 2013 and we do not believe that any Section 382 limitations existed at that time, Section 382 of the Internal Revenue Code is an extremely complex provision with respect to which there are many uncertainties and we have not established whether the IRS agrees with our determination. In any event, changes in our stock ownership, some of which are outside of our control, could in the future result in an ownership change and an accompanying Section 382 limitation. If a limitation were to apply, utilization of a portion of our domestic net operating loss and tax credit carryforwards could be limited in future periods and a portion of the carryforwards could expire before being available to reduce future income tax liabilities.

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:

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- 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.

If there are substantial sales of our common stock, the market price of our common stock could drop substantially, even if our business is doing well.

For the 12-month period ended December 31, 2013, the average daily trading volume of our common stock on The NASDAQ Global Market was 696,413 shares. As a result, future sales of a substantial number of shares of our common stock in the public market, or the perception that such sales may occur, could adversely affect the then-prevailing market price of our common stock. As of December 31, 2013, we had 63,185,389 shares of common stock outstanding.

In March 2014, we completed a private placement of 11,976,048 shares of our common stock and warrants to purchase 10,179,642 shares of our common stock. Pursuant to the terms of a registration rights agreement we entered into in connection with the private placement, we agreed to file a registration statement under the Securities Act registering the resale of the 11,976,048 shares of common stock we issued to the investors in the private placement, which include J.R. Hyde, III, our largest stockholder, as well as the 10,179,642 shares of common stock underlying the warrants we issued to those investors. In

addition, if we propose to register any of our securities under the Securities Act, either for our own account or for the account of others, the investors in the private placement are entitled to notice of the registration and are entitled to include, at our expense, their shares of common stock in the registration and any related underwriting, provided, among other conditions, that the underwriters may limit the number of shares to be included in the registration. Moreover, J.R. Hyde, III and certain of his affiliates, have rights under a separate registration rights agreement with us to require us to file resale registration statements covering an additional 7.9 million shares of common stock held in the aggregate or to include these shares in registration statements that we may file for ourselves or other stockholders. If Mr. Hyde or his affiliates or any of our other significant stockholders, including the other investor in our March 2014 private placement, were to sell large blocks of shares in a short period of time, the market price of our common stock could drop substantially.

ITEM 1B. UNRESOLVED STAFF COMMENTS

None.

ITEM 2. PROPERTIES

We sublease approximately 31,000 square feet of office space located at 175 Toyota Plaza, Memphis, Tennessee, under an operating lease which expires on April 30, 2015. Subsequent to the reduction in force implemented in October 2013, we cancelled our sublease of approximately 31,000 square feet of laboratory and office space located at 3 North Dunlap Street, Memphis, Tennessee from the University of Tennessee Research Foundation, effective December 31, 2013.

ITEM 3. LEGAL PROCEEDINGS

We are not currently involved in any material legal proceedings.

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ITEM 4. MINE SAFETY DISCLOSURES

Not applicable.

PART II

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

Market for Registrant's Common Equity

Our common stock began trading on The NASDAQ Global Market under the symbol "GTXI" on February 3, 2004. The following table presents, for the periods indicated, the high and low intraday sales prices per share of our common stock as reported on The NASDAQ Global Market.

	2013		2012	
	High	Low	High	Low
First Quarter	$ 5.41	$ 4.15	$ 6.55	$ 3.15
Second Quarter	7.24	3.85	3.92	2.62
Third Quarter	7.14	1.31	5.35	3.29
Fourth Quarter	2.09	1.41	4.92	3.39

On March 7, 2014, the closing price of our common stock as reported on The NASDAQ Global Market was $1.70 per share and there were approximately 76 holders of record of our common stock.

Performance Graph(1)

The rules of the SEC require that we include in our annual report to stockholders a line-graph presentation comparing cumulative stockholder returns on our common stock with a broad equity market index that includes companies whose equity securities are traded on the NASDAQ and either a published industry or line-of-business standard index or an index of peer companies selected by us. We have elected to use The NASDAQ Composite Index (which tracks the aggregate price performance of equity securities of companies traded on NASDAQ Stock Market) and The NASDAQ Biotechnology Index (consisting of a group of approximately 122 companies in the biotechnology sector) for purposes of the performance comparison that appears below.

The following graph shows the cumulative total stockholder return assuming the investment of $100.00 at the closing prices on December 31, 2008 on The NASDAQ Global Market for: (1) our common stock; (2) The NASDAQ Composite Index and (3) The NASDAQ Biotechnology Index. All values assume reinvestment of the full amounts of all dividends. No dividends have been declared on our common stock. The closing sale price of our common stock on December 31, 2013 as reported on The NASDAQ Global Market was $1.65.

The stockholder return shown on the graph below is not necessarily indicative of future performance, and we do not make or endorse any predictions as to future stockholder returns.

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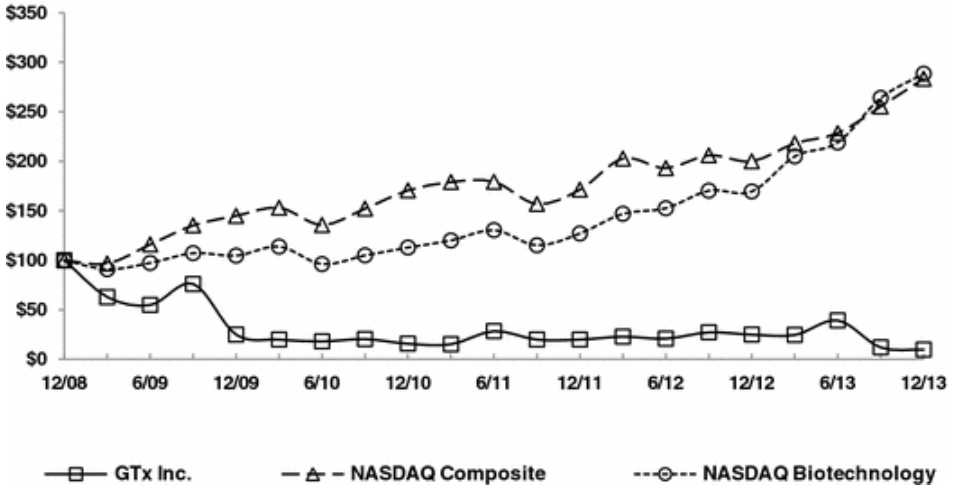
COMPARISON OF 5 YEAR CUMULATIVE TOTAL RETURN*

Among GTx Inc., the NASDAQ Composite Index, and the NASDAQ Biotechnology Index



*$100 invested on 12/31/08 in stock or index, including reinvestment of dividends.
Fiscal year ending December 31.

(1) The material in this section is not "soliciting material," is not deemed filed with the SEC and is not to be incorporated by reference in any filing of GTx, Inc. under the Securities Act of 1933 or the Securities Exchange Act of 1934 whether made before or after the date hereof and irrespective of any general incorporation language in such filing.

Dividend Policy

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.

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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. The following selected financial data have been derived from our audited historical financial statements, certain of which are included elsewhere in the Annual Report on Form 10-K. Historical results are not indicative of the results to be expected in the future.

	Years Ended December 31,				
	2013	2012	2011	2010	2009
	(in thousands, except per share data)				
Statement of Operations Data:					
Revenues:					
Collaboration revenue	$ —	$ —	$ 8,066	$ 56,786	$ 11,441
Expenses:					
Research and development expenses	32,318	38,887	31,938	28,495	32,344
General and administrative expenses	11,281	10,845	12,027	13,194	27,165
Total expenses	43,599	49,732	43,965	41,689	59,509
(Loss) income from operations	(43,599)	(49,732)	(35,899)	15,097	(48,068)
Other (expense) income, net	1,488	(19)	398	1,363	188
(Loss) income from operations before income taxes	(42,111)	(49,751)	(35,501)	16,460	(47,880)
Income tax benefit	—	8,821	886	—	770
Net (loss) income from continuing operations	(42,111)	(40,930)	(34,615)	16,460	(47,110)
Income (loss) from discontinued operations before income taxes	—	22,676	2,207	(1,166)	1,386
Income tax expense	—	(8,821)	(886)	—	(532)
Net (loss) income from discontinued operations	—	13,855	1,321	(1,166)	854
Net (loss) income	$ (42,111)	$ (27,075)	$ (33,294)	$ 15,294	$ (46,256)
Net (loss) income per share — basic and diluted:					
Net (loss) income from continuing operations	$ (0.67)	$ (0.65)	$ (0.60)	$ 0.42	$ (1.29)
Net (loss) income from discontinued operations	—	0.22	0.02	(0.03)	0.02
Net (loss) income per share	$ (0.67)	$ (0.43)	$ (0.58)	$ 0.39	$ (1.27)

	As of December 31,				
	2013	2012	2011	2010	2009
	(in thousands)				
Balance Sheet Data:					
Cash, cash equivalents and short-term investments	$ 14,729	$ 56,089	$ 74,440	$ 58,631	$ 49,044
Working capital	10,604	47,320	71,015	55,149	34,876

Total assets	15,605	57,774	78,656	64,583	56,034
Accumulated deficit	(455,360)	(413,249)	(386,174)	(352,880)	(368,174)
Total stockholders' equity (deficit)	10,684	47,701	71,874	51,727	(8,750)

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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 contains 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 Part I, Item 1A "Risk Factors" and elsewhere in this Annual Report on Form 10-K. See "Special Note Regarding Forward-Looking Statements" in this Annual Report on Form 10-K.

Overview

Business Overview

We are a biopharmaceutical company dedicated to the discovery, development and commercialization of small molecules for the treatment of cancer, including treatments for prostate and breast cancer, cancer supportive care, including prevention and treatment of cancer-related muscle wasting, and other serious medical conditions.

Business Highlights

We are developing selective androgen receptor modulators, or SARMs, a new class of drugs with the potential to prevent and treat muscle wasting in patients with cancer and other musculoskeletal wasting or muscle loss conditions, including chronic sarcopenia (age related muscle loss), as well as the potential to be used as a hormonal therapy for the treatment of metastatic breast cancer. Our lead SARM product candidate, enobosarm (GTx-024), has to date been evaluated in fourteen completed or ongoing clinical trials enrolling approximately 1,320 subjects, including in three Phase 2 and two Phase 3 clinical trials. Enobosarm is the generic name given to the compound by the USAN Council and the World Health Organization and is the first compound to receive the SARM stem in its name, recognizing enobosarm as the first in this new class of compounds.

We announced in August 2013 that the POWER 1 (platinum plus taxane chemotherapy) and POWER 2 (platinum plus non-taxane chemotherapy) (Prevention and treatment Of muscle Wasting in patients with cancER) Phase 3 clinical trials evaluating enobosarm 3 mg for the prevention and treatment of muscle wasting in patients with advanced non-small cell lung cancer, or NSCLC, failed to meet the primary statistical criterion for the co-primary endpoints of lean body mass and physical function that were assessed statistically using responder analyses as pre-specified for the United States Food and Drug Administration, or FDA. However, efficacy data from the studies demonstrated enobosarm's consistent effect on maintaining or improving lean body mass compared to placebo and that maintenance or improvement in lean body mass is potentially associated with longer survival in patients, regardless of treatment. As for safety, enobosarm was generally well tolerated, with the occurrence of serious adverse events similar across the placebo and treated groups.

Enobosarm 3 mg demonstrated a statistically significant effect versus placebo on the primary endpoint of physical function through Day 84 in the POWER 1 clinical trial as assessed by continuous variable analysis, as pre-specified in our statistical analysis plan for the European Medicines Agency, or EMA. Therefore, we met with representatives from two member countries to the EMA in January of 2014 to review and discuss the results of the POWER trials to determine an appropriate path forward for submitting a marketing authorization application, or MAA, in the European Union for enobosarm 3 mg for the prevention and treatment of muscle wasting in patients with advanced NSCLC. Based on input from the two member countries, we believe data from the POWER 1 trial, as well as supporting data from the POWER 2 trial, are sufficient to support the submission of a MAA to the EMA seeking marketing authorization for enobosarm 3 mg in the more narrow indication of the prevention and treatment of muscle wasting in patients with advanced NSCLC treated with platinum plus taxane chemotherapy. We plan to initiate seven Phase 1 clinical studies that are typically required for registration purposes and develop a pediatric investigational plan, or PIP, necessary for submission of the MAA. We currently expect to submit the MAA to the EMA for enobosarm 3 mg for the prevention and treatment of muscle wasting in patients with advanced NSCLC treated with platinum plus taxane chemotherapy by the first quarter of 2015, after completion of the Phase 1 studies and acceptance of the PIP by the EMA's Pediatric Committee.

In our meeting with the FDA in February 2014 to review and discuss the results of the POWER clinical trials, we learned that since data from the two POWER trials failed to meet the primary statistical criterion pre-specified for the co-primary endpoints of lean body mass and physical function, the FDA was not willing to accept a new drug

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application, or NDA, for enobosarm 3 mg. However, based on input from the FDA meeting, we believe there is a regulatory path forward for enobosarm 3 mg in the United States, and we plan to meet again with the FDA to discuss a potential Phase 3 clinical program evaluating enobosarm 3 mg for an indication of muscle wasting or cachexia in patients with NSCLC. Any such Phase 3 clinical program would be subject to our ability to obtain additional funding and would be required to be successfully completed prior to any NDA submission to the FDA for enobosarm 3 mg.

We conducted the POWER 1 and POWER 2 clinical trials of enobosarm at sites in the United States, Europe, Russia and South America. Each of the placebo-controlled, double-blind clinical trials was fully enrolled during the fourth quarter of 2012, with approximately 325 patients in each clinical trial. In each of these clinical trials, patients with Stage III or IV NSCLC were randomized to placebo or enobosarm 3 mg at the time they began first line chemotherapy. The last patients completed the Phase 3 clinical trials in May 2013. The vital status (survival) of patients participating in the trials will continue to be periodically monitored in accordance with the clinical trial protocols and is still ongoing. The trials evaluated as co-primary endpoints the effect of enobosarm versus placebo on total lean body mass (muscle) assessed by dual x-ray absorptiometry, or DXA, and on physical function assessed by the Stair Climb Test at three months. Durability of effect was assessed as a secondary endpoint at five months in those patients who responded at Day 84.

SARMs also have the potential to be used as a novel hormonal therapy for the treatment of metastatic breast cancer. Nonselective steroidal androgens have been used to treat breast cancer; however, the unwanted virilizing side effects have limited their widespread clinical use. We believe that enobosarm, by targeting the androgen receptor, or AR, in estrogen receptor, or ER, positive breast cancer, has the potential to provide clinical benefit to women with metastatic breast cancer by treating their disease while minimizing the unwanted masculinizing side effects associated with steroidal androgens, and unlike androgens, cannot be converted into an estrogen. In the second quarter of 2013, we initiated a Phase 2 open label study evaluating enobosarm 9 mg for the treatment of AR positive and ER positive metastatic breast cancer in women who have previously responded to hormonal therapy for the treatment of their metastatic breast cancer. Nine clinical study sites in the U.S. have fully enrolled the study with 22 postmenopausal women with metastatic breast cancer to assess clinical benefit response after six months of enobosarm 9 mg treatment, which is defined as either those women receiving treatment who have demonstrated a complete response (disappearance of all targeted lesions), a partial response (at least a 30 percent decrease in the sum of the diameters of the targeted lesions) or stable disease (no disease progression from baseline). In February 2014, we reported that enobosarm 9 mg continues to be well tolerated by patients in the study, and that we expect to meet the pre-specified goal of demonstrating, after six months of treatment, at least three clinical benefit responses in at least 14 patients with AR positive and ER positive breast cancer. The study is ongoing and data from all patients in the study is expected late in the second quarter of 2014.

Additionally, we are developing GTx-758 (Capesaris®), an oral nonsteroidal selective estrogen receptor alpha agonist, for secondary hormonal therapy in men with metastatic and nonmetastatic castration resistant prostate cancer, or CRPC, and, potentially, as a secondary hormonal treatment for advanced prostate cancer used in combination with androgen deprivation therapy, or ADT. We believe GTx-758 has the potential to reduce free testosterone without also causing certain estrogen deficiency side effects, such as bone loss and hot flashes, which are common with current androgen deprivation therapies for prostate cancer. We also believe that GTx-758 may be effective, in combination with ADT, as a secondary hormonal treatment of advanced prostate cancer by reducing free testosterone to levels lower than those attainable with ADT alone and potentially reducing the estrogen deficiency side effects caused by the use of ADT.

In May 2012, we announced that the FDA had removed its full clinical hold on our Investigational New Drug, or IND, application for GTx-758. The full clinical hold was placed on our three then ongoing Phase 2 clinical trials evaluating GTx-758 to treat men with advanced prostate cancer in February 2012 and resulted in the discontinuation of these trials. The full clinical hold followed our reports to the FDA of venous thromboembolic events (blood clots), or VTEs, in subjects treated with GTx-758 at the doses being studied in those trials (1000 mg and higher per day). Based upon feedback from the FDA in connection with the removal of the full clinical hold, we initiated in the third quarter of 2012 a Phase 2 clinical trial to evaluate the safety and efficacy of lower doses of GTx-758 as secondary hormonal therapy in men with metastatic CRPC.

GTx is currently conducting a Phase 2 open-label clinical trial in men who have developed metastatic or nonmetastatic CRPC while on ADT. GTx-758 has previously demonstrated the ability to increase the production of a protein called sex hormone binding globulin, or SHBG, that binds testosterone and thereby reduces free testosterone. By reducing free testosterone, we believe serum prostate specific antigen, or PSA, will be reduced in men with CRPC. The primary endpoint of the current Phase 2 clinical trial is the proportion of subjects with a ≥

50% decline from baseline in serum PSA by Day 90. Other key endpoints include serum SHBG and total and free testosterone levels, as well as prostate cancer progression, in the study subjects. In addition, the clinical study will evaluate the ability of GTx-758 to treat certain estrogen deficiency side effects associated with luteinizing hormone releasing hormone agonists such as hot flashes and bone loss. The Phase 2 clinical trial allows us to assess the safety and tolerability of GTx-758 in these subjects, including the incidence of VTEs. The original trial design provided for 75 total subjects to be enrolled in three sequential dosing arms, with the first 25 subjects in the study being enrolled in the GTx-758 125 mg dosing arm. Assuming that an acceptable incidence of VTEs was observed when the last subject enrolled in the GTx-758 125 mg dosing arm completed one 30 day cycle of therapy, enrollment of the next 25 subjects would commence in the GTx-758 250 mg dosing arm. Similarly, the GTx-758 500 mg dosing arm was to have commenced enrollment of the final 25 subjects when the last subject enrolled in the 250 mg dose arm completed one 30 day cycle of therapy, assuming an acceptable incidence of VTEs has been observed in both of the lower dosage arms and management had decided to continue testing at the next higher dose. After reviewing data collected from the GTx-758 125 mg dosing arm indicating the ability of the drug to substantially increase SHBG and lower free testosterone without any unexpected side effects occurring, the clinical trial protocol was amended in the third quarter of 2013 to eliminate the 500 mg dosing arm and to increase the number of subjects to be enrolled in the 125 mg and the 250 mg dosing arms to 38 patients per arm. Enrollment in the 125 mg cohort has been completed without any occurrence of VTEs, and after a pre-specified safety review by the independent Data Safety Monitoring Board, we are now enrolling subjects in the 250 mg arm. Depending upon the safety and efficacy data observed in the 125 mg cohort and assuming no safety issues are observed in the first ten metastatic patients enrolled in the 250 mg cohort, enrollment of the 250 mg cohort will be opened to individuals with metastatic or nonmetastatic CRPC. The study is ongoing and data from all patients in the study is expected late in the second half of 2014.

Financial Highlights

Our net loss for the year ended December 31, 2013 was $42.1 million. We expect to incur significant operating losses for the foreseeable future as we continue our clinical development activities and potentially seek regulatory approval of our product candidates. We have funded our operations primarily through the sale of equity securities, collaboration and license agreements, and prior to September 2012, product revenue from sales of FARESTON®, the rights to which we sold to a third party in the third quarter of 2012. We currently have no ongoing collaborations for the development and commercialization of our product candidates and no source of revenue, nor do we expect to generate revenue for the foreseeable future. We do not expect to obtain FDA or EMA approval, or any other regulatory approvals, to market any of our product candidates, including enobosarm, for the foreseeable future, and it is possible that none of our product candidates will ever receive any regulatory approvals.

At December 31, 2013, we had cash, cash equivalents and short-term investments of $14.7 million compared to $56.1 million at December 31, 2012. On March 6, 2014, we completed a private placement of units consisting of 11,976,048 shares of common stock and warrants to purchase 10,179,642 shares of our common stock for gross proceeds of approximately $21.3 million. The warrants, which have a one year term, have a per share exercise price of $1.67 that is payable only in cash. If exercised in full, the warrants could result in additional gross proceeds of approximately $17.0 million. As of the date of this Annual Report on Form 10-K, we estimate that our current cash, cash equivalents and short-term investments, together with interest thereon, will be sufficient to meet our projected operating requirements for at least the next twelve months. We have based this estimate on our current business plan and assumptions that may prove to be wrong. We could utilize our available capital resources sooner than we currently expect, and we could need additional funding sooner than currently anticipated. In any event, to complete the development and seek regulatory approval for any of our product candidates, we will need to obtain substantial additional funding. Based on our current business plan and assumptions, we will need to obtain substantial additional funding to conduct any

additional clinical development of our product candidates beyond the Phase 2 clinical trials we are currently conducting and the Phase 1 clinical trials we plan to conduct to support a potential MAA submission to the EMA for enobosarm 3 mg.

While we have been able to fund our operations to date, we do not currently have any commitments for future external funding and we would need to obtain substantial additional funding to conduct and complete any additional Phase 3 clinical trials of enobosarm 3 mg. Until we can generate a sufficient amount of product revenue, which we may never do, we expect to finance future cash needs through public or private equity offerings, debt financings or collaboration and licensing arrangements, or a combination of the above, as well as through interest income earned on the investment of our cash balances and short-term investments. In October 2013, following our receipt of the negative results in our POWER 1 and POWER 2 Phase 3 clinical trials evaluating enobosarm 3 mg, we announced and implemented a workforce reduction of approximately 60%. If we are unable to raise additional funds when needed, we may need to further reduce our expenditures, perhaps significantly, to preserve our cash. Cost-cutting measures that we may take

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in the future may not be sufficient to enable us to meet our cash requirements, and they may negatively affect our business and growth prospects.

Research and Development

Since our inception in 1997, we have been focused on drug discovery and development programs. Research and development expenses include, but are not limited to, our expenses for personnel and supplies associated with our research activities, screening and identification of product candidates, formulation and synthesis activities, manufacturing, preclinical studies, toxicology studies, clinical trials, regulatory and medical affairs activities, quality assurance activities and license fees. As a result of the October 2013 reduction in our workforce, we are no longer conducting drug discovery activities and we are focusing our research and development activities on the ongoing clinical development of our current product candidates.

We expect that our research and development expenses for fiscal year 2014 will decrease as compared to fiscal year 2013 due to the completion of the POWER 1 and POWER 2 clinical trials in 2013.

There is a substantial risk that any development program may not produce revenue. Moreover, because of uncertainties inherent in drug development, including those factors described in Part I, Item 1A “Risk Factors” of this Annual Report on Form 10-K, we may not be able to successfully develop and commercialize any of our product candidates.

The successful development and commercialization 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 and commercialization 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 and commercializing drugs, including the uncertainty of:

- the scope, rate of progress and cost of our clinical development programs, including our ongoing and any future clinical trials of enobosarm and GTx-758;
- the terms and timing of any potential collaborative, licensing and other strategic arrangements that we may establish;
- the amount and timing of any licensing fees, milestone payments and royalty payments from potential collaborators, if any;
- future clinical trial results;
- the cost and timing of regulatory filings and/or approvals to commercialize our product candidates and any related restrictions, limitations, and/or warnings in the label of an approved product candidate;
- 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, and the cost of defending any other litigation claims.

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 development and commercialization efforts on schedule, or at all, and some consequences of failing to do so, are set forth under Part I, Item 1A “Risk Factors” of this Annual Report on Form 10-K.

General and Administrative Expenses

Our general and administrative expenses consist primarily of salaries and other related costs for personnel serving executive, finance, legal, human resources, information technology, and investor relations functions. General and administrative expenses also include facility costs, insurance costs, and professional fees for legal, accounting, and public relation services. We expect our general and administrative expenses for fiscal year 2014 to decrease from fiscal year 2013 due to the reduction in force that was implemented in October 2013.

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Critical Accounting Policies and Significant Judgments and Estimates

Our management’s discussion and analysis of our financial condition and results of operations is based on our 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. On an ongoing basis, we evaluate our estimates and judgments related to revenue recognition, income taxes, intangible assets, long-term service contracts, share-based compensation, 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 on Form 10-K, we believe that the following accounting policies are most critical to aid you in fully understanding and evaluating our reported financial results.

Research and Development Expenses

Research and development expenses include, but are not limited to, our expenses for personnel, supplies, and facilities associated with research activities, screening and identification of product candidates, formulation and synthesis activities, manufacturing, preclinical studies, toxicology studies, clinical trials, regulatory and medical affairs activities, quality assurance activities and license fees. We expense these costs in the period in which they are incurred. We estimate our liabilities for research and development expenses in order to match the recognition of expenses to the period in which the actual services are received. As such, accrued liabilities related to third party research and development activities are recognized based upon our estimate of services received and degree of completion of the services in accordance with the specific third party contract.

Share-Based Compensation

We have stock option and equity incentive plans that provide for the purchase of our common stock by certain of our employees and non-employee directors. We measure compensation expense for our share-based payments based on the fair value of the awards on the grant date and recognize the expense over the period during which an employee or non-employee director is required to provide service in exchange for the award.

The determination of the fair value of stock options on the date of grant include the expected life of the award, the expected stock price volatility over the expected life of the awards, and risk-free interest rate. We estimate the expected life of options by calculating the average of the vesting term and contractual term of the options. We estimate the expected stock price volatility based on the historical volatility of our common stock. The risk-free interest rate is determined using U.S. Treasury rates where the term is consistent with the expected life of the stock options. Expected dividend yield is not considered as we have not made any dividend payments and have no plans of doing so in the foreseeable future. The amount of share-based compensation expense recognized is reduced ratably over the vesting period by an estimate of the percentage of options granted that are expected to be forfeited or canceled before becoming fully vested. This estimate is adjusted periodically based on the extent to which actual forfeitures differ, or are expected to differ, from the previous estimate.

Share-based compensation also includes, beginning October 2013, restricted stock units, or RSUs, granted to employees under our 2013 equity incentive plan. We estimate the fair value of RSUs using the closing price of our stock on the grant date. The fair value of RSUs is amortized on a straight-line basis over the requisite service period of the awards.

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The following table summarizes share-based compensation expense included within the statements of operations for the years ended December 31, 2013, 2012 and 2011:

	Years ended December 31,		
	2013	2012	2011
	(in thousands)		
Research and development expenses	$ 1,875	$ 1,046	$ 1,972
General and administrative expenses	1,993	1,771	2,432
Total share-based compensation	$ 3,868	$ 2,817	$ 4,404

Share-based compensation expense recorded in the statement of operations as general and administrative expense for the years ended December 31, 2013, 2012 and 2011 included share-based compensation expense related to deferred compensation arrangements for our non-employee directors of $135,000, $169,000 and $178,000, respectively. At December 31, 2013, the total compensation cost related to non-vested stock options not yet recognized was approximately $4.4 million with a weighted average expense recognition period of 1.97 years. At December 31, 2013, the total compensation cost related to non-vested RSUs not yet recognized was approximately $1.4 million with a weighted average expense recognition period of five months.

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. Accordingly, at December 31, 2013 and 2012, net of the valuation allowance, the net deferred tax assets were reduced to zero.

We have recognized the tax effect of discontinued operations in the statements of operations in accordance with the intra-period accounting rules for the year ended December 31, 2012 and 2011. An offsetting tax benefit is recorded in continuing operations in each year in which tax expense was recognized for discontinued operations.

Impairment of Long-Lived Assets

We review long-lived assets for impairment whenever events or changes in facts and circumstances are present, both internally and externally, that may indicate impairment of long-lived assets. An impairment loss is recognized when estimated future cash flows are less than the carrying amount. The cash flow estimates are based on management’s best estimates, using appropriate and customary assumptions and projections at the time.

In 2011, after discontinuing our toremifene 80 mg development program, we recorded an impairment charge of $1.6 million. The impaired intangible asset consisted of capitalized license fees paid to Orion Corporation related to our toremifene 80 mg program. The impairment charge was included in research and development expenses in the statement of operations for the year ended December 31, 2011.

Discontinued Operations

Effective September 30, 2012, we completed the sale of FARESTON® for a total cash purchase price of $21.7 million, including payment for purchased inventory. We have accounted for FARESTON® as a discontinued operation. As a result, revenue, cost of goods sold, and operating expenses relating to FARESTON® have been excluded from their respective captions in the statements of operations and have been included in discontinued operations for the years ended December 31, 2012 and 2011.

Revenue Recognition

Our revenues for the years ended December 31, 2012 and 2011 consisted of product sales of FARESTON®, which is included in income from discontinued operations before income taxes, and in 2011, also consisted of revenues derived from our former collaboration and license agreements.

Revenue from product sales of FARESTON® was recognized less deductions for estimated sales discounts and sales returns. Revenue from product sales was recognized when persuasive evidence of an arrangement existed, title passed, the price was fixed or determinable, and collectability was reasonably assured. We accounted for rebates to

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certain governmental agencies as a reduction of product sales. We allow customers to return product within a specified time period prior to and subsequent to the product's labeled expiration date. Although we sold our rights and certain assets related to FARESTON® effective September 30, 2012, we retain the liability for future product returns relating to sales of FARESTON® by us prior to September 30, 2012. Therefore, we estimate an accrual for product returns based on factors which include historical product returns and estimated product in the distribution channel which is expected to exceed its expiration date. At December 31, 2013 and December 31, 2012, our accrual for product returns, was $918,000 and $1.2 million, respectively.

Collaboration revenue for the year ended December 31, 2011 consisted of non-refundable upfront payments and license fees associated with our former collaboration and license agreement with Ipsen Biopharm Limited, or Ipsen, and was based on the performance requirements of the agreement. We analyzed the agreement, which included multiple element arrangements, to determine whether the deliverables under the agreement, including license and performance obligations such as joint steering committee participation and research and development activities, could have been separated or whether all of the deliverables must have been accounted for as a single unit of accounting.

Results of Operations

Collaboration Revenue

There was no collaboration revenue recognized for the years ended December 31, 2013 and 2012. Collaboration revenue was $8.1 million for the year ended December 31, 2011, which resulted from recognition of all remaining unamortized deferred revenue due to the termination of our former license and collaboration agreement with Ipsen in March 2011.

Research and Development Expenses

The following table identifies the research and development expenses for each of our clinical product candidates, as well as research and development expenses 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.

Proposed Candidate / Proposed Indication	Program	Years Ended December 31, 2013	2012	2011
			(in thousands)	
Enobosarm 3 mg Prevention and treatment of muscle wasting in patients with advanced non-small cell lung cancer	**SARM**	$ 18,541	$ 24,320	$ 10,474
Enobosarm 9 mg Treatment of women with AR positive and ER positive metastatic breast cancer	**SARM**	1,980	—	—
GTx-758 Secondary hormonal therapy in men with metastatic and nonmetastatic CRPC	**Selective ER alpha agonist**	5,492	7,458	12,052
Other research and development		6,305	7,109	9,412
Total research and development expenses		$ 32,318	$ 38,887	$ 31,938

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Research and development expenses decreased 17% to $32.3 million for the year ended December 31, 2013 from $38.9 million for the year ended December 31, 2012. The $5.8 million decrease in enobosarm 3 mg research and development expenses was due primarily to a decrease in expenses as the last patients completed the POWER 1 and POWER 2 Phase 3 clinical trials for enobosarm 3 mg in May 2013. Research and development expenses for enobosarm 9 mg increased as we initiated in the second quarter of 2013 a Phase 2 clinical trial evaluating enobosarm 9 mg for the treatment of AR positive and ER positive metastatic breast cancer in women who have previously responded to hormonal therapy for the treatment of their metastatic breast cancer. Additionally, research and development expenses related to GTx-758 decreased $2.0 million from the year ended December 31, 2012. During year ended December 31, 2013, we were conducting our ongoing Phase 2 clinical trial evaluating GTx-758 as secondary hormonal therapy in men with metastatic and nonmetastatic CRPC, which was initiated in the third quarter of 2012. In the first quarter of 2012, we discontinued our three Phase 2 clinical trials of GTx-758 to treat men with advanced prostate cancer.

Research and development expenses increased 22% to $38.9 million for the year ended December 31, 2012 from $31.9 million for the year ended December 31, 2011. The $13.8 million increase in research and development expenses related to enobosarm 3 mg was due primarily to an increase in the costs related to the enrollment of and operations related to the two POWER Phase 3 clinical trials for enobosarm. The decrease in research and development expenses related to GTx-758 was due to the discontinuance in the first quarter of 2012 of the three Phase 2 clinical trials of GTx-758 to treat men with advanced prostate cancer that were placed on full clinical hold by the FDA, partially offset by the initiation in the third quarter of 2012 of the Phase 2 clinical trial to evaluate lower doses of GTx-758 as secondary hormonal therapy in men with metastatic CRPC.

"Other research and development" expenses include the cost of personnel, supplies and facilities associated with preclinical and discovery research and development activities for the years ended December 31, 2013, 2012 and 2011. For the year ended December 31, 2011, "Other research and development" also included research and development expenses of $2.0 million, which included an impairment charge of $1.6 million, for toremifene 80 mg development and $486,000 for toremifene 20 mg development.

General and Administrative Expenses

General and administrative expenses increased 4% to $11.3 million for the year ended December 31, 2013 from $10.8 million for the year ended December 31, 2012. This increase was primarily due to increased legal expenses related to intellectual property activities and the preparation of new equity incentive plans.

General and administrative expenses decreased 10% to $10.8 million for the year ended December 31, 2012 from $12.0 million for the year ended December 31, 2011. This decrease was primarily due to reduced salary and benefit expenses related to employee attrition of $739,000 and decreased legal expenses of $373,000.

Other (Expense) Income, Net

Other income, net for the year ended December 31, 2013 was $1.5 million compared to other expense, net of $19,000 for the year ended December 31, 2012. For the year ended December 31, 2013, we recorded a gain of $1.4 million from the sale of research and development property and equipment sold subsequent to the workforce reduction that occurred in October 2013. Other expense, net for the year ended December 31, 2012 was $19,000 compared to other income, net of $398,000 for the year ended December 31, 2011.

Discontinued Operations

Income from discontinued operations before income taxes was $22.7 million for the year ended December 31, 2012 and consisted of FARESTON® operating income of $3.8 million and the recognition of a gain of $18.8 million on the sale of rights and certain assets related to FARESTON®. Income from discontinued operations before income taxes for the year ended December 31, 2011 consisted of FARESTON® operating income.

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The components of FARESTON® operating income for the years ended December 31, 2012 and 2011 were as follows:

	Years Ended December 31,	
	2012	2011
	(in thousands)	
Product sales, net	$ 5,284	$ 6,673
Cost of product sales	(784)	(1,055)
Operating expenses	(655)	(3,411)
FARESTON® operating income	$ 3,845	$ 2,207

FARESTON® product sales, net decreased for the year ended December 31, 2012 compared to the prior year due primarily to the sale of the rights and certain assets related to FARESTON®, effective September 30, 2012. FARESTON® operating expenses decreased for the year ended December 31, 2012 as compared to the prior year due to the sale of FARESTON® and due to a reduction in FARESTON® marketing and medical education expenses.

Liquidity and Capital Resources

We have financed our operations to date primarily through public offerings and private placements of our common stock, as well as payments from our former collaborators. 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, 2013, we had an accumulated deficit of $455.4 million, which resulted primarily from:

- our research and development activities associated with:
  - the preclinical and clinical development of our SARM compounds, including enobosarm for the prevention and treatment of muscle wasting in patients with cancer;

- the preclinical and clinical development of GTx-758 for the treatment of advanced prostate cancer;
- the development of our discontinued toremifene 80 mg product candidate to reduce fractures and treat other estrogen deficiency side effects of androgen deprivation therapy in men with prostate cancer, including two Phase 2 clinical trials, a Phase 3 clinical trial, and the preparation and submission of a NDA to the FDA;
- the development of our discontinued toremifene 20 mg product candidate for the prevention of prostate cancer in high risk men with high grade prostatic intraepithelial neoplasia, including a Phase 2b clinical trial and a Phase 3 clinical trial;
- the preclinical development of other product candidates; and

- general and administrative expenses.

We expect to incur significant operating losses for the foreseeable future as we continue our clinical development activities and potentially seek regulatory approval of our product candidates. We do not expect to obtain FDA or EMA approval, or any other regulatory approvals, to market any of our product candidates, including enobosarm, for the foreseeable future, and it is possible that none of our product candidates will ever receive regulatory approvals.

At December 31, 2013, we had cash, cash equivalents and short-term investments of $14.7 million, compared to $56.1 million at December 31, 2012 and $74.4 million at December 31, 2011. On March 6, 2014, we completed a private placement of units consisting of 11,976,048 shares of common stock and warrants to purchase 10,179,642 shares of our common stock for gross proceeds of approximately $21.3 million. The warrants, which have a one year term, have a per share exercise price of $1.67 that is payable only in cash. If exercised in full, the warrants could result in additional gross proceeds of approximately $17.0 million.

In October 2012, we increased our cash and short-term investments when we received net cash proceeds of $18.9 million related to the sale of our rights and certain assets related to FARESTON®.

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In June 2011, we completed an underwritten public offering of 11,023,000 shares of our common stock at a price to the public of $4.75 per share. Net cash proceeds from the public offering were approximately $49.0 million, after deducting the underwriting discount and offering expenses.

In November 2010, we completed an underwritten public offering of 15,285,715 shares of our common stock at a price to the public of $2.80 per share. Net cash proceeds from the public offering were approximately $40.3 million after deducting underwriting discounts and commissions and other offering expenses.

In September 2006, we entered into a collaboration and license agreement with Ipsen, under which Ipsen paid us €21.5 million (approximately $27.1 million) as a license fee and expense reimbursement and paid us €1.5 million in equal installments over a three year period from the date of the agreement. In September of 2009, 2008, and 2007, we received €500,000 (approximately $726,000, $711,000, and $688,000, respectively) from Ipsen for the three annual installment payments. In February 2008, we earned a milestone of €1.0 million (approximately $1.5 million) with the achievement of the primary endpoint in the toremifene 80 mg Phase 3 clinical trial. As a result of the termination of our collaboration with Ipsen in March of 2011, we will not receive any future milestone payments, royalties or reimbursement of clinical development expenses from Ipsen that were provided for under our former collaboration with Ipsen.

In December 2007, we entered into an exclusive license and collaboration agreement with Merck, or the Merck Collaboration Agreement, which was terminated in March 2010. In connection with entering into this agreement, we received an upfront licensing fee of $40.0 million in January 2008, and Merck purchased approximately $30.0 million of our common stock in December 2007. Merck also paid us $15.0 million in guaranteed cost reimbursements for research and development activities in equal annual installments over a three year period beginning on the first anniversary of the effective date of the agreement. We received the three annual payments of $5 million from Merck in December 2010, 2009, and 2008, respectively. As a result of the termination of our collaboration with Merck, we will not receive any milestone payments or royalties from Merck that were provided for under our former collaboration with Merck. We have no ongoing collaborations for the development and commercialization of our product candidates, and we do not currently have any commitments for future external funding.

	Years Ending December 31,		
	2013	2012	2011
	(in thousands)		
Net cash used in operating activities	$ (43,971)	$ (37,109)	$ (33,089)
Net cash provided by (used in) investing activities	9,237	21,405	(10,299)
Net cash provided by financing activities	1,219	3	48,952
Net (decrease) increase in cash and cash equivalents	$ (33,515)	$ (15,701)	$ 5,564

Net cash used in operating activities in all periods resulted primarily from funding our operations.

Net cash provided by investing activities for the year ended December 31, 2013 resulted from the maturities of short-term investments of $9.3 million and proceeds from the sale of property and equipment of $1.4 million, offset partially by the purchase of short-term investments of $1.4 million and the purchase of information technology equipment and research and development equipment of approximately $32,000. Net cash used in investing activities for the year ended December 31, 2012 resulted from the proceeds from the sale of FARESTON®, net of cash expenses, of $18.9 million and the maturities of short-term investments of $14.6 million, offset by the purchase of short-term investments of $12.0 million and the purchase of information technology equipment and research and development equipment of approximately $142,000. Net cash used in investing activities for the year ended December 31, 2011 resulted from the purchase of short-term investments of $15.1 million and the purchase of information technology equipment and research and development equipment of approximately $54,000, offset by the maturities of short-term investments of $4.9 million.

Net cash provided by financing activities for the year ended December 31, 2013, 2012 and 2011 reflected proceeds from the exercise of employee stock options of $1.2 million, $85,000 and $55,000, respectively. For the year ended December 31, 2011, net cash provided by financing activities also reflected net proceeds from our underwritten public offering of common stock of $49.0 million in June 2011. Proceeds in all years presented were reduced by payments on our capital lease and financed equipment obligations.

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As of the date of this Annual Report on Form 10-K, we estimate that our current cash, cash equivalents and short-term investments, together with interest thereon, will be sufficient to meet our projected operating requirements for at least the next twelve months. We have based this estimate on our current business plan and assumptions that may prove to be wrong. We could utilize our available capital resources sooner than we currently expect, and we could need additional funding sooner than currently anticipated. In any event, to complete the development of and seek regulatory approval for enobosarm and GTx-758, we will need to obtain substantial additional funding. Based on our current business plan and assumptions, we will need to obtain substantial additional funding to conduct any additional clinical development of our product candidates beyond the Phase 2 clinical trials we are currently conducting and the Phase 1 clinical trials we plan to conduct to support a potential MAA submission to the EMA for enobosarm 3 mg.

Our estimate of the period of time or events 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 under Part I, Item 1A “Risk Factors” section of this Quarterly Report on Form 10-Q. Because of the numerous risks and uncertainties associated with the development and potential commercialization 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 the future development of our product candidates, if any. Our future funding requirements will depend on many factors, including:

- the scope, rate of progress and cost of our clinical development programs, including our ongoing and any future clinical trials of enobosarm and GTx-758;
- the terms and timing of any potential collaborative, licensing and other strategic arrangements that we may establish;
- the amount and timing of any licensing fees, milestone payments and royalty payments from potential collaborators, if any;
- future clinical trial results;
- the cost and timing of regulatory filings and/or approvals to commercialize our product candidates and any related restrictions, limitations, and/or warnings in the label of an approved product candidate;
- 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, and the cost of defending any other litigation claims.

We do not currently have any commitments for future external funding nor do we currently have any sources of revenue. Until we can generate a sufficient amount of product revenue, which we may never do, we expect to finance future cash needs through public or private equity offerings, debt financings or collaboration and licensing arrangements, or a combination of the above, as well as through interest income earned on the investment of our cash balances and short-term investments. In October 2013, following our receipt of the negative results in our POWER 1 and POWER 2 clinical trials evaluating enobosarm 3 mg, we announced and implemented a workforce reduction of approximately 60%. If we are unable to raise additional funds when needed, we may need to further reduce our expenditures, perhaps significantly, to preserve our cash. Cost-cutting measures that we may take in the future may not be sufficient to enable us to meet our cash requirements, and they may negatively affect our business and growth prospects.

To the extent that we raise additional funds through potential collaboration, partnering or other strategic arrangements, it may be necessary to relinquish rights to some of our technologies or product candidates, or grant licenses on terms that are not favorable to us, any of which could result in the stockholders of GTx having little or no continuing interest in our enobosarm and/or GTx-758 programs as stockholders or otherwise. To the extent we raise additional funds by issuing equity securities, our stockholders may experience significant dilution, particularly given our currently depressed stock price, and debt financing, if available, may involve restrictive covenants. For example, we announced in March 2014 that we completed a private placement of common stock and warrants to purchase common stock for gross proceeds of approximately $21.3 million, which financing was substantially dilutive, and our stockholders may experience additional, perhaps substantial, dilution should we again raise additional funds by issuing equity securities. Any debt financing or additional equity that we raise may contain terms that are not favorable to us or our stockholders. Our ability to raise additional funds and the terms upon which we are able to raise such funds have been severely harmed by the failure of the two enobosarm 3 mg Phase 3 clinical

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trials to meet both of the co-primary endpoints, and may in the future be adversely impacted by the uncertainty regarding our ability to obtain approval of enobosarm 3 mg in the European Union in the absence of additional Phase 3 development of enobosarm 3 mg and the terms of any such approval. As a result of these and other factors, we cannot be certain that additional funding will be available on acceptable terms, or at all. If adequate funds are not available when we need them, we may be required to delay, reduce the scope of or eliminate one or more of our research or development programs, including our enobosarm and GTx-758 programs, or conduct additional workforce or other expense reductions, any of which could have a material adverse effect on our business and our prospects.

Contractual Obligations

At December 31, 2013, we had contractual obligations as follows:

Contractual Obligations(1)	Payment Due by Period (in thousands) Total	Less than 1 year	1-3 years	4-5 years	More than 5 years
Operating lease obligations(2)	$ 738	$ 553	$ 185	$ —	$ —

(1) This table does not include any royalty obligations under our license agreement with UTRF as the timing and likelihood of such payments are not known. In addition to the minimum payments due under our current license agreement, we may be required to pay royalties on any net sales of product if we receive regulatory approval for a SARM product candidate, including enobosarm, and successfully market the product. Additionally, if we sublicense rights under our SARM License Agreement, we also are obligated to pay a sublicense royalty on any licensing fee or milestone payments we may receive from a sublicensee.

(2) Our long-term commitment under the operating lease consists of payments relating to a sublease for office space at 175 Toyota Plaza, Memphis, Tennessee. The sublease for the premises at 175 Toyota Plaza expires on April 30, 2015 and includes escalating rental payments.

Off-Balance Sheet Arrangements

We have not engaged in any off-balance sheet arrangements, including the use of standard finance, special purpose entities or variable interest entities.

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 and investments in Federal Deposit Insurance Corporation insured certificates of deposit. 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. The effect of a hypothetical decrease of ten percent in the average yield earned on our cash equivalents and short-term investments would have resulted in an immaterial decrease in our interest income for the year ended December 31, 2013.

In addition, we have exposure to fluctuations in certain foreign currencies in countries in which we conduct clinical trials. Most of our foreign expenses incurred are associated with conducting clinical trials for enobosarm at clinical trial sites in Europe. Consequently, changes in exchange rates could result in material exchange losses and could unpredictably, materially and adversely affect our financial position, results of operations and cash flows. A hypothetical 10% increase or decrease in foreign exchange rates would result in an immaterial change in our financial assets and liabilities denominated in euros. This potential change is based on a sensitivity analysis performed on our financial position at December 31, 2013. Actual results may differ materially. We have elected not to hedge our exposure to foreign currency fluctuations.

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ITEM 8. FINANCIAL STATEMENTS AND SUPPLEMENTARY DATA

Our financial statements and the reports 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 these reports and our financial statements is included in Part IV, Item 15 below.

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

Not applicable.

ITEM 9A. CONTROLS AND PROCEDURES

Disclosure Controls and Procedures

We maintain disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) of the Securities Exchange Act of 1934, as amended (the "Exchange Act")) that are designed to ensure that information required to be disclosed in the reports that we file or submit under the 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 principal executive officer and principal financial officer, as appropriate, to allow for timely decisions regarding required disclosures.

We have carried out an evaluation, under the supervision and with the participation of our management, including our principal executive officer and principal financial officer, of the effectiveness of the design and operation of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act) as of the end of the period covered by this report. Based on the evaluation of these disclosure controls and procedures, our principal executive officer and principal financial officer have concluded that our disclosure controls and procedures were effective.

Management's Report on Internal Control Over Financial Reporting

We, as management of GTx, Inc., are responsible for establishing and maintaining adequate internal control over financial reporting, as such term is defined in Securities Exchange Act Rule 13a-15(f). Internal control over financial reporting is a process designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with United States generally accepted accounting principles. Any system of internal control, no matter how well designed, has inherent limitations, including the possibility that a control can be circumvented or overridden and misstatements due to error or fraud may occur and not be detected. Also, because of changes in conditions, internal control effectiveness may vary over time. Accordingly, even an effective system of internal control will provide only reasonable assurance that the objectives of the internal control system are met.

Under the supervision and with the participation of management, including our principal executive officer and principal financial officer, we conducted an evaluation of the effectiveness of our internal control over financial reporting as of December 31, 2013 using the criteria for effective internal control over financial reporting as described in "Internal Control — Integrated Framework," issued by the Committee of Sponsoring Organizations of the Treadway Commission (1992 framework). Based on this evaluation, we concluded that, as of December 31, 2013, our internal control over financial reporting was effective. The effectiveness of our internal control over financial reporting has been audited by Ernst & Young LLP, independent registered public accounting firm.

Attestation Report of the Independent Registered Public Accounting Firm

Ernst & Young LLP, an independent registered public accounting firm, has issued an audit report on our internal control over financial reporting, which report is included elsewhere herein.

Changes in Internal Control Over Financial Reporting

There were no changes in our internal control over financial reporting during the fourth quarter of 2013 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

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ITEM 9B. OTHER INFORMATION

None.

PART III

Certain information required by Part III is omitted from this Annual Report on Form 10-K because we will file our definitive proxy statement for our 2014 Annual Meeting of Stockholders with the U.S. Securities and Exchange Commission pursuant to Regulation 14A (the "2014 Proxy Statement") not later than 120 days after the end of the fiscal year covered by this Annual Report on Form 10-K, and certain information included in the 2014 Proxy Statement is incorporated herein by reference.

ITEM 10. DIRECTORS, EXECUTIVE OFFICERS AND CORPORATE GOVERNANCE

(1) The information required by this Item concerning our directors and nominees for director, including information with respect to our audit committee and audit committee financial experts, may be found under the section entitled "Proposal No. 1 — Election of Directors" and "Additional Information About the Board of Directors and Certain Corporate Governance Matters" appearing in the 2014 Proxy Statement. Such information is incorporated herein by reference.

(2) The information required by this Item concerning compliance with Section 16(a) of the Securities Exchange Act of 1934 may be found in the section entitled "Section 16(a) Beneficial Ownership Reporting Compliance" appearing in the 2014 Proxy Statement. Such information is incorporated herein by reference.

(3) The information required by this Item concerning our executive officers is set forth in the section entitled "Management — Executive Officers of Registrant" in Part I, Item 1 of this Form 10-K.

(4) Our Board has adopted a Code of Business Conduct and Ethics applicable to all officers, directors and employees as well as Guidelines on Governance Issues. These documents are available on our Web site (www.gtxinc.com) under "About GTx" at "Governance." We will provide a copy of these documents to any person, without charge, upon request, by writing to us at GTx, Inc., Director, Corporate Communications and Financial Analysis, 175 Toyota Plaza, Suite 700, Memphis, Tennessee 38103. We intend to satisfy the disclosure requirement under Item 5.05 of Form 8-K regarding an amendment to, or waiver from, a provision of the Code of Business Conduct and Ethics by posting such information on our Web site at the address and the location specified above.

ITEM 11. EXECUTIVE COMPENSATION

(1) The information required by this Item concerning director and executive compensation is incorporated herein by reference to the information from the 2014 Proxy Statement under the sections entitled "Compensation Discussion and Analysis," "Executive Compensation" and "Director Compensation."

(2) The information required by this Item concerning Compensation Committee interlocks and insider participation is incorporated herein by reference to the information from the 2014 Proxy Statement under the section entitled "Compensation Committee Interlocks and Insider Participation."

(3) The information required by this Item concerning our Compensation Committee's review and discussion of our Compensation Discussion and Analysis is incorporated herein by reference to the information from the 2014 Proxy Statement under the section entitled "Compensation Committee Report."

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

(1) The information required by this Item with respect to security ownership of certain beneficial owners and management is incorporated herein by reference to the information from the 2014 Proxy Statement under the section entitled "Security Ownership of Certain Beneficial Owners and Management."

(2) The information required by this Item with respect to securities authorized for issuance under our equity

compensation plans is incorporated herein by reference to the information from the 2014 Proxy Statement under the section entitled "Equity Compensation Plan Information."

ITEM 13. CERTAIN RELATIONSHIPS AND RELATED TRANSACTIONS, AND DIRECTOR INDEPENDENCE

(1) The information required by this Item concerning related party transactions is incorporated herein by reference to the information from the 2014 Proxy Statement under the section entitled "Certain Relationships and Related Party Transactions."

(2) The information required by this Item concerning director independence is incorporated herein by reference to the information from the 2014 Proxy Statement under the section entitled "Additional Information About the Board of Directors and Certain Corporate Governance Matters — Director Independence."

ITEM 14. PRINCIPAL ACCOUNTING FEES AND SERVICES

The information required by this Item is incorporated herein by reference to the information from the 2014 Proxy Statement under the section entitled "Proposal No. 3 — Ratification of Appointment of Independent Registered Public Accounting Firm."

PART IV

ITEM 15. EXHIBITS AND FINANCIAL STATEMENT SCHEDULES

(a)(1) Index to Financial Statements

Page	Description
F-2	Management's Report on Internal Control Over Financial Reporting
F-3	Reports of Independent Registered Public Accounting Firm
F-5	Balance Sheets at December 31, 2013 and 2012
F-6	Statements of Operations for the Years Ended December 31, 2013, 2012 and 2011
F-7	Statements of Stockholders' Equity for the Years Ended December 31, 2013, 2012 and 2011
F-8	Statements of Cash Flows for the Years Ended December 31, 2013, 2012 and 2011
F-9	Notes to Financial Statements

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

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

(b) Exhibits — The following exhibits are included herein or incorporated herein by reference:

Exhibit Number	Exhibit Description	Incorporation By Reference			
		Form	SEC File No.	Exhibit	Filing Date
2.1	Asset Purchase Agreement dated as of September 28, 2012 between the Registrant and Strakan International S.à r.l.	8-K	000-50549	2.1	10/03/2012
3.1	Restated Certificate of Incorporation of GTx, Inc.	S-3	333-127175	4.1	08/04/2005
3.2	Certificate of Amendment of Restated Certificate of Incorporation of GTx, Inc.	8-K	000-50549	3.2	05/06/2011
3.3	Amended and Restated Bylaws of GTx, Inc.	8-K	000-50549	3.2	07/26/2007
4.1	Reference is made to Exhibits 3.1, 3.2 and 3.3				
4.2	Specimen of Common Stock Certificate	S-1	333-109700	4.2	12/22/2003
4.3	Amended and Restated Registration Rights Agreement between Registrant and J. R. Hyde, III dated August 7, 2003	S-1	333-109700	4.4	10/15/2003

4.4	Consent, Waiver and Amendment among Registrant, J. R. Hyde, III and Pittco Associates, L.P. dated December 3, 2007	S-3	333-148321	4.6	12/26/2007
4.5+	Waiver and Amendment Agreement among Registrant, J.R. Hyde, III and Pittco Associates, L.P. dated March 6, 2014	—	—	—	—
4.6+	Registration Rights Agreement among Registrant, J.R. Hyde, III and The Pyramid Peak Foundation, dated March 6, 2014	—	—	—	—
4.7+	Form of Common Stock Warrant, issued by Registrant pursuant to the Securities Purchase Agreement, dated March 3, 2014, among the Registrant, J.R. Hyde, III and The Pyramid Peak Foundation	—	—	—	—

10.1†	Consolidated, Amended, and Restated License Agreement dated July 24, 2007, between Registrant and University of Tennessee Research Foundation	10-Q	000-50549	10.40	11/09/2007
10.2	First Amendment, dated December 29, 2008, to the Consolidated, Amended and Restated License Agreement dated July 24, 2007 between the Registrant and University of Tennessee Research Foundation	10-K	000-50549	10.47	03/03/2009
10.3*	Form of Indemnification Agreement	S-1	333-109700	10.12	12/22/2003
10.4*	Genotherapeutics, Inc. 1999 Stock Option Plan, as amended through December 10, 2009, and Form of Stock Option Agreement	10-K	000-50549	10.1	03/15/2010
10.5*	GTx, Inc. 2000 Stock Option Plan, as amended through December 10, 2009, and Form of Stock Option Agreement	10-K	000-50549	10.2	03/15/2010
10.6*	GTx, Inc. 2001 Stock Option Plan, as amended through November 3, 2009, and Form of Stock Option Agreement	10-K	000-50549	10.3	03/15/2010
10.7*	GTx, Inc. 2002 Stock Option Plan, as amended through November 3, 2009, and Form of Stock Option Agreement	10-K	000-50549	10.4	03/15/2010
10.8*	GTx, Inc. 2004 Equity Incentive Plan and Form of Stock Option Agreement	S-1	333-109700	10.5	01/15/2004
10.9*	GTx, Inc. 2004 Equity Incentive Plan, as amended effective April 30, 2008	8-K	000-50549	10.6	05/06/2008
10.10*	GTx, Inc. 2004 Equity Incentive Plan, as amended effective November 4, 2008 and Form of Stock Option Agreement	10-K	000-50549	10.52	03/03/2009
10.11*	GTx, Inc. 2004 Non-Employee Directors' Stock Option Plan and Form of Stock Option Agreement	S-1	333-109700	10.6	01/15/2004
10.12*	Amended and Restated GTx, Inc. 2004 Non-Employee Directors' Stock Option Plan, effective April 26, 2006	8-K	000-50549	10.1	04/27/2006
10.13*	Form of Stock Option Agreement under the Amended and Restated GTx, Inc. 2004 Non-Employee Directors' Stock Option Plan	10-Q	000-50549	10.35	08/09/2006
10.14*	Amended and Restated GTx, Inc. 2004 Non-Employee Directors' Stock Option Plan, as amended effective November 4, 2008	10-K	000-50549	10.51	03/03/2009
10.15*	GTx, Inc. 2013 Equity Incentive Plan	S-8	333-188377	99.1	05/06/2013
10.16*	Form of Stock Option Grant Notice and Option Agreement under the GTx, Inc. 2013 Equity Incentive Plan (Standard Form)	10-Q	000-50549	10.2	07/22/2013
10.17*	Form of Retention Stock Option Grant Notice and Option Agreement under the GTx, Inc. 2013 Equity Incentive Plan	10-Q	000-50549	10.3	11/12/2013
10.18*	Form of Retention Restricted Stock Unit Grant Notice and Restricted Stock Unit Award Agreement under the GTx, Inc. 2013 Equity Incentive Plan	10-Q	000-50549	10.4	11/12/2013
10.19*	GTx, Inc. 2013 Non-Employee Director Equity Incentive Plan	S-8	333-188377	99.2	05/06/2013
10.20*	Form of Stock Option Grant Notice and Option Agreement under the GTx, Inc. 2013 Non-Employee Director Equity Incentive Plan	10-Q	000-50549	10.4	07/22/2013
10.21*	Amended and Restated Employment Agreement dated February 14, 2013, between Registrant and Mitchell S. Steiner, M.D.	10-K	000-50549	10.19	03/05/2013
10.22*	Amended and Restated Employment Agreement dated February 14, 2013, between Registrant and Marc S. Hanover	10-K	000-50549	10.20	03/05/2013
10.23*	Amended and Restated Employment Agreement dated February 14, 2013, between Registrant and Mark E. Mosteller	10-K	000-50549	10.21	03/05/2013

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10.24*	Amended and Restated Employment Agreement dated February 14,	10-K	000-50549	10.22	03/05/2013

	2013, between Registrant and Henry P. Doggrell				
10.25*	Amended and Restated Employment Agreement dated February 14, 2013 between Registrant and James T. Dalton	10-K	000-50549	10.23	03/05/2013
10.26*	Form of Retention Benefits Letter Agreement for Mitchell S. Steiner and Marc S. Hanover	10-Q	000-50549	10.1	11/12/2013
10.27*	Form of Retention Benefits Letter Agreement for James T. Dalton and Henry P. Doggrell	10-Q	000-50549	10.2	11/12/2013
10.28*+	Confidential Separation Agreement and General Release, dated December 20, 2014, between Registrant and Mark E. Mosteller	—	—	—	—
10.29*	Amended and Restated GTx, Inc. Executive Bonus Compensation Plan, effective November 4, 2008	10-K	000-50549	10.53	03/03/2009
10.30*	2013 Compensation Information for Registrant's Executive Officers	10-K	000-50549	10.26	03/05/2013
10.31*+	2014 Compensation Information for Registrant's Executive Officers	—	—	—	—
10.32*	Directors' Deferred Compensation Plan, as amended effective November 4, 2008	10-K	000-50549	10.49	03/03/2009
10.33*	Directors' Deferred Compensation Plan, as amended and restated effective February 14, 2013	10-K	000-50549	10.28	03/05/2013
10.34*	Non-Employee Director Compensation Policy of GTx, Inc., effective February 18, 2011	10-Q	000-50549	10.50	05/09/2011
10.35*	Non-Employee Director Compensation Policy of GTx, Inc., effective February 14, 2013	10-K	000-50549	10.30	03/05/2013
10.36	Lease Agreement, dated March 7, 2001, between The University of Tennessee and TriStar Enterprises, Inc.	S-1	333-109700	10.13	10/15/2003
10.37	Sublease Agreement dated October 1, 2000, as amended, between Registrant and TriStar Enterprises, Inc.	S-1	333-109700	10.14	10/15/2003
10.38	Sublease Agreement dated April 1, 2005, as amended, between Registrant and TriStar Enterprises, Inc.	10-Q	000-50549	10.27	07/27/2005
10.39	Sublease Agreement dated October 1, 2009 between Registrant and University of Tennessee Research Foundation	10-K	000-50549	10.55	03/15/2010
10.40	Memorandum of Understanding Concerning the Lease Agreement between The University of Tennessee Research Foundation and the Registrant as Amended July 20, 2009	10-Q	000-50549	10.59	08/09/2011
10.41	Second Memorandum of Understanding Concerning the Lease Agreement between Registrant and The University of Tennessee Research Foundation as Amended July 20, 2009	10-Q	000-50549	10.5	07/22/2013
10.42	Third Memorandum of Understanding, made effective as of October 1, 2013, Concerning the Lease Agreement between Registrant and The University of Tennessee Research Foundation as Amended July 20, 2009	10-Q	000-50549	10.5	11/12/2013
10.43	Sublease Agreement, dated December 17, 2007, by and between the Registrant and ESS SUSA Holdings, LLC	10-K	000-50549	10.46	03/11/2008
10.44	First Amendment, dated July 21, 2008, to the Sublease and Parking Sublicense Agreements dated December 17, 2007 by and between the Registrant and ESS SUSA Holdings, LLC	10-K	000-50549	10.54	03/03/2009
10.45	Second Amendment to Sublease and Parking Sublicense Agreements dated January 1, 2011 by and between the Registrant and ESS SUSA Holdings, LLC	10-K	000-50549	10.57	03/08/2011
10.46+	Securities Purchase Agreement, dated March 3, 2014, by and among Registrant, J.R. Hyde, III and The Pyramid Peak Foundation	—	—	—	—
23.1+	Consent of Independent Registered Public Accounting Firm	—	—	—	—
24.1+	Power of Attorney (included on the signature pages hereto)	—	—	—	—

31.1+	Certification of Chief Executive Officer, as required by Rule 13a-14(a) or Rule 15d-14(a)	—	—	—	—

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31.2+	Certification of Principal Financial Officer, as required by Rule 13a-14(a) or Rule 15d-14(a)	—	—	—	—
32.1+	Certification of Chief Executive Officer, as required by Rule 13a-14(b) or Rule 15d-14(b) and Section 1350 of Chapter 63 of Title 18 of the United States Code (18 U.S.C. §1350)(1)	—	—	—	—
32.2+	Certification of Principal Financial Officer, as required by Rule 13a-14(b) or Rule 15d-14(b) and Section 1350 of Chapter 63 of Title 18 of the United States Code (18 U.S.C. §1350)(1)	—	—	—	—
101.INS+	XBRL Instance Document	—	—	—	—
101.SCH+	XBRL Taxonomy Extension Schema Document	—	—	—	—
101.CAL+	XBRL Taxonomy Extension Calculation Linkbase Document	—	—	—	—
101.DEF+	XBRL Taxonomy Extension Definition Linkbase Document	—	—	—	—
101.LAB+	XBRL Taxonomy Extension Labels Linkbase Document	—	—	—	—
101.PRE+	XBRL Taxonomy Extension Presentation Linkbase Document	—	—	—	—

† Confidential treatment has been granted with respect to certain portions of this exhibit. This exhibit omits the information subject to this confidentiality request. Omitted portions have been filed separately with the SEC.

* Indicates a management contract or compensation plan or arrangement.

+ Filed herewith

(1) This certification accompanies the Form 10-K to which it relates, is not deemed filed with the Securities and Exchange Commission and is not to be incorporated by reference into any filing of the Registrant under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended (whether made before or after the date of the Form 10-K), irrespective of any general incorporation language contained in such filing.

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SIGNATURES

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

GTx, Inc.

By /s/ Mitchell S. Steiner
Mitchell S. Steiner, M.D., F.A.C.S.
Chief Executive Officer, Vice Chairman and Director Date: March 12, 2014

POWER OF ATTORNEY

KNOW ALL PERSONS BY THESE PRESENT, that each person whose signature appears below constitutes and appoints Mitchell S. Steiner and Marc S. Hanover, and each of them, acting individually, as his attorney-in-fact, each with full power of substitution and resubstitution, for him or her and in his or her name, place and stead, in any and all capacities, to sign any and all amendments to this Annual Report on Form 10-K, and to file the same, with all exhibits thereto, and other documents in connection therewith, with the Securities and Exchange Commission, granting unto said attorneys-in-fact and agents, and each of them, full power and authority to do and perform each and every act and thing requisite and necessary to be done in connection therewith and about the premises, as fully to all intents and purposes as he or she might or could do in person, hereby ratifying and confirming all that said attorneys-in-fact and agents, or any of them, or their or his or her substitute or substitutes, may lawfully do or cause to be done by virtue hereof.

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

		Date
/s/ J. R. Hyde, III	Chairman of the Board of Directors	March 12, 2014

J. R. Hyde, III

/s/ Mitchell S. Steiner Mitchell S. Steiner, M.D., F.A.C.S.	Chief Executive Officer, Vice Chairman and Director (Principal Executive Officer)	March 12, 2014
/s/ Marc S. Hanover Marc S. Hanover	President and Chief Operating Officer and Acting Principal Financial Officer (*Principal Financial Officer*)	March 12, 2014
/s/ Jason T. Shackelford Jason T. Shackelford	Corporate Controller, Director of Accounting and Acting Principal Accounting Officer (*Principal Accounting Officer*)	March 12, 2014
/s/ Michael G. Carter Michael G. Carter, M. D.	Director	March 12, 2014
/s/ Barrington J. A. Furr Barrington J. A. Furr	Director	March 12, 2014
/s/ J. Kenneth Glass J. Kenneth Glass	Director	March 12, 2014
/s/ Kenneth S. Robinson Kenneth S. Robinson, M.D.	Director	March 12, 2014

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GTx, Inc.

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MANAGEMENT'S REPORT ON
INTERNAL CONTROL OVER FINANCIAL REPORTING

We, as management of GTx, Inc., are responsible for establishing and maintaining adequate internal control over financial reporting, as such term is defined in Securities Exchange Act Rule 13a-15(f). Internal control over financial reporting is a process designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with United States generally accepted accounting principles. Any system of internal control, no matter how well designed, has inherent limitations, including the possibility that a control can be circumvented or overridden and misstatements due to error or fraud may occur and not be detected. Also, because of changes in conditions, internal control effectiveness may vary over time. Accordingly, even an effective system of internal control will provide only reasonable assurance that the objectives of the internal control system are met.

Under the supervision and with the participation of management, including our principal executive officer and principal financial officer, we conducted an evaluation of the effectiveness of our internal control over financial reporting as of December 31, 2013 using the criteria for effective internal control over financial reporting as described in "Internal Control — Integrated Framework," issued by the Committee of Sponsoring Organizations of the Treadway Commission (1992 framework). Based on this evaluation, we concluded that, as of December 31, 2013, our internal control over financial reporting was effective. The effectiveness of our internal control over financial reporting has been audited by Ernst & Young LLP, independent registered public accounting firm who also audited the Company's financial statements included in this Annual Report of Form 10-K. Ernst & Young LLP's report on the Company's internal control over financial reporting is included in the Annual Report of the 10-K.

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

/s/ Marc S. Hanover
Marc S. Hanover
President and Principal Financial Officer

Memphis, Tennessee
March 12, 2014

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Report of Independent Registered Public Accounting Firm

The Board of Directors and Stockholders of GTx, Inc.

We have audited GTx, Inc.'s internal control over financial reporting as of December 31, 2013, based on criteria established in Internal Control—Integrated Framework issued by the Committee of Sponsoring Organizations of the Treadway Commission (1992 framework), (the COSO criteria). GTx, Inc.'s management is responsible for maintaining effective internal control over financial reporting, and for its assessment of the effectiveness of internal control over financial reporting included in the accompanying Management's Report on Internal Control Over Financial Reporting. Our responsibility is to express an opinion on the Company's internal control over financial reporting based on our audit.

We conducted our audit 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 effective internal control over financial reporting was maintained in all material respects. Our audit included obtaining an understanding of internal control over financial reporting, assessing the risk that a material weakness exists, testing and evaluating the design and operating effectiveness of internal control based on the assessed risk, and performing such other procedures as we considered necessary in the circumstances. We believe that our audit provides a reasonable basis for our opinion.

A company's internal control over financial reporting is a process designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles. A company's internal control over financial reporting includes those policies and procedures that (1) pertain to the maintenance of records that, in reasonable detail, accurately and fairly reflect the transactions and dispositions of the assets of the company; (2) provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that receipts and expenditures of the company are being made only in accordance with authorizations of management and directors of the company; and (3) provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use or disposition of the company's assets that could have a material effect on the financial statements.

Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Also, projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate.

In our opinion, GTx, Inc. maintained, in all material respects, effective internal control over financial reporting as of December 31, 2013, based on the COSO criteria**.**

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States), the balance sheets of GTx, Inc. as of December 31, 2013 and 2012, and the related statements of operations, stockholders' equity and cash flows for each of the three years in the period ended December 31, 2013 and our report dated March 12, 2014 expressed an unqualified opinion thereon.

/s/ Ernst & Young LLP

Memphis, Tennessee
March 12, 2014

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Report of Independent Registered Public Accounting Firm

The Board of Directors and Stockholders of GTx, Inc.

We have audited the accompanying balance sheets of GTx, Inc. as of December 31, 2013 and 2012, and the related statements of operations, stockholders' equity and cash flows for each of the three years in the period ended December 31, 2013. 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. An audit includes examining, on a test basis, evidence supporting the amounts and disclosures in the financial statements. An audit also includes assessing the accounting principles used and significant estimates made by management, as well as evaluating the overall financial statement presentation. We believe that our audits provide a reasonable basis for our opinion.

In our opinion, the financial statements referred to above present fairly, in all material respects, the financial position of GTx, Inc. at December 31, 2013 and 2012, and the results of its operations and its cash flows for each of the three years in the period ended December 31, 2013, in conformity with U.S.

generally accepted accounting principles.

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States), GTx, Inc.'s internal control over financial reporting as of December 31, 2013, based on criteria established in Internal Control-Integrated Framework issued by the Committee of Sponsoring Organizations of the Treadway Commission (1992 framework) and our report dated March 12, 2014 expressed an unqualified opinion thereon.

/s/ Ernst & Young LLP

Memphis, Tennessee
March 12, 2014

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

	December 31, 2013	December 31, 2012
ASSETS		
Current assets:		
Cash and cash equivalents	$ 14,529	$ 48,044
Short-term investments	200	8,045
Prepaid expenses and other current assets	442	726
Total current assets	15,171	56,815
Property and equipment, net	112	507
Intangible and other assets, net	322	452
Total assets	$ 15,605	$ 57,774
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	$ 808	$ 1,707
Accrued expenses and other current liabilities	3,759	7,788
Total current liabilities	4,567	9,495
Other long-term liabilities	354	578
Commitments and contingencies		
Stockholders' equity:		
Common stock, $0.001 par value: 120,000,000 shares authorized at December 31, 2013 and December 31, 2012; 63,185,389 and 62,818,424 shares issued and outstanding at December 31, 2013 and December 31, 2012, respectively	63	63
Additional paid-in capital	465,981	460,887
Accumulated deficit	(455,360)	(413,249)
Total stockholders' equity	10,684	47,701
Total liabilities and stockholders' equity	$ 15,605	$ 57,774

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

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

	Years Ended December 31, 2013	2012	2011
Revenues:			
Collaboration revenue	$ —	$ —	$ 8,066
Expenses:			
Research and development expenses	32,318	38,887	31,938
General and administrative expenses	11,281	10,845	12,027
Total expenses	43,599	49,732	43,965
Loss from operations	(43,599)	(49,732)	(35,899)
Other income (expense), net	1,488	(19)	398
Loss from operations before income taxes	(42,111)	(49,751)	(35,501)
Income tax benefit	—	8,821	886
Net loss from continuing operations	(42,111)	(40,930)	(34,615)
Income from discontinued operations before income taxes	—	22,676	2,207

Income tax expense	—	(8,821)	(886)
Net income from discontinued operations	—	13,855	1,321
Net loss	$ (42,111)	$ (27,075)	$ (33,294)
Net (loss) income per share — basic and diluted:			
Net loss from continuing operations	$ (0.67)	$ (0.65)	$ (0.60)
Net income from discontinued operations	—	0.22	0.02
Net loss per share	$ (0.67)	$ (0.43)	$ (0.58)
Weighted average shares outstanding:			
Basic and diluted	63,057,142	62,809,219	57,359,466

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

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GTx, Inc.
STATEMENTS OF STOCKHOLDERS' EQUITY
For the Years Ended December 31, 2013, 2012 and 2011
(in thousands, except share data)

	Stockholders' Equity				
	Common Stock		Additional Paid-in Capital	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount			
Balances at January 1, 2011	51,719,187	$ 52	$ 404,555	$ (352,880)	$ 51,727
Issuance of common stock, net of offering costs	11,023,000	11	48,971	—	48,982
Issuance of common stock under deferred compensation arrangements	35,036	—	—	—	—
Exercise of employee stock options	13,000	—	55	—	55
Directors' deferred compensation	—	—	178	—	178
Share-based compensation	—	—	4,226	—	4,226
Net loss	—	—	—	(33,294)	(33,294)
Balances at December 31, 2011	62,790,223	63	457,985	(386,174)	71,874
Exercise of employee stock options	28,201	—	85	—	85
Directors' deferred compensation	—	—	169	—	169
Share-based compensation	—	—	2,648	—	2,648
Net loss	—	—	—	(27,075)	(27,075)
Balances at December 31, 2012	62,818,424	63	460,887	(413,249)	47,701
Issuance of common stock under deferred compensation arrangements	45,667	—	—	—	—
Exercise of employee stock options	321,298	—	1,226	—	1,226
Directors' deferred compensation	—	—	135	—	135
Share-based compensation	—	—	3,733	—	3,733
Net loss	—	—	—	(42,111)	(42,111)
Balances at December 31, 2013	63,185,389	$ 63	$ 465,981	$ (455,360)	$ 10,684

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

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

	Years Ended December 31,		
	2013	2012	2011
Cash flows from operating activities:			
Net loss	$ (42,111)	$ (27,075)	$ (33,294)
Adjustments to reconcile net loss to net cash used in operating activities:			
Gain on sale of FARESTON®	—	(18,831)	—
Share-based compensation	3,733	2,648	4,226
Directors' deferred compensation	135	169	178
Depreciation and amortization	384	750	1,040
Gain on sale of property and equipment	(1,366)	—	—
Deferred revenue amortization	—	—	(8,066)
Impairment of intangible assets	—	—	1,598
Changes in assets and liabilities:			
Prepaid expenses and other assets	399	1,857	(849)
Accounts payable	(899)	488	371
Accrued expenses and other liabilities	(4,246)	2,885	1,707

Net cash used in operating activities	(43,971)	(37,109)	(33,089)
Cash flows from investing activities:			
Purchase of property and equipment	(32)	(142)	(54)
Proceeds from the sale of property and equipment	1,424	—	—
Purchase of short-term investments, held to maturity	(1,425)	(11,980)	(15,145)
Proceeds from maturities of short-term investments, held to maturity	9,270	14,630	4,900
Proceeds from the sale of FARESTON®, net of cash expenses	—	18,897	—
Net cash provided by (used in) investing activities	9,237	21,405	(10,299)
Cash flows from financing activities:			
Proceeds from issuance of common stock	—	—	48,982
Proceeds from exercise of employee stock options	1,226	85	55
Payments on capital lease and financed equipment obligations	(7)	(82)	(85)
Net cash provided by financing activities	1,219	3	48,952
Net (decrease) increase in cash and cash equivalents	(33,515)	(15,701)	5,564
Cash and cash equivalents, beginning of period	48,044	63,745	58,181
Cash and cash equivalents, end of period	$ 14,529	$ 48,044	$ 63,745

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

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

1. Business

GTx, Inc. ("GTx" or the "Company"), a Delaware corporation incorporated on September 24, 1997 and headquartered in Memphis, Tennessee, is a biopharmaceutical company dedicated to the discovery, development and commercialization of small molecules for the treatment of cancer, including treatments for prostate and breast cancer, cancer supportive care, including prevention and treatment of cancer-related muscle wasting, and other serious medical conditions.

The Company is developing selective androgen receptor modulators ("SARMs"), including its lead product candidate, enobosarm (GTx-024). SARMs are a new class of drugs with the potential to prevent and treat muscle wasting in patients with cancer and other musculoskeletal wasting or muscle loss conditions, including chronic sarcopenia (age related muscle loss), as well as the potential to be used as a novel hormonal therapy for the treatment of metastatic breast cancer. The Company announced in August 2013 that its two Phase 3 clinical trials evaluating enobosarm 3 mg for the prevention and treatment of muscle wasting in patients with advanced non-small cell lung cancer ("NSCLC") failed to meet the primary statistical criterion for the co-primary endpoints of lean body mass and physical function that were assessed statistically using responder analyses as pre-specified for the United States Food and Drug Administration ("FDA"). The Company met with representatives from two member countries to the European Medicines Agency ("EMA") in January of 2014 to review and discuss the results of the POWER trials to determine an appropriate path forward for submitting a marketing authorization application ("MAA") in the European Union for enobosarm 3 mg for the prevention and treatment of muscle wasting in patients with advanced NSCLC. Based on input from the two member countries, the Company believes data from the POWER 1 trial, as well as supporting data from the POWER 2 trial, are sufficient to support the submission of a MAA to the EMA seeking marketing authorization for enobosarm 3 mg in the more narrow indication of the prevention and treatment of muscle wasting in patients with advanced NSCLC treated with platinum plus taxane chemotherapy. The Company plans to initiate seven Phase 1 clinical studies that are typically required for registration purposes and develop a pediatric investigational plan, or PIP, necessary for submission of the MAA. The Company currently expects to submit the MAA to the EMA for enobosarm 3 mg for the prevention and treatment of muscle wasting in patients with advanced NSCLC treated with platinum plus taxane chemotherapy by the first quarter of 2015, after completion of the Phase 1 studies and acceptance of the PIP by the EMA's Pediatric Committee. In the Company's meeting with the FDA in February 2014 to review and discuss the results of the POWER clinical trials, the Company learned that since data from the two POWER trials failed to meet the primary statistical criterion pre-specified for the co-primary endpoints of lean body mass and physical function, the FDA was not willing to accept a new drug application ("NDA") for enobosarm 3 mg. However, based on input from the FDA meeting, the Company believes there is a regulatory path forward for enobosarm 3 mg in the United States, and the Company plans to meet again with the FDA to discuss a potential Phase 3 clinical program for an indication of muscle wasting or cachexia in patients with NSCLC.

The Company is conducting a Phase 2 open label study evaluating enobosarm 9 mg for the treatment of androgen receptor positive and estrogen receptor positive metastatic breast cancer in women who have previously responded to hormonal therapy for the treatment of their metastatic breast cancer. Additionally, the Company is developing GTx-758 (Capesaris®), an oral nonsteroidal selective estrogen receptor alpha agonist, for secondary hormonal therapy in men with castration resistant prostate cancer, and, potentially, as a secondary hormonal treatment for advanced prostate cancer used in combination with androgen deprivation therapy. The Company is presently conducting a Phase 2 clinical trial evaluating GTx-758 as secondary hormonal therapy in men with metastatic and nonmetastatic castration resistant prostate cancer.

2. Significant Accounting Policies

Basis of Presentation

The accompanying financial statements have been prepared in accordance with U.S. generally accepted accounting principles ("U.S. GAAP"). Additionally, GTx operates in one business segment.

Use of Estimates

The preparation of financial statements in conformity with U.S. GAAP 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.

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Cash and Cash Equivalents

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

Short-term Investments

At December 31, 2013 and 2012, short-term investments consisted of Federal Deposit Insurance Corporation ("FDIC") insured certificates of deposit with original maturities of greater than three months and less than one year.

Property and Equipment

Property and equipment is stated at cost. Amortization of leasehold improvements is recognized over the shorter of the estimated useful life of the leasehold improvement or the lease term. Depreciation is computed using the straight-line method over the estimated useful lives as follows:

Laboratory and office equipment	3 to 5 years
Leasehold improvements	3 to 7 years
Furniture and fixtures	5 years
Computer equipment and software	3 years

Fair Value of Financial Instruments

The carrying amounts of the Company's financial instruments, which include cash, cash equivalents, short-term investments, and accounts payable approximate their fair values. As the Company has the positive intent and ability to hold the certificates of deposit classified as short-term investments until maturity, these investments have been classified as held to maturity investments and are stated at cost, which approximates fair value. The Company considers these to be Level 2 investments as the fair values of these investments are determined using third-party pricing sources, which generally utilize observable inputs, such as interest rates and maturities of similar assets.

Concentration of Risk

Financial instruments that potentially subject the Company to a concentration of credit risk consist of cash and cash equivalents and short-term investments. The Company has established guidelines relating to diversification and maturities of its cash equivalents and short-term investments which are designed to manage risk. The Company's cash and cash equivalents consist of bank deposits, certificates of deposit, and money market mutual funds. Bank deposits may at times be in excess of FDIC insurance limits. The Company's short-term investments consist of FDIC insured certificates of deposit with original maturities of greater than three months and less than one year.

Research and Development Expenses

Research and development expenses include, but are not limited to, the Company's expenses for personnel, supplies, and facilities associated with research activities, screening and identification of product candidates, formulation and synthesis activities, manufacturing, preclinical studies, toxicology studies, clinical trials, regulatory and medical affairs activities, quality assurance activities and license fees. The Company expenses these costs in the period in which they are incurred. The Company estimates its liabilities for research and development expenses in order to match the recognition of expenses to the period in which the actual services are received. As such, accrued liabilities related to third party research and development activities are recognized based upon the Company's estimate of services received and degree of completion of the services in accordance with the specific third party contract. As a result of the October 2013 reduction in its workforce, the Company is no longer conducting drug

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discovery activities and is focusing its research and development activities on the ongoing clinical development of the Company's current product candidates. See Note 13, *Restructuring*, for further discussion.

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. Accordingly, at December 31, 2013 and December 31, 2012, net of the valuation allowance, the net deferred tax assets were reduced to zero. See Note 9, *Income Taxes*, for further discussion.

The Company has recognized the tax effect of discontinued operations in the statements of operations in accordance with the intra-period accounting rules. An offsetting tax benefit was recorded in continuing operations in each year in which tax expense was recognized for discontinued operations.

Share-Based Compensation

The Company has stock option and equity incentive plans that provide for the purchase or acquisition of the Company's common stock by certain of the Company's employees and non-employee directors. The Company recognizes compensation expense for its share-based payments based on the fair value of the awards over the period during which an employee or non-employee director is required to provide service in exchange for the award. See Note 3, *Share-Based Compensation*, for further discussion.

Other Income (Expense), Net

Other income (expense), net consists of foreign currency transaction gains and losses, interest earned on the Company's cash, cash equivalents and short-term investments, interest expense, and other non-operating income or expense. Additionally, other income (expense), net for the year ended December 31, 2013 included a gain of $1,366 from the sale of research and development property and equipment sold subsequent to the workforce reduction that occurred in October 2013. See Note 4, *Property and Equipment, Net*, and Note 13, *Restructuring*, for further discussion.

Basic and Diluted Net Loss Per Share

Basic and diluted 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 gives effect to the dilutive potential of common stock consisting of stock options and unvested restricted stock units ("RSUs").

Weighted average potential shares of common stock of 6,773,394, 5,574,915, and 5,327,752 were excluded from the calculation of diluted net loss per share for the years ended December 31, 2013, 2012 and 2011, respectively, as inclusion of the potential shares would have had an anti-dilutive effect on the net loss per share for the periods. At December 31, 2013, the Company had outstanding 63,185,389 shares of common stock.

Comprehensive Loss

For all periods presented, there were no differences between net loss and comprehensive loss.

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

Discontinued Operations

Effective September 30, 2012, the Company entered into an asset purchase agreement (the "FARESTON® Purchase Agreement") with Strakan International S.á r.l., an affiliate of ProStrakan Group plc ("ProStrakan") pursuant to which the Company agreed to transfer, sell and assign to ProStrakan all of the Company's rights and certain assets related to FARESTON®. The Company has accounted for FARESTON® as a discontinued operation. As a result, revenue, cost of goods sold, and operating expenses related to FARESTON® were excluded from the respective captions in the statement of operations and were included in discontinued operations for the years ended December 31, 2012 and 2011. See Note 14, *Discontinued Operations*, for further discussion.

FARESTON® Revenue Recognition

Revenue from product sales of FARESTON®, which is included in income from discontinued operations before income taxes for the years ended December 31, 2012 and 2011, was recognized less deductions for estimated sales discounts and sales returns. Revenue from product sales was recognized when persuasive evidence of an arrangement existed, title passed, the price was fixed or determinable, and collectability was reasonably assured. The Company accounted for rebates to certain governmental agencies as a reduction of product sales. The Company allows customers to return product within a specified time period prior to and subsequent to the product's labeled expiration date. Although the Company sold its rights and certain assets related to FARESTON® effective September 30, 2012, the Company retains the liability for future product returns relating to sales of FARESTON® made by the Company prior to September 30, 2012. Therefore, the Company estimates an accrual for product returns based on factors which include historical product returns and estimated product in the distribution channel which is expected to exceed its expiration date. At December 31, 2013 and December 31, 2012, the Company's accrual for product returns, was $918 and $1,189, respectively. Of these amounts, $332 and $370 have been included in "Other long-term liabilities" in the balance sheet at December 31, 2013 and December 31, 2012, respectively, and represents the portion of the Company's product returns accrual estimated to be payable after one year. See Note 14, *Discontinued Operations*, for further discussion.

Collaboration Revenue Recognition

Collaboration revenue for the year ended December 31, 2011 consisted of non-refundable upfront payments and license fees associated with the Company's former collaboration and license agreement with Ipsen Biopharm Limited. Revenues from the prior collaboration and license agreement were recognized based on the performance requirements of the agreement. The Company analyzed the agreement, which included multiple element arrangements,

to determine whether the deliverables under the agreement, including license and performance obligations such as joint steering committee participation and research and development activities, could have been separated or whether all of the deliverables must have been accounted for as a single unit of accounting. See Note 7, *Collaboration and License Agreements*, for further discussion.

Impairment of Long-Lived Assets

The Company reviews long-lived assets for impairment whenever events or changes in facts and circumstances are present, both internally and externally, that may indicate impairment of long-lived assets. An impairment loss is recognized when estimated future cash flows are less than the carrying amount. The cash flow estimates are based on management's best estimates, using appropriate and customary assumptions and projections at the time. See Note 8, *Intangible Assets, Net* for further discussion.

Subsequent Events

The Company has evaluated all events or transactions that occurred after December 31, 2013 up through the date the financial statements were issued. Other than as set forth below, there were no material recognizable or nonrecognizable subsequent events during the period evaluated.

On March 6, 2014, the Company completed a private placement of units consisting of an aggregate of 11,976,048 shares of common stock and warrants to purchase an aggregate of 10,179,642 shares of our common

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

stock per unit for gross proceeds of $21,272. The warrants, which have a one year term, have a per share exercise price of $1.67 that is payable only in cash. Pursuant to the terms of a registration rights agreement dated March 6, 2014 that the Company entered into with the investors, the Company agreed to file a registration statement under the Securities Act registering the resale of all 22,155,690 shares held by or issuable to the investors. No underwriting discounts or commissions or similar fees were payable in connection with the issuance.

3. Share-Based Compensation

Share-based payments include stock option grants and, beginning in October 2013, RSUs under the Company's stock option and equity incentive plans and deferred compensation arrangements for the Company's non-employee directors.

The Company has granted and continues to grant to employees and non-employee directors options to purchase common stock under various plans, including the GTx, Inc. 2013 Equity Incentive Plan (the "2013 EIP") and the GTx, Inc. 2013 Non-Employee Director Equity Incentive Plan (the "2013 NEDEIP"). On May 2, 2013, the Company's stockholders approved the 2013 EIP and the 2013 NEDEIP, each of which became effective on that date. The 2013 EIP is the successor to the Company's 2004 Equity Incentive Plan (the "2004 EIP"), and the 2013 NEDEIP is the successor to the Company's Amended and Restated 2004 Non-Employee Directors' Stock Option Plan (the "2004 NEDSOP"). The total number of shares of the Company's common stock available for issuance under the 2013 EIP was initially 4,208,157 shares plus up to an additional 6,093,559 shares subject to outstanding awards granted under the 2004 EIP and each of the Genotherapeutics, Inc. Stock Option Plan, the GTx, Inc. 2000 Stock Option Plan, the GTx, Inc. 2001 Stock Option Plan and the GTx, Inc. 2002 Stock Option Plan (collectively, the "Prior Plans") that, from and after the effective date of the 2013 EIP, expire or terminate for any reason prior to exercise or settlement, are forfeited because of the failure to vest in those shares or are otherwise returned to the 2013 EIP share reserve pursuant to the terms of the plan. In addition, the shares of the Company's common stock available for issuance under the 2013 EIP will automatically increase on January 1st of each year, for ten years, commencing on January 1, 2014, in an amount equal to 4% of the total number of shares of the Company's common stock outstanding on December 31 of the preceding calendar year, or such lesser (or no) amount as may be approved by the Company's Board of Directors. The total number of shares of the Company's common stock available for issuance under the 2013 NEDEIP was initially 404,000 shares plus up to an additional 449,667 shares subject to outstanding awards granted under the 2004 NEDSOP that, from and after the effective date of the 2013 NEDEIP, expire or terminate for any reason prior to exercise or settlement, are forfeited because of the failure to vest in those shares or are otherwise returned to the 2013 NEDEIP share reserve pursuant to the terms of the plan. In addition, the shares of the Company's common stock available for issuance under the 2013 NEDEIP will automatically increase on January 1st of each year, for ten years, commencing on January 1, 2014, in an amount equal to the lesser of 1% of the total number of shares of the Company's common stock outstanding on December 31 of the preceding calendar year and 500,000 shares, or such lesser (or no) amount as may be approved by the Company's Board of Directors. From and after the effective date of 2013 EIP and the 2013 NEDEIP, no further awards will be made under the Prior Plans and the 2004 NEDSOP. Stock options previously granted under the Prior Plans and the 2004 NEDSOP continue to be governed by the terms of the applicable plan. The 2013 EIP provides for the grant of incentive stock options, nonstatutory stock options, stock appreciation rights, restricted stock awards, RSU awards, other stock awards, and performance awards that may be settled in cash, stock, or other property. The 2013 NEDEIP provides for the grant of nonstatutory stock options, stock appreciation rights, restricted stock awards, RSU awards, other stock awards, and performance awards that may be settled in cash, stock, or other property.

Options granted under the 2013 EIP and the 2013 NEDEIP, and prior to May 2, 2013, the Prior Plans and the 2004 NEDSOP, are granted at prices equal to the fair market value of the stock on the dates the options are granted as determined in accordance with the terms of the applicable plan. The options have a term of ten years from the grant date and generally vest over three years from the grant date for director options and over periods of up to five years from the grant date for employee options. Under the terms of the Company's stock option and equity incentive plans, employees generally have three months after the employment relationship ends to exercise all vested options except in the case of voluntary retirement, disability or death, where post-termination exercise periods are generally longer. As described below, however, certain of the Company's outstanding options were modified to provide an extended post-termination exercise period. The Company issues new shares of common stock upon the exercise of options. The Company estimates the fair value of certain stock option awards as of the

date of the grant by applying the Black-Scholes-Merton option pricing valuation model. The application of this valuation model involves assumptions that are judgmental and highly sensitive in the determination of compensation expense.

The fair value of each option is amortized into compensation expense on a straight-line basis between the grant date for the award and each vesting date. The amount of share-based compensation expense recognized is reduced ratably over the vesting period by an estimate of the percentage of options granted that are expected to be forfeited or canceled before becoming fully vested.

The Company estimates the fair value of RSUs using the closing price of its stock on the grant date. The fair value of RSUs is amortized on a straight-line basis over the requisite service period of the awards.

As part of the October 2013 workforce reduction, the Company modified stock options of the terminated employees to accelerate the vesting of certain outstanding non-vested stock options and to extend the post-termination exercise period of their vested stock options. The terminated employees' stock options were modified to accelerate the vesting of all outstanding and unvested stock options as if an employee had remained in continuous service as an employee of the Company through January 1, 2014. Further, the Company extended the post-termination exercise period of each terminated employee's outstanding and vested options until the earliest to occur of (i) June 1, 2014, (ii) the expiration of the original term of the particular option, and (iii) a change of control of the Company (as defined in the applicable form of award agreement).

As part of the Company's efforts to retain the essential employees continuing with the Company following the October 2013 workforce reduction, the Company modified certain stock options held by continuing employees to accelerate the vesting of certain outstanding non-vested stock options and to extend the post-termination exercise period of vested stock options. For these continuing employees, each of their outstanding stock options was modified to provide that if the employee's service continues through the earlier to occur of (i) the end of business on May 31, 2014, and (ii) an involuntary termination of employment by the Company (excluding a termination for cause (as defined in the applicable form of retention benefits agreement) or a voluntary resignation) (the "Determination Date"), then, as of the Determination Date: (i) an additional number of shares subject to such option will immediately vest as if the employee's service had continued through January 1, 2015 and (ii) the period during which the vested portion of such options will generally expire will be extended from 90 days to six months after the employee's termination of service, subject in each case to the earlier expiration of the original term of the applicable stock option award.

Additionally, effective October 1, 2013, the Compensation Committee approved a grant of stock options and RSUs under the 2013 EIP to the continuing employees. Both the options granted and the RSUs vest in full on the earlier to occur of (i) June 1, 2014, (ii) an involuntary termination of the award holder's continuous service by the Company other than for cause (as defined in the applicable form of award agreement) and (iii) a change of control of GTx (as defined in the applicable form of award agreement), except that vesting will not occur in the event of a voluntary resignation or involuntary termination for cause occurring prior to any of these events.

The following table summarizes share-based compensation expense included within the statements of operations for each of the three years in the period ended December 31, 2013:

	Years Ended December 31,		
	2013	2012	2011
Research and development expenses	$ 1,875	$ 1,046	$ 1,972
General and administrative expenses	1,993	1,771	2,432
Total share-based compensation	$ 3,868	$ 2,817	$ 4,404

Share-based compensation expense recorded in the statement of operations as general and administrative expense for the years ended December 31, 2013, 2012 and 2011 included share-based compensation expense related to deferred compensation arrangements for the Company's non-employee directors of $135, $169 and $178,

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respectively. See Note 10, *Directors' Deferred Compensation Plan*, for further discussion of deferred compensation arrangements for the Company's non-employee directors.

As a result of the October 2013 modifications of certain stock options held by terminated employees, the Company recognized a net benefit of approximately $370 resulting from the reversal of previously recognized share-based compensation expense that was in excess of the modified fair value of the options. Of this amount, $81 was included as a benefit to general and administrative expenses and $289 was included as a benefit to research and development expenses for the year ended December 31, 2013. Additionally, share-based compensation expense for the year ended December 31, 2013 included expense related to the amortization of RSUs which were granted in the fourth quarter of 2013. The modifications of certain stock options held by the Company's continuing employees did not have a material impact on share-based compensation expense recognized during the period.

Share-based compensation expense recorded as research and development expenses for the year ended December 31, 2012 was reduced by the reversal of previously recognized share-based compensation expense for non-vested stock options that were canceled in conjunction with the resignation of an executive officer during the year.

As part of a June 2011 workforce reduction, the Company modified certain stock options of three terminated non-executive officers to accelerate the vesting of certain outstanding non-vested stock options and to extend the post-termination exercise period of their vested stock options. As a result of these

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modifications, the Company incurred a one-time share-based compensation charge of $481, which was included in general and administrative expenses for the year ended December 31, 2011. This charge was offset by the reversal of $704 of previously recognized share-based compensation expense for non-vested stock options that were canceled in conjunction with the total workforce reduction. Of this amount, $646 was included in general and administrative expenses and $58 was included in research and development expenses for the year ended December 31, 2011.

Additionally, share-based compensation expense of $137 included in the table above as general and administrative expenses was reported as discontinued operations in the statement of operations for the year ended December 31, 2011. There was no share-based compensation expense included in discontinued operations for the years ended December 31, 2013 or 2012.

For the years ended December 31, 2013, 2012 and 2011, the weighted average grant date fair value per share of options granted was $2.13, $2.14 and $1.75, respectively. The weighted average for key assumptions used in determining the grant date fair value of options granted in 2013, 2012 and 2011, and a summary of the methodology applied to develop each assumption is as follows:

	Years Ended December 31,		
	2013	2012	2011
Expected price volatility	82.9%	69.6%	64.9%
Risk-free interest rate	1.27%	1.22%	2.54%
Weighted average expected life in years	5.9 years	6.5 years	6.5 years
Dividend yield	0%	0%	0%

Expected Price Volatility - This is a measure of the amount by which a price has fluctuated or is expected to fluctuate. The Company based its determination of expected volatility on its historical stock price volatility. An increase in the expected price volatility will increase compensation expense.

Risk-Free Interest Rate - This is determined using U.S. Treasury rates where the term is consistent with the expected life of the stock options. An increase in the risk-free interest rate will increase compensation expense.

Expected Life - This is the period of time over which the options granted are expected to remain outstanding and is determined by calculating the average of the vesting term and the contractual term of the options. The Company has utilized this method due to the lack of historical option exercise information related to the Company's stock option and equity incentive plans. Options granted have a maximum term of ten years. An increase in the expected life will increase compensation expense.

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Dividend Yield - The Company has not made any dividend payments nor does it have plans to pay dividends in the foreseeable future. An increase in the dividend yield will decrease compensation expense.

The following is a summary of stock option transactions for all of the Company's stock option and equity incentive plans for the three year period ended December 31, 2013:

	Number of Shares	Weighted Average Exercise Price Per Share
Options outstanding at January 1, 2011	4,430,495	10.91
Options granted	1,395,000	2.82
Options forfeited or expired	(866,930)	8.23
Options exercised	(13,000)	4.20
Options outstanding at December 31, 2011	4,945,565	9.12
Options granted	1,141,250	3.35
Options forfeited or expired	(675,755)	8.82
Options exercised	(28,201)	3.05
Options outstanding at December 31, 2012	5,382,859	7.96
Options granted	2,784,200	3.12
Options forfeited or expired	(1,400,419)	5.86
Options exercised	(321,298)	3.81
Options outstanding at December 31, 2013	6,445,342	6.58
Options vested and expected to vest at December 31, 2013	6,378,284	6.61

The following table summarizes information about stock options outstanding at December 31, 2013:

	Options Outstanding			Options Exercisable	
Exercise Price	Number Outstanding	Weighted Average Remaining Contractual Life (years)	Weighted Average Exercise Price	Number Exercisable	Weighted Average Exercise Price
$1.51 - $3.36	2,662,100	7.79	$ 2.49	642,501	$ 2.96
$3.44 - $9.71	2,150,947	5.77	4.88	1,023,482	5.58
$9.80 - $20.40	1,632,295	2.63	15.48	1,515,137	15.43
	6,445,342	5.81	6.58	3,181,120	9.74

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At December 31, 2013, the aggregate intrinsic value of all outstanding options was less than $1 with a weighted average remaining contractual term of 5.81 years. Of the Company's outstanding options, 3,181,120 options were exercisable and had a weighted average remaining contractual term of 2.92 years and no aggregate intrinsic value. Additionally, the Company's vested and expected to vest options had a weighted average remaining contractual term of 5.78 years and an intrinsic value of less than $1.

Options to purchase 321,298 shares were exercised during the year ended December 31, 2013. The total intrinsic value of options exercised during the years ended December 31, 2013, 2012 and 2011 was $688, $36 and $15, respectively. At December 31, 2013, the total compensation cost related to non-vested options not yet recognized was $4,390, with a weighted average expense recognition period of 1.97 years. Shares available for future issuance under the Company's stock option and equity incentive plans were 3,239,443 at December 31, 2013. On January 1, 2014, shares available for future issuance under the 2013 EIP and 2013 NEDEIP increased by an aggregate of 3,027,416 shares in accordance with the automatic increase provisions of such plans.

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The following is a summary of the Company's RSUs for the year ended December 31, 2013:

	Number of Shares	Weighted Average Grant Date Fair Value Per Share
Unvested RSUs at January 1, 2013	—	—
RSUs granted	1,325,000	1.87
RSUs vested	—	—
RSUs forfeited	(100,000)	1.88
Unvested RSUs at December 31, 2013 2013	1,225,000	1.87

At December 31, 2013, the total compensation cost related to non-vested RSUs not yet recognized was $1,443, with a weighted average expense recognition period of five months.

4. Property and Equipment, Net

Property and equipment, net consisted of the following:

	December 31,	
	2013	2012
Computer equipment and software	$ 2,130	$ 4,177
Furniture and fixtures	1,032	2,706
Leasehold improvements	355	1,361
Office and laboratory equipment	261	1,024
	3,778	9,268
Less: accumulated depreciation	(3,666)	(8,761)
	$ 112	$ 507

Depreciation and amortization expense for the years ended December 31, 2013, 2012 and 2011 was $369, $730 and $998, respectively. Of these amounts, $169, $290 and $425, respectively, were included in research and development expenses in the statements of operations.

Subsequent to the reduction in workforce implemented in October 2013 and determination to cease drug discovery activities, the Company sold its related property and equipment and recognized a gain of $1,366 during the year ended December 31, 2013. The carrying value associated with the property and equipment that was sold was $58 and related to laboratory equipment. See Note 13, *Restructuring*, for further discussion.

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5. Accrued Expenses and Other Current Liabilities

Accrued expenses and other current liabilities consisted of the following:

	December 31,	
	2013	2012
Employee compensation	$ 1,354	$ 294
Clinical trials	1,127	5,621
Product returns	586	819
Selling, general and administrative	497	737
Research and development	39	46

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Net deferred income tax liabilities	156	271
	$ 3,759	$ 7,788

6. Common and Preferred Stock

The Company's certificate of incorporation authorizes the Company to issue 120,000,000 shares of common stock, $0.001 par value per share, and 5,000,000 shares of preferred stock, $0.001 par value per share.

On November 1, 2010, the Company completed an underwritten public offering of 14,285,715 shares of its common stock at a price to the public of $2.80 per share. Net cash proceeds from the public offering were approximately $37,700 after deducting underwriting discounts and commissions and other offering expenses. The Company also granted the underwriter a 30-day option to purchase up to an additional 2,142,857 shares of common stock to cover over-allotments, if any. On November 24, 2010, the underwriter exercised its option and purchased an additional 1,000,000 shares of the Company's common stock at a price of $2.80 per share. Net cash proceeds from the exercise of the over-allotment option were approximately $2,600 after deducting underwriting discounts and commissions and other offering expenses.

On May 6, 2011, the Company filed a Certificate of Amendment to the Company's Restated Certificate of Incorporation with the Secretary of the State of Delaware to increase the number of authorized shares of the Company's common stock, par value $0.001 per share, from 60,000,000 shares to 120,000,000 shares. The amendment was approved by the Company's stockholders at the Company's 2011 Annual Meeting of Stockholders held on May 5, 2011.

On June 28, 2011, the Company completed an underwritten public offering of 10,000,000 shares of its common stock at a price to the public of $4.75 per share. The Company also granted the underwriters a 30-day option to purchase up to an additional 1,500,000 shares of common stock to cover over-allotments, if any. The underwriters exercised this option and purchased an additional 1,023,000 shares of the Company's common stock on June 28, 2011 at a price of $4.75 per share. Net cash proceeds from the public offering were approximately $49,000 after deducting the underwriting discount and offering expenses.

7. Collaboration and License Agreements

University of Tennessee Research Foundation License Agreement

The Company and the University of Tennessee Research Foundation ("UTRF") are parties to a consolidated, amended and restated license agreement (the "SARM License Agreement") pursuant to which the Company has been granted exclusive worldwide rights in all existing SARM technologies owned or controlled by UTRF, including all improvements thereto, and exclusive rights to future SARM technology that may be developed by certain scientists at the University of Tennessee or subsequently licensed to UTRF under certain existing inter-institutional agreements with The Ohio State University. Under the SARM License Agreement, the Company is obligated to pay UTRF annual license maintenance fees, low single-digit royalties on net sales of products and mid single-digit royalties on sublicense revenues.

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Former Orion Corporation License and Supply Agreement

In connection with the Company's sale of its rights and certain assets related to FARESTON® to ProStrakan, the Company and Orion agreed to terminate the Amended and Restated License and Supply Agreement, dated January 1, 2005, as amended, between the Company and Orion (the "Orion Supply Agreement") as well as certain other agreements between the Company and Orion related to the Orion Supply Agreement (collectively, the "Orion Agreements"). Pursuant to the Orion Supply Agreement, the Company obtained an exclusive license from Orion to develop and commercialize toremifene-based products for all human indications worldwide, except breast cancer outside of the United States, and Orion agreed to manufacture and supply all of the Company's needs for clinical trial and commercial grade material for toremifene-based products developed and marketed in the United States and abroad, including toremifene globally and FARESTON® in the United States. The termination of the Orion Agreements was effective September 30, 2012. As consideration for Orion's agreement to terminate the Orion Agreements and to enter into certain agreements with ProStrakan to effect the FARESTON® sale, the Company paid Orion $1,000 in October 2012. See Note 14, *Discontinued Operations*, for further discussion.

Former Ipsen Collaboration and License Agreement

In September 2006, the Company entered into a collaboration and license agreement with Ipsen Biopharm Limited (the "Ipsen Collaboration Agreement") pursuant to which the Company granted Ipsen exclusive rights in the European Union, Switzerland, Norway, Iceland, Lichtenstein, and the Commonwealth of Independent States (the "European Territory") to develop and commercialize toremifene in all indications which the Company has licensed from Orion, which include all indications in humans except the treatment and prevention of breast cancer outside of the United States. In accordance with the terms of the Ipsen Collaboration Agreement, Ipsen paid the Company €23,000 as a license fee and expense reimbursement. Under the Ipsen Collaboration Agreement, the Company recorded deferred revenue of $29,330 related to the Ipsen upfront license fee and expense reimbursement which was being amortized into revenue on a straight-line basis over the estimated ten year development period for toremifene in the European Territory.

In March 2011, the Company reacquired full rights to its toremifene program following the termination by the Company and Ipsen of the collaboration and license agreement, as amended. During the first quarter of 2011, the Company recognized as collaboration revenue all of the remaining $8,066 unamortized revenue. This amount is included in collaboration revenue in the statement of operations for the year ended December 31, 2011.

8. Intangible Assets, Net

In accordance with the terms of the former Orion Supply Agreement, the Company paid a license fee to Orion of $4,826. In accordance with the terms of the SARM License Agreement that the Company entered into with UTRF in July 2007, the Company paid a one-time up-front fee of $290.

In 2011 after discontinuing the toremifene 80 mg development program, the Company recorded an impairment charge of $1,598. The impaired intangible asset consisted of capitalized license fees related to the Company's toremifene 80 mg program paid under the Orion Supply Agreement. The impairment charge was included in research and development expenses in the statement of operations for the year ended December 31, 2011.

The Company's remaining intangible asset, net at December 31, 2013 and 2012 consisted of $166 and $181, respectively, related to the SARM License Agreement.

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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 tax assets and liabilities consisted of the following:

	December 31,	
	2013	2012
Deferred income tax assets, net:		
Net federal and state operating loss carryforwards	$ 127,819	$ 112,233
Research and development credits	11,934	9,799
Share-based compensation	8,670	8,852
Depreciation and amortization	170	331
Other, net	268	328
Total deferred tax assets, net	148,861	131,543
Valuation allowance	(148,861)	(131,543)
Net deferred tax assets and liabilities	$ —	$ —

Realization of deferred income tax assets is dependent upon future earnings, if any, the timing and amount of which are uncertain. Accordingly, due to the Company's history of net operating losses, the net deferred tax assets have been fully offset by a valuation allowance. The valuation allowance increased by $17,318, $8,836 and $13,218 in 2013, 2012, and 2011, respectively.

At December 31, 2013, the Company had net federal operating loss carryforwards of approximately $329,400, which expire from 2018 to 2033 if not utilized. The Company had state operating loss carryforwards of approximately $310,451, which expire from 2014 to 2033 if not utilized. The Company also had research and development credits at December 31, 2013 of approximately $11,934, which expire from 2020 to 2033 if not utilized.

Both of the net federal and state operating loss carryforwards include approximately $2,301 of deductions related to the exercise of stock options. This amount represents an excess tax benefit and has not been included in the gross deferred income tax asset reflected for net federal and state operating loss carryforwards. If utilized, the benefits from these deductions will be recorded as an adjustment to additional paid in capital.

The Company will recognize the impact of a tax position in the financial statements if that position is more likely than not of being sustained on audit based on the technical merits of the position. As of December 31, 2013, the Company had no unrecognized tax benefits. Utilization of the Company's net operating loss carryforwards may be subject to a substantial annual limitation due to ownership change limitations provided by Section 382 of the Internal Revenue Code of 1986, as amended, and similar state provisions. The annual limitations may result in the expiration of net operating loss carryforwards before utilization. The Company completed a study of its net operating losses through December 31, 2013 to determine whether such amounts are likely to be limited by Section 382. As a result of this study, the Company does not currently believe any Section 382 limitation exists through December 31, 2013. However, any future ownership changes under Section 382 may limit our ability to fully utilize these tax benefits. The Company has not yet conducted an in depth study of its research and development credits, although the Company periodically reviews assumptions in its calculations to reflect its best estimate of expected credit. The Company reduced the cumulative eligible credit by $1,122 as a result of its review during the year ended December 31, 2012. An in depth study may result in an increase or decrease to the Company's research and development credits and until such study is conducted of the Company's research and development credits, no amounts are being presented as an uncertain tax position. The Company's net deferred income tax assets have been fully offset by a valuation allowance. Therefore, future changes to the Company's unrecognized tax benefits would be offset by an adjustment to the valuation allowance and there would be no impact on the Company's balance sheet, statement of operations, or cash flows. The Company does not expect its unrecognized tax benefits to change significantly over the next 12 months.

The Company is currently open to audit under the statute of limitations by the Internal Revenue Service and the appropriate state income taxing authorities for all years due to the net loss carryforwards from those years. The Company is currently not under examination by the Internal Revenue Service or any other taxing authorities. The Company has not recorded any interest and penalties on any unrecognized tax benefits since its inception.

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The Company has recognized the tax effect of discontinued operations in the statements of operations for the years ended December 31, 2012 and 2011 in accordance with the intra-period accounting rules. An offsetting tax benefit is recorded in continuing operations in each year in which tax expense was

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recognized for discontinued operations.

10. Directors' Deferred Compensation Plan

Non-employee directors may defer all or a portion of their fees under the Company's Directors' Deferred Compensation Plan until termination of their status as directors. Deferrals can be made into a cash account, a stock account, or a combination of both. Stock accounts will be paid out in the form of Company common stock, except that any fractional shares will be paid out in cash valued at the then current market price of the Company's common stock. Cash accounts and stock accounts under the Directors' Deferred Compensation Plan are credited with interest or the value of any cash and stock dividends, respectively. Non-employee directors are fully vested in any amounts that they elect to defer under the Directors' Deferred Compensation Plan.

For the years ended December 31, 2013, 2012 and 2011, the Company incurred non-employee director fee expense of $259, $237 and $260, respectively, of which $135, $169 and $178 was deferred into stock accounts and will be paid in common stock following separation from service as a director. At December 31, 2013, 169,384 shares of the Company's common stock had been credited to individual director stock accounts under the Directors' Deferred Compensation Plan, and no amounts had been credited to individual director cash accounts under the Directors' Deferred Compensation Plan.

11. 401(k) Plan

The Company sponsors a 401(k) retirement savings plan that is available to all eligible employees. 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 $17.5 for employees under age 50 and $23 for employees 50 and older in calendar year 2013. 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. The Company elected to match a portion of employee's contributions to the plan in the amount of $338, $363 and $388 in 2013, 2012 and 2011, respectively.

12. Commitments and Contingencies

Operating Lease Commitments

The Company previously leased laboratory facilities and office space pursuant to a sublease, which had been accounted for as an operating lease. Subsequent to the reduction in force implemented in October 2013, this lease was cancelled effective December 31, 2013. See Note 13, *Restructuring*, for further discussion. The Company subleases office space under a sublease that is accounted for as an operating lease. This sublease has escalating rent payments and expires on April 30, 2015. Total rent expense under the operating leases was approximately $674, $963 and $933 for the years ended December 31, 2013, 2012 and 2011, respectively.

As of December 31, 2013, annual minimum payments under operating lease arrangements were as follows:

2014	$	553
2015		185
Total	$	738

13. Restructuring

In October 2013, the Company implemented a reduction in its workforce following the announced results from its two Phase 3 clinical trials evaluating enobosarm 3 mg for the prevention and treatment of muscle wasting in patients with advanced NSCLC. The reduction in force was effective immediately and represented approximately 60% of the Company's total workforce.

As a result of the workforce reduction, the Company incurred severance related cash expenses of $1,306, of which $351 was included in general and administrative expenses and $955 was included in research and development expenses for the year ended December 31, 2013. All of these expenses have been paid as of December 31, 2013. Additionally, the Company recognized a net benefit of approximately $370 resulting from the reversal of share-based compensation expense related to the modification of certain stock option provisions for the severed employees. Of this amount, $81 was included as a benefit to general and administrative expenses and $289 was included as a benefit to research and development expenses for the year ended December 31, 2013.

As a result of the October 2013 reduction in its workforce, the Company is no longer conducting drug discovery activities. During the fourth quarter of 2013, the Company cancelled its sublease for the laboratory facilities and office space utilized for drug discovery. Additionally, the Company sold its related property and equipment for a gain of $1,366, which was included in other income (expense), net for the year ended December 31, 2013.

14. Discontinued Operations

On September 28, 2012, the Company entered into the FARESTON® Purchase Agreement with ProStrakan pursuant to which the Company agreed to transfer, sell and assign to ProStrakan all of the Company's rights to FARESTON® and certain assets related thereto. Effective September 30, 2012, the Company completed the sale of FARESTON® pursuant to the FARESTON® Purchase Agreement for a total cash purchase price of $21,671, including payment for purchased inventory. The Company recognized a gain of $18,831 on the sale of FARESTON® for the year ended December 31, 2012. The gain represents the gross proceeds received from the sale reduced by a contract termination fee of $1,000 due to Orion (as discussed further in Note 7, *Collaboration and License Agreements)*, a financial advisory fee related to the transaction of $1,712, and other transaction expenses of approximately $128.

The Company has accounted for FARESTON® as a discontinued operation. The FARESTON® operating income for the years ended December 31, 2012 and 2011, along with the gain recognized on the sale of FARESTON® for the year ended December 31, 2012, has been reported in "net income from discontinued operations" in the statements of operations.

FARESTON® operating income for each period presented was as follows:

	Years Ended December	
	2012	2011
Product sales, net	$ 5,284	$ 6,673
Cost of product sales	(784)	(1,055)
Operating expenses	(655)	(3,411)
FARESTON® operating income	$ 3,845	$ 2,207

Under the FARESTON® Purchase Agreement, the Company remains liable for future product returns relating to sales of FARESTON® made by the Company prior to September 30, 2012. At December 31, 2013, liabilities related to FARESTON® discontinued operations were $918 and consisted of the Company's accrual for product returns. At December 31, 2012, total assets related to FARESTON® discontinued operations were $14 and consisted of accounts receivable, prepaid expenses and other assets. At December 31, 2012, the total liabilities related to FARESTON® discontinued operations were $1,398 and consisted of accounts payable and other liabilities and the accrual for product returns of $1,189.

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

15. Quarterly Financial Data (Unaudited)

The following is a summary of the quarterly results of operations for the years ended December 31, 2013 and 2012:

	2013 Quarters Ended			
	March 31	June 30	September 30	December 31
Expenses:				
Research and development expenses	$ 9,614	$ 10,139	$ 6,477	$ 6,088
General and administrative expenses	3,023	2,684	2,483	3,091
Total expenses	12,637	12,823	8,960	9,179
Loss from operations	(12,637)	(12,823)	(8,960)	(9,179)
Other income, net	55	21	23	1,389
Net loss	$ (12,582)	$ (12,802)	$ (8,937)	$ (7,790)
Net loss per share — basic and diluted	$ (0.20)	$ (0.20)	$ (0.14)	$ (0.12)
Weighted average shares outstanding:				
Basic and diluted	62,864,140	62,994,771	63,179,394	63,185,389

	2012 Quarters Ended			
	March 31	June 30	September 30	December 31
Expenses:				
Research and development expenses	$ 9,835	$ 9,237	$ 9,764	$ 10,051
General and administrative expenses	2,588	2,400	2,999	2,858
Total expenses	12,423	11,637	12,763	12,909
Loss from operations	(12,423)	(11,637)	(12,763)	(12,909)
Other income (expense), net	8	53	(47)	(33)
Loss from operations before income taxes	(12,415)	(11,584)	(12,810)	(12,942)
Income tax benefit	381	355	5,812	2,273
Net loss from continuing operations	(12,034)	(11,229)	(6,998)	(10,669)
Income (loss) from discontinued operations before income taxes	1,335	1,203	20,214	(76)
Income tax (expense) benefit	(381)	(355)	(8,115)	30
Net income (loss) from discontinued operations	954	848	12,099	(46)
Net (loss) income	$ (11,080)	$ (10,381)	$ 5,101	$ (10,715)
Net (loss) income per share — basic and diluted:				
Net loss from continuing operations	$ (0.19)	$ (0.18)	$ (0.11)	$ (0.17)
Net income from discontinued operations	0.01	0.01	0.19	—
Net (loss) income per share	$ (0.18)	$ (0.17)	$ 0.08	$ (0.17)
Weighted average shares outstanding:				
Basic and diluted	62,798,008	62,805,662	62,815,549	62,817,495

GTx, INC.

WAIVER AND AMENDMENT AGREEMENT

THIS WAIVER AND AMENDMENT AGREEMENT (the "Agreement") is made effective as of March 6, 2014 (the "Effective Date"), by and among GTx, INC., a Delaware corporation (the "Company"), and the undersigned Holder (the "Consenting Holder").

RECITALS

WHEREAS, the Company and the Consenting Holder are parties to that certain Amended and Restated Registration Rights Agreement dated as of August 7, 2003 (the "Registration Rights Agreement").

WHEREAS, the Consenting Holder acknowledges that the Company expects to enter into a Registration Rights Agreement, as the same may be amended from time to time (the "New Registration Rights Agreement"), with certain parties named therein (the "New Holders"), in connection with the entry into by the Company and the New Holders of a Securities Purchase Agreement (the "Purchase Agreement"), dated on or about the date hereof, pursuant to which the Company anticipates selling immediately separable units, which units consist of shares of the Company's Common Stock (the "Common Shares") and warrants exercisable for the Company's Common Stock (the "Warrants," and the shares of the Company's Common Stock issuable upon exercise of the Warrants, the "Warrant Shares"). The Common Shares, together with the Warrant Shares, shall be referred to herein as the "Shares."

WHEREAS, the Consenting Holder acknowledges that pursuant to the terms of the New Registration Rights Agreement, the Company will be obligated to prepare and file one or more registration statements (the "Resale Registration Statements") under the Securities Act of 1933, as amended (the "Securities Act"), registering the resale of the Shares by the New Holders (or any subsequent transferees or assignees thereof), and that the Company will grant to the New Holders certain piggyback registration rights. As used in this Agreement, (i) the term "Shares" also includes any securities issued or issuable with respect to any of the Shares by way of exchange, stock dividend or stock split or in connection with a combination of shares, recapitalization, merger, consolidation or other reorganization or otherwise; and (ii) the term "Resale Registration Statements" includes (A) any registration statements filed by the Company under the Securities Act pursuant to the terms of the New Registration Rights Agreement and (B) any amendments or supplements to any of such registration statements or the prospectuses included therein.

WHEREAS, pursuant the Registration Rights Agreement, the Consenting Holder has under certain circumstances the right to be notified if the Company decides to Register any of its Common Stock and to include certain Registrable Securities held by such Holders in such Registration (and any related qualification under Blue Sky laws or other compliance), and in any underwriting involved therein (the "RRA Piggyback Registration Rights").

WHEREAS, pursuant to Section 8(g) of the Registration Rights Agreement, the Company and the Consenting Holder wish to (i) amend the Registration Rights Agreement as set forth below; and (ii) waive the RRA Piggyback Registration Rights in connection with the filing of the Resale Registration Statements and any offerings made pursuant thereto.

WHEREAS, the Consenting Holder and his Affiliates are holders of at least a majority of the Registrable Securities held by all Holders and, together with the Company, have the right, pursuant to Section 8(g) of the Registration Rights Agreement, to amend the Registration Rights Agreement and to waive certain provisions thereof.

NOW, THEREFORE, in consideration of the mutual agreements, covenants and considerations contained herein, the Company and the Consenting Holder agree as follows:

AGREEMENT

1. WAIVER.

1.1 The Consenting Holder hereby waives (i) any and all RRA Piggyback Registration Rights in connection with the filing of, and any offerings made pursuant to, the Resale Registration Statements and (ii) any rights to any notices with respect to the foregoing under the Registration Rights Agreement.

1.2 The foregoing waiver in Section 1.1 is irrevocable and shall be effective with respect to the Consenting Holder, as well as its affiliates, successors, heirs, executors, administrators and assigns.

2. AMENDMENTS TO REGISTRATION RIGHTS AGREEMENT.

2.1 Defined Terms. Section 1 of the Registration Rights Agreement is hereby amended to add the following definitions to the list of defined terms thereunder, each of which shall read in full as follows:

"RRA Holder" means the holder of the Registrable Securities under this Agreement.

"RRA Registration" means any registration effected pursuant to this Agreement.

"New Registration Rights Agreement" means that certain Registration Rights Agreement, dated as of March 6, 2014, by and between the Company and the parties identified therein, as the same may be amended from time to time in accordance with the terms thereof.

"New Holder" means a holder of New Registrable Securities.

"New Registrable Securities" shall have the same meaning as the meaning ascribed to the term "Registrable Securities" under the New Registration Rights Agreement.

"Piggyback Registrable Securities" means all Registrable Securities under this Agreement and all New Registrable Securities."

"Special Registration Statement" means (i) any registration statement relating to any employee benefit plan, (ii) with respect to any corporate reorganization or transaction under Rule 145, any registration statement related to the issuance or resale of securities issued in such a transaction, (iii) any registration statement related to stock issued upon conversion of debt securities, (iv) any RRA Registration and (v) the first Registration Statement on Form S-3 filed after the date hereof by the Company with the SEC that registers solely a Company primary offering on a continuous basis pursuant to Rule 415.

2.2 Section 8(c) of the Registration Rights Agreement is hereby amended and restated to read in full as follows:

"(c) [Intentionally Omitted]"

2.3 Section 8(d)(i) and (ii) of the Registration Rights Agreement is hereby amended and restated to read in full as follows:

"(i) If the Company determines to prepare and file with the SEC a Registration Statement, but excluding in all cases any Special Registration Statements, relating to an offering for its own account or the account of others of any of its equity securities at any time prior to March 6, 2019 or until such earlier date that no Registrable Securities are outstanding, then the Company shall send to the RRA Holder written notice of such determination and, if within 15 days after the date of such notice, any such Holder shall so request in writing, the Company shall include in such Registration Statement all or any part of the Registrable Securities such Holder requests to be registered, subject to Section 8(d)(ii)"

"(ii) If the Company proposes to register any of its securities under the Securities Act as contemplated by Section 8(d)(i) and such securities are to be distributed in an underwritten offering through one or more underwriters, the Company shall, if requested by any Holders pursuant to Section 8(d)(i), use its reasonable best efforts to arrange for such underwriters to include on the same terms and conditions that apply to the other sellers in such registration all the Registrable Securities to be offered and sold by such Holders among the securities of the Company to be distributed by such underwriters in such registration. If the managing underwriter or underwriters of any proposed underwritten offering including Piggyback Registrable Securities informs the Company and each New Holder and the RRA Holder that, in its or their opinion, the number of securities which the New Holders and the RRA Holder intend to include in such offering exceeds the number which can be sold in such offering without being likely to have a significant adverse effect on the price, timing or distribution of the securities offered or the market for the securities offered, then the securities to be included in such registration shall be allocated *pro rata* among the New Holders and/or RRA Holder that have requested to participate in such registration based on the relative number of Piggyback Registrable Securities requested by each New Holder and/or RRA Holder, as applicable, to be included in such underwritten offering. Notwithstanding anything in this Agreement to the contrary, the provisions of this Section 8(d)(i) may be amended or waived (either generally or in a particular instance, either retroactively or prospectively and either for a specified period of time or indefinitely), with the written consent of (i) the Company and (ii) the holders holding at least sixty percent (60%) of the then outstanding Piggyback Registrable Securities; *provided, however*, that if any such waiver or amendment effected pursuant to this Section 8(d)(i) materially and adversely affects the rights of the New Holders and does not materially and adversely affect the rights of the IRA Holder in the same manner, then such waiver or amendment shall require the consent of the holders of a majority-in-interest of the then outstanding Registrable Securities; *provided further* that if any such waiver or amendment effected pursuant to this Section 8(d)(i) materially and adversely affects the rights of the RRA Holder and does not materially and adversely affect the rights of the Holders in the same manner, then such waiver or amendment shall require the consent of the RRA Holder."

3. MISCELLANEOUS.

3.1 Defined Terms. Capitalized terms not otherwise defined herein shall have the meanings ascribed to them in the Registration Rights Agreement.

3.2 Full Power and Authority. The Consenting Holder represents and warrants to the Company that (i) the Consenting Holder has the full right, power and authority to execute and deliver this Agreement, and (ii) this Agreement has been duly executed and delivered by the Consenting Holder and constitutes the legal, valid and binding obligation of the Consenting Holder enforceable in accordance with its terms, except (A) as such enforcement is limited by bankruptcy, insolvency or other similar laws affecting the enforcement of creditors' rights generally and (B) for limitations imposed by general principles of equity.

3.3 Effect of Agreement. Except as modified by the terms of this Agreement, the terms and provisions of the Registration Rights Agreement shall remain in full force and effect. Other than as stated in this Agreement, this Agreement shall not operate as a waiver of any condition or obligation imposed on the parties under the Registration Rights Agreement. In the event of any conflict, inconsistency, or incongruity between any provision of this Agreement and any provision of the Registration Rights Agreement, the provisions of this Agreement shall

govern and control. This Agreement shall not be changed or modified orally, but only by an instrument in writing signed by the parties hereto.

3.4 Governing Law. This Agreement shall be governed by, and construed in accordance with, the laws of the State of Delaware excluding those laws that direct the application of the laws of another jurisdiction.

3.5 Successors and Assigns. The provisions hereof shall inure to the benefit of, and be binding upon, the successors, assigns, heirs, executors and administrators of the parties hereto, and shall be enforceable by the Company or the Consenting Holder.

3.6 Counterparts. This Agreement may be executed in several counterparts, each of which shall constitute an original and all of which, when taken together, shall constitute one instrument.

3.7 Certain Confidential Information. Certain of the information contained in this Agreement is confidential and has not been publicly disclosed by the Company, including the transactions contemplated by the Purchase Agreement and the contemplated filing of the Resale Registration Statements pursuant to the terms of the New Registration Rights Agreement (the "Confidential Information"). Accordingly, the Consenting Holder agrees to maintain the Confidential Information in confidence until such time as the Confidential Information has been publicly disclosed by the Company.

[Remainder of Page Intentionally Left Blank; Signature Pages Follow]

IN WITNESS WHEREOF, the undersigned have executed this AGREEMENT effective as of the Effective Date.

GTx, INC.

By: /s/ Marc S. Hanover
Name: Marc S. Hanover
Title: President and Chief Operating Officer

SIGNATURE PAGE TO
WAIVER AND AMENDMENT AGREEMENT

IN WITNESS WHEREOF, the undersigned have executed this AGREEMENT effective as of the Effective Date.

J.R. HYDE, III

By: /s/ J.R. Hyde, III

PITTCO ASSOCIATES, L.P.

By: /s/ J.R. Hyde, III
Name: J.R. Hyde, III
Title: Chairman

SIGNATURE PAGE TO
WAIVER AND AMENDMENT AGREEMENT

REGISTRATION RIGHTS AGREEMENT

This REGISTRATION RIGHTS AGREEMENT (the "Agreement") is made as of March 6, 2014 by and among GTx, Inc., a corporation organized and existing under the laws of the State of Delaware (the "Company"),The Pyramid Peak Foundation ("PPF") and J.R. Hyde III ("JRH and together with PPF, the "Principal Purchasers") and the other purchasers named in the Purchase Agreement (as defined below) (collectively, the "Purchasers").

RECITALS

WHEREAS, the Company and the Purchasers are parties to a Securities Purchase Agreement, dated as of March 3, 2014 (the "Purchase Agreement"), pursuant to which the Purchasers are purchasing an aggregate of 11,976,048 immediately separable Units, each Unit consisting of one share of Common Stock and a Warrant (as defined in the Purchase Agreement) to purchase one (1) share of Common Stock; and

WHEREAS, in connection with the consummation of the transactions contemplated by the Purchase Agreement, and pursuant to the terms of the Purchase Agreement, the parties desire to enter into this Agreement in order to grant certain rights to the Purchasers as set forth below.

NOW, THEREFORE, in consideration of the covenants and promises set forth herein, and for other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, the parties hereby agree as follows:

AGREEMENT

1. Certain Definitions. Unless the context otherwise requires, the following terms, for all purposes of this Agreement, shall have the meanings specified in this Section 1.

"Affiliate" has the meaning set forth in Rule 12b-2 of the rules and regulations promulgated under the Exchange Act; provided, however, that for purposes of this Agreement, the Purchasers and their Affiliates, on the one hand, and the Company and its Affiliates, on the other, shall not be deemed to be "Affiliates" of one another.

"Allowed Delay" has the meaning set forth in Section 2.1(b)(ii).

"Beneficially Own," "Beneficially Owned," or "Beneficial Ownership" have the meaning set forth in Rule 13d-3 of the rules and regulations promulgated under the Exchange Act.

"Board" means the board of directors of the Company.

"Business Days" has the meaning ascribed to such term in the Purchase Agreement.

"Change of Control" means the consummation of any transaction or series of related transactions involving (i) any purchase or acquisition (whether by way of tender offer, exchange offer, merger, consolidation, amalgamation, scheme or arrangement, acquisition, business combination or similar transaction or otherwise) by any Person or group (within the meaning of 13(d)(3) of the Exchange Act) of any of (A) securities representing a majority of the outstanding voting power of the Company entitled to elect the Board or (B) the majority of the outstanding Common Stock, (ii) any sale, lease, exchange, transfer, exclusive worldwide license or disposition of all or substantially all of the assets of the Company, taken together as a whole, to such Person or group, (iii) any merger, consolidation, amalgamation, scheme or arrangement, acquisition, business combination or similar transaction in which the holders of shares of the Common Stock and any other securities of the Company having the ordinary power to vote in the election of members of the Board of the Company and any securities convertible, exchangeable for or otherwise exercisable to acquire voting securities immediately prior to the transaction, as a

group, do not hold securities representing a majority of the outstanding voting power entitled to elect the board of directors of the surviving entity in such merger, consolidation, amalgamation, scheme or arrangement, acquisition, business combination or similar transaction or (iv) a liquidation, dissolution or winding up of the Company.

"Closing Date" has the meaning ascribed to such term in the Purchase Agreement.

"Commission" means the United States Securities and Exchange Commission.

"Common Stock" means shares of the common stock, par value $0.001 per share, of the Company.

"Exchange Act" means the Securities Exchange Act of 1934, as amended, and the rules and regulations of the Commission promulgated thereunder.

"Filing Deadline" has the meaning set forth in Section 2.1(a).

"FINRA" means the Financial Industry Regulatory Authority.

"Form S-3" means such form under the Securities Act as in effect on the date hereof or any successor or similar registration form under the Securities Act subsequently adopted by the Commission that permits inclusion or incorporation of substantial information by reference to other documents filed by the Company with the Commission.

"Free Writing Prospectus" means an issuer free writing prospectus, as defined in Rule 433 under the Securities Act, relating to an offer of Registrable Securities.

"Fully Diluted Basis" means all outstanding Common Stock assuming the exercise of all outstanding stock, warrants, rights, calls, options or other securities exchangeable or exercisable for, or convertible into, Common Stock without regard to any restrictions or conditions with respect to the

exercisability thereof.

"GAAP" has the meaning ascribed to such term in the Purchase Agreement.

"Holder" means any person owning of record Registrable Securities that have not been sold to the public or any transferee or assignee of record of such Registrable Securities to which the registration rights conferred by this Agreement have been transferred or assigned in accordance with Section 3.2 hereof.

"Initiating Shelf Take-Down Holder" has the meaning set forth in Section 2.1(c).

"Insolvency Event" means any of the following: (a) the Company files a petition under any chapter of title 11 of the United States Code (the "Bankruptcy Code") or commences a proceeding under any similar law in any other jurisdiction or any other similar law of any jurisdiction affecting creditors' rights; makes an assignment for the benefit of its creditors; or commences a proceeding for the appointment of a receiver, trustee, liquidator, custodian or conservator of itself or of the whole or substantially all of its property; (b) a petition is filed against the Company under any chapter of the Bankruptcy Code or any proceeding is commenced under any similar law of any other jurisdiction, or any other similar law of any jurisdiction affecting creditors' rights or for the appointment of a receiver, trustee, liquidator, custodian or conservator of the Company or of the whole or substantially all of its property and such petition or the related proceeding remains undismissed for a period of 30 days; or the Company by any act indicates its consent to, approval of or acquiescence in any such petition or proceeding; (c) a court of competent jurisdiction enters an order for relief against the Company under any chapter of the Bankruptcy Code or any other similar law of any jurisdiction affecting creditors' rights or enters an order, judgment or decree appointing or authorizing a receiver, trustee, liquidator, custodian or conservator of the Company or of the whole or substantially all of its or their property; or a court of competent jurisdiction or a receiver, trustee, liquidator, custodian or conservator, under the provisions of any law for the relief or aid of debtors, assumes custody or control

or takes possession of the Company or of the whole or substantially all of the property of the Company; or (d) the Company admits in writing its inability, or is generally unable, to pay its debts as such debts become due.

"RRA" means the Amended and Restated Registration Rights Agreement, dated August 7, 2003, by and between the Company and J.R. Hyde, III.

"RRA Holder" means the holder of the RRA Registrable Securities.

"RRA Registrable Securities" has the meaning ascribed to the term "Registrable Securities" under the RRA.

"RRA Registration" means any registration effected pursuant to the RRA.

"Nasdaq" has the meaning ascribed to such term in the Purchase Agreement.

"Participating Holder" means with respect to any registration, any Holder of Registrable Securities covered by the applicable Registration Statement.

"Person" has the meaning ascribed to such term in the Purchase Agreement.

"Piggyback Registrable Securities" means all Registrable Securities under this Agreement and all RRA Registrable Securities.

"Principal Purchasers" has the meaning set forth in the preamble.

"Prospectus" means the prospectus included in any Registration Statement, all amendments and supplements to such prospectus, including pre- and post-effective amendments to such Registration Statement, and all other material incorporated by reference in such prospectus.

"Register," "registered" and "registration" refer to a registration effected by preparing and filing a registration statement in compliance with the Securities Act, and the declaration or ordering of effectiveness of such registration statement or document.

"Registrable Securities" means shares of Common Stock held by the Purchasers (whether acquired prior to, on or following the Closing Date), and any Common Stock issued as (or issuable upon the conversion or exercise of any warrant (including the Warrants), right or other security which is issued as) a dividend or other distribution with respect to, or in exchange for or in replacement of, any Common Stock, warrant, right or other security held by the Purchasers. Notwithstanding the foregoing, Registrable Securities shall not include any securities of the Company sold by any person to the public either pursuant to a registration statement under the Securities Act or that is freely tradeable under Rule 144.

"Registration Statement" means any registration statement of the Company that covers Registrable Securities pursuant to the provisions of this Agreement filed with, or to be filed with, the SEC under the rules and regulations promulgated under the Securities Act, including the related Prospectus, amendments and supplements to such registration statement, including pre- and post-effective amendments, and all exhibits and all material incorporated by reference in such registration statement.

"Registration Expenses" has the meaning set forth in Section 2.3.

"Representatives" shall mean the directors, officers, employees and independent contractors, agents or advisors (including, without limitation, attorneys, accountants, and investment bankers) of the specified party or any of its Subsidiaries.

"Rule 144" means Rule 144 as promulgated by the SEC under the Securities Act, as such rule may be amended from time to time, or any similar successor rule that may be promulgated by the SEC.

"Rule 145" means Rule 145 promulgated by the Commission pursuant to the Securities Act, as such Rule may be amended from time to time, or any similar rule or regulation hereafter adopted by the Commission having substantially the same effect as such Rule.

"SEC" or "Commission" means the Securities and Exchange Commission or any other federal agency at the time administering the Securities Act.

"Securities Act" means the Securities Act of 1933, as amended, or any similar successor federal statute and the rules and regulations thereunder, all as the same shall be in effect from time to time.

"Shelf Registration Statement" has the meaning set forth in Section 2.1(a).

"Shelf Take-Down" has the meaning set forth in Section 2.1(c).

"Special Registration Statement" means (i) any registration statement relating to any employee benefit plan, (ii) with respect to any corporate reorganization or transaction under Rule 145, any registration statement related to the issuance or resale of securities issued in such a transaction, (iii) any registration statement related to stock issued upon conversion of debt securities, (iv) any RRA Registration and (v) the first Registration Statement on Form S-3 filed after the date hereof by the Company with the SEC that registers solely a Company primary offering on a continuous basis pursuant to Rule 415.

"Subsidiaries" means each corporation, limited liability company, partnership, association, joint venture or other business entity of which any party or any of its Affiliates owns, directly or indirectly, more than 50% of the stock or other equity interest entitled to vote on the election of the members of the board of directors or similar governing body.

"Transaction Documents" means this Agreement, the Purchase Agreement and the Warrants (as defined in the Purchase Agreement), all exhibits and schedules thereto and hereto and any other documents or agreement executed in connection with the transactions contemplated hereunder or thereunder.

2. Registration Rights.

2.1 Shelf Registration.

(a) Registration Statements. On or prior to the date that is 180 days after the Closing Date (the "Filing Deadline"), the Company shall prepare and file with the SEC one Registration Statement on Form S-3 (or, if Form S-3 is not then available to the Company, on such form of registration statement as is then available to effect a registration for resale of the Registrable Securities) for an offering to be made on a continuous basis pursuant to Rule 415 under the Securities Act (the "Shelf Registration Statement"). Such Shelf Registration Statement shall include the aggregate amount of Registrable Securities (including the Shares and Warrant Shares (as defined in the Purchase Agreement)) to be registered therein and the intended methods of distribution thereof, subject to the limitations of Form S-3. To the extent the rules and regulations of the Commission do not permit such Shelf Registration Statement to include all of the Registrable Securities, the Company shall use its reasonable best efforts to register the maximum amount permitted by the Commission and the Registrable Securities required to be omitted from such Shelf Registration Statement shall be determined in the sole discretion of the Principal Purchasers.

(b) Effectiveness.

(i) The Company shall use reasonable best efforts to have the Shelf Registration Statement declared effective as soon as practicable. The Company shall notify the Purchasers by facsimile or e-mail as promptly as practicable, and in any event, within twenty-four (24) hours, after any Registration Statement is declared effective and shall simultaneously provide the Purchasers with copies of any related Prospectus to be used in connection with the sale or other disposition of the securities covered thereby. Subject to any limitations provided herein, the Company shall cause the Shelf Registration Statement to remain effective until the earlier to occur of: (i) the date two years from the Closing or (ii) the date on which all of the Registrable Securities registered under the Shelf Registration Statement are either sold pursuant to the Shelf Registration Statement or sold or available for resale under Rule 144.

(ii) For not more than twenty (20) consecutive days or for a total of not more than forty-five (45) days in any twelve (12) month period, the Company may suspend the use of any Prospectus included in any Registration Statement contemplated by this Section in the event that the Company determines in good faith that such suspension is necessary to (A) delay the disclosure of material non-public information concerning the Company, the disclosure of which at the time is not, in the good faith opinion of the Company, in the best interests of the Company or (B) amend or supplement the affected Registration Statement or the related Prospectus so that such Registration Statement or Prospectus shall not include an untrue statement of a material fact or omit to state a material fact required to be stated therein or necessary to make the statements therein, in the case of the Prospectus in light of the circumstances under which they were made, not misleading (an "Allowed Delay"); provided, that the Company shall promptly (a) notify each Purchaser in writing of the commencement of and the reasons for an Allowed Delay, but shall not (without the prior written consent of a Purchaser) disclose to such Purchaser any material non-public information giving rise to an Allowed Delay, (b) advise the Purchasers in writing to cease all sales under the Registration Statement until the end of the Allowed Delay and (c) use commercially reasonable efforts to terminate an Allowed Delay as promptly as practicable.

(c) Shelf Take-Downs. An underwritten offering or sale of Registrable Securities pursuant to a Shelf Registration Statement (a "Shelf Take-Down") may be initiated by a Principal Purchaser who is a Participating Holder (an "Initiating Shelf Take-Down Holder"). Upon written request to the Company, the Company shall amend or supplement the Shelf Registration Statement for such purpose as soon as practicable. The Company shall send to each Participating Holder in the Shelf Registration Statement written notice of such Shelf Take-Down and, if within 5 days after the date of such notice, any such Participating Holder shall so request in writing, the Company shall include in such Shelf Take-Down all or any part of the Registrable Securities such Participating Holder requests to be included, subject to Section 2.6(a)(ii), it being understood the Company shall not be responsible for any underwriting discounts or commissions in connection with any Shelf Take Down.

2.2 Piggyback Registrations. If the Company determines to prepare and file with the SEC a Registration Statement, but excluding in all cases any Special Registration Statements, relating to an offering for its own account or the account of others of any of its equity securities at any time prior to March 6, 2019 or until such earlier date that no Registrable Securities are outstanding, then the Company shall send to each Holder written notice of such

determination and, if within 15 days after the date of such notice, any such Holder shall so request in writing, the Company shall include in such Registration Statement all or any part of the Registrable Securities such Holder requests to be registered, subject to Section 2.6(b)(ii).

2.3 Expenses. All expenses incident to the Company's performance of or compliance with this Agreement shall be paid by the Company, including reasonable expenses of one counsel to the Participating Holders in an amount not to exceed $50,000 in the aggregate during the term of this Agreement, other than underwriting discounts or commissions deducted from the proceeds in respect of any Registrable Securities, including (i) all registration and filing fees, and any other fees and expenses associated with filings required to be made with the SEC, FINRA or any other regulatory authority and, if applicable, the fees and expenses of any "qualified independent underwriter" as such term is defined in NASD Rule 2720 (or any successor provision) and of its counsel, (ii) all fees and expenses in connection with compliance with any securities or "Blue Sky" laws (including fees and disbursements of counsel for the underwriters in connection with "Blue Sky" qualifications of the Registrable Securities), (iii) all printing, duplicating, word processing, messenger, telephone, facsimile and delivery expenses (including expenses of printing certificates for the Registrable Securities in a form eligible for deposit with

The Depository Trust Company and of printing Prospectuses and Free Writing Prospectuses), (iv) all fees and disbursements of counsel for the Company and of all independent certified public accountants of the Company (including the expenses of any special audit and cold comfort letters required by or incident to such performance), (v) Securities Act liability insurance or similar insurance if the Company so desires or the underwriters so require in accordance with then-customary underwriting practice, (vi) all fees and expenses incurred in connection with the listing of Registrable Securities on any securities exchange or quotation of the Registrable Securities on any inter-dealer quotation system, (vii) all reasonable fees and disbursements of one legal counsel for the Participating Holders, as selected by the Principal Purchasers, (viii) any reasonable fees and disbursements of underwriters customarily paid by issuers or sellers of securities, (ix) all fees and expenses of any special experts or other Persons retained by the Company in connection with any registration, (x) all of the Company's internal expenses (including all salaries and expenses of its officers and employees performing legal or accounting duties), (xi) all expenses related to the "road-show" for any underwritten offering, including all travel, meals and lodging and (xii) any other fees and disbursements customarily paid by the issuers of securities. All such expenses are referred to herein as "Registration Expenses." The Company shall not be required to pay any underwriting discounts and commissions and transfer taxes, if any, attributable to the sale of Registrable Securities.

2.4 Company Obligations. The Company will use reasonable best efforts to effect the registration of the Registrable Securities in accordance with the terms hereof, and pursuant thereto the Company will:

(a) prepare the required Registration Statement including all exhibits and financial statements required under the Securities Act to be filed therewith, and before filing a Registration Statement, Prospectus or any Free Writing Prospectus, or any amendments or supplements thereto, (x) furnish to the underwriters, if any, and the Participating Holders, if any, copies of all documents prepared to be filed, which documents shall be subject to the review of such underwriters and the Participating Holders and their respective counsel and (y) except in the case of a registration under Section 2.2, not file any Registration Statement or Prospectus or amendments or supplements thereto to which any Participating Holders or the underwriters, if any, shall reasonably object;

(b) file with the SEC a Registration Statement relating to the Registrable Securities including all exhibits and financial statements required by the SEC to be filed therewith, and use commercially reasonable efforts to cause such Registration Statement to become effective under the Securities Act;

(c) prepare and file with the SEC such pre- and post-effective amendments to such Registration Statement, supplements to the Prospectus and such amendments or supplements to any Free Writing Prospectus as may be (y) reasonably requested by any Participating Holder or (z) necessary to keep such Registration effective for the period of time required by this Agreement, and comply with provisions of the applicable securities laws with respect to the sale or other disposition of all securities covered by such Registration Statement during such period in accordance with the intended method or methods of disposition by the sellers thereof set forth in such Registration Statement;

(d) promptly notify the Participating Holders and the managing underwriter or underwriters, if any, and (if requested) confirm such advice in writing and provide copies of the relevant documents, as soon as reasonably practicable after notice thereof is received by the Company (A) when the applicable Registration Statement or any amendment thereto has been filed or becomes effective, and when the applicable Prospectus or Free Writing Prospectus or any amendment or supplement thereto has been filed, (B) of any written comments by the SEC or any request by the SEC for amendments or supplements to such Registration Statement, Prospectus or Free Writing Prospectus or for additional information, (C) of the issuance by the SEC of any stop order suspending the effectiveness of such Registration Statement or any order by the SEC preventing or suspending the use of any preliminary or final Prospectus or any Free Writing Prospectus or the initiation or threatening of any proceedings for such purposes, (D) if, at any time, the representations and warranties of the Company in any applicable underwriting agreement cease to be true and correct in all material respects, (E) of the receipt by the Company of any notification with respect to the suspension of the qualification of the Registrable Securities for offering or sale in any jurisdiction and (F) of the receipt by the Company of any notification with respect to the initiation or threatening of any

proceeding for the suspension of the qualification of the Registrable Securities for offering or sale in any jurisdiction;

(e) promptly notify the Participating Holders and the managing underwriter or underwriters, if any, when the Company becomes aware of the happening of any event as a result of which the Registration Statement, the Prospectus included in such Registration Statement (as then in effect) or any Free Writing Prospectus contains any untrue statement of a material fact or omits to state a material fact necessary to make the statements therein (in the case of such Prospectus, any preliminary Prospectus or any Free Writing Prospectus, in light of the circumstances under which they were made) not misleading, when any Free Writing Prospectus includes information that may conflict with the information contained in the Registration Statement, or, if for any other reason it shall be necessary during such time period to amend or supplement such Registration Statement, Prospectus or Free Writing Prospectus in order to comply with the Securities Act and, in either case as promptly as reasonably practicable thereafter, prepare and file with the SEC and furnish without charge to the Participating Holders and the managing underwriter or underwriters, if any, an amendment or supplement to such Registration Statement, Prospectus or Free Writing Prospectus which shall correct such misstatement or omission or effect such compliance;

(f) promptly incorporate in a Prospectus supplement, Free Writing Prospectus or post-effective amendment to the applicable Registration Statement such information as the managing underwriter or underwriters and the Participating Holders agree should be included therein relating to the plan of distribution with respect to such Registrable Securities, and make all required filings of such Prospectus supplement, Free Writing Prospectus or post-effective amendment as soon as reasonably practicable after being notified of the matters to be incorporated in such Prospectus supplement, Free Writing Prospectus or post-effective amendment;

(g) furnish to each Participating Holder and each underwriter, if any, without charge, as many conformed copies as such Participating Holder or underwriter may reasonably request of the applicable Registration Statement and any amendment or post-effective amendment thereto, including financial statements and schedules, all documents incorporated therein by reference and all exhibits (including those incorporated by reference);

(h) deliver to each Participating Holder and each underwriter, if any, without charge, as many copies of the applicable Prospectus (including each preliminary Prospectus), any Free Writing Prospectus and any amendment or supplement thereto as such Participating Holder or underwriter may reasonably request (it being understood that the Company consents to the use of such Prospectus, any Free Writing Prospectus and any amendment or supplement thereto by such Participating Holder and the underwriters, if any, in connection with the offering and sale of the Registrable Securities thereby) and such other documents as such Participating Holder or underwriter may reasonably request in order to facilitate the disposition of the Registrable Securities by such Participating Holder or underwriter;

(i) on or prior to the date on which the Registration Statement is declared effective, use its reasonable best efforts to register or qualify, and cooperate with the Participating Holders, the managing underwriter or underwriters, if any, and their respective counsel, in connection with the registration or qualification of such Registrable Securities for offer and sale under the securities or "Blue Sky" laws of each state and other jurisdiction of the United States as any Participating Holder or managing underwriter or underwriters, if any, or their respective counsel reasonably request in writing and do any and all other acts or things reasonably necessary or advisable to keep such registration or qualification in effect for such period as required by this Agreement, provided that the Company shall not be required to qualify generally to do business in any jurisdiction where it is not then so qualified or to take any action which would subject it to taxation or general service of process in any such jurisdiction where it is not then so subject;

(j) cooperate with the Participating Holders and the managing underwriter or underwriters, if any, to facilitate the timely preparation and delivery of certificates representing Registrable Securities to be sold and not bearing any restrictive legends, and enable such Registrable Securities to be in such denominations and

registered in such names as the managing underwriters may request at least two (2) Business Days prior to any sale of Registrable Securities to the underwriters;

(k) use its reasonable best efforts to cause the Registrable Securities covered by the Registration Statement to be registered with or approved by such other governmental agencies or authorities as may be necessary to enable the seller or sellers thereof or the underwriter or underwriters, if any, to consummate the disposition of such Registrable Securities;

(l) make such representations and warranties to the Participating Holders and the underwriters or agents, if any, in form, substance and scope as are customarily made by issuers in secondary underwritten public offerings;

(m) enter into such customary agreements (including underwriting and indemnification agreements) and take all such other actions as the Purchasers or the managing underwriter or underwriters, if any, reasonably request in order to expedite or facilitate the registration and disposition of such Registrable Securities;

(n) obtain for delivery to the Participating Holders and to the underwriter or underwriters, if any, an opinion or opinions from counsel for the Company dated the effective date of the Registration Statement or, in the event of an underwritten offering, the date of the closing under the underwriting agreement, in customary form, scope and substance, which opinions shall be reasonably satisfactory to such Participating Holders or underwriters, as the case may be, and their respective counsel;

(o) in the case of an underwritten offering, obtain for delivery to the Company and the managing underwriter or underwriters, with copies to the Participating Holders, a cold comfort letter from the Company's independent certified public accountants in customary form and covering such matters of the type customarily covered by cold comfort letters as the managing underwriter or underwriters reasonably request, dated the date of execution of the underwriting agreement and brought down to the date of the closing under the underwriting agreement;

(p) cooperate with each Participating Holder and each underwriter, if any, participating in the disposition of such Registrable Securities and their respective counsel in connection with any filings required to be made with FINRA or any other securities regulatory authority;

(q) use its reasonable best efforts to comply with all applicable securities laws and make available to its security holders, as soon as reasonably practicable, an earnings statement satisfying the provisions of Section 11(a) of the Securities Act and the rules and regulations promulgated thereunder;

(r) provide and cause to be maintained a transfer agent and registrar for all Registrable Securities covered by the applicable Registration Statement from and after a date not later than the effective date of such Registration Statement;

(s) use commercially reasonable efforts to cause all Registrable Securities covered by the Registration Statement to be listed on each securities exchange on which any of the Common Stock is then listed or quoted and on each inter-dealer quotation system on which any of the Common Stock is then quoted;

(t) the Company shall make available, during normal business hours, for inspection and review by the Purchasers, advisors to and representatives of the Purchasers (who may or may not be affiliated with the Purchasers and who are reasonably acceptable to the Company), all financial and other records, all SEC Reports (as defined in the Purchase Agreement) and other filings with the SEC, and all other corporate documents and properties of the Company as may be reasonably necessary for the purpose of such review, and cause the Company's officers, directors and employees, within a reasonable

time period, to supply all such information reasonably requested by the Purchasers or any such representative, advisor or underwriter in connection with such Registration Statement (including, without limitation, in response to all questions and other inquiries reasonably

made or submitted by any of them), prior to and from time to time after the filing and effectiveness of the Registration Statement for the sole purpose of enabling the Purchasers and such representatives, advisors and underwriters and their respective accountants and attorneys to conduct initial and ongoing due diligence with respect to the Company and the accuracy of such Registration Statement; and

(u) with a view to making available to the Purchasers the benefits of Rule 144 (or its successor rule) and any other rule or regulation of the SEC that may at any time permit the Purchasers to sell shares of Common Stock to the public without registration, the Company covenants and agrees to: (i) make and keep public information available, as those terms are understood and defined in Rule 144, until the earlier of (A) the date as all of the Registrable Securities may be sold without restriction by the holders thereof pursuant to Rule 144 or any other rule of similar effect or (B) such date as all of the Registrable Securities shall have been resold; (ii) file with the SEC in a timely manner all reports and other documents required of the Company under the Exchange Act; and (iii) furnish to each Purchaser upon request, as long as such Purchaser owns any Registrable Securities, (A) a written statement by the Company that it has complied with the reporting requirements of the Exchange Act, (B) a copy of the Company's most recent Annual Report on Form 10-K or Quarterly Report on Form 10-Q, and (C) such other information as may be reasonably requested in order to avail such Purchaser of any rule or regulation of the SEC that permits the selling of any such Registrable Securities without registration.

2.5 Obligations of the Purchasers.

(a) Each Purchaser shall furnish in writing to the Company such information regarding itself, the Registrable Securities held by it and the intended method of disposition of the Registrable Securities held by it, as shall be reasonably required to effect the registration of such Registrable Securities and shall execute such documents in connection with such registration as the Company may reasonably request. At least five (5) Business Days prior to the first anticipated filing date of any Registration Statement, the Company shall notify each Purchaser of the information the Company requires from such Purchaser if such Purchaser elects to have any of its Registrable Securities included in the Registration Statement. A Purchaser shall provide such information to the Company at least two (2) Business Days prior to the first anticipated filing date of such Registration Statement if such Purchaser elects to have any of its Registrable Securities included in the Registration Statement.

(b) Each Purchaser, by its acceptance of the Registrable Securities agrees to cooperate with the Company as reasonably requested by the Company in connection with the preparation and filing of a Registration Statement hereunder, unless such Purchaser has notified the Company in writing of its election to exclude all of its Registrable Securities from such Registration Statement.

(c) Each Purchaser agrees that, upon receipt of any notice from the Company of either (i) the commencement of an Allowed Delay pursuant to Section 2.1(b)(ii) the happening of an event pursuant to Section 2.4(d) and Section 2.4(e) hereof, such Purchaser will immediately discontinue disposition of Registrable Securities pursuant to the Registration Statement covering such Registrable Securities, until the Purchaser is advised by the Company that such dispositions may again be made.

2.6 Underwriting.

(a) Shelf Registrations.

(i) If the Initiating Shelf Take-Down Holder so requests, an offering of Registrable Securities shall be in the form of an underwritten offering, and such Initiating Shelf Take-Down Holder shall have the right to select the managing underwriter or underwriters to administer the offering. In the case of an underwritten offering under Section 2.1, the price, underwriting discount and other financial terms for the Registrable Securities shall be determined by the Initiating Shelf Take-Down Holder.

(ii) If the managing underwriter or underwriters of a proposed underwritten offering of the Registrable Securities included in a Shelf Take-Down advise the Board in writing that, in its or their opinion,

the number of securities requested to be included in such Shelf Take-Down exceeds the number which can be sold in such offering without being likely to have a significant adverse effect on the price, timing or distribution of the securities offered or the market for the securities offered, the securities to be included in such Shelf Take-Down (i) first, shall be allocated *pro rata* among the Participating Holders that have requested to participate in such Shelf Take-Down based on the relative number of Registrable Securities requested by each Participating Holder to be included in such Shelf Take-Down and (ii) second, and only if all the securities referred to in clause (i) have been included in such Shelf Take-Down, the number of securities that the Company proposes to include in such Shelf Take-Down that, in the opinion of the managing underwriter or underwriters, can be sold without having such adverse effect.

(iii) If requested by the underwriters for any underwritten offering requested by an Initiating Shelf Take-Down Holder under Section 2.1, the Company shall enter into an underwriting agreement with such underwriters for such offering, such agreement to be reasonably satisfactory in substance and form to the Company, the Initiating Shelf Take-Down Holder and the underwriters, and to contain such representations and warranties by the Company and such other terms as are generally prevailing in agreements of that type, including customary indemnities.

(b) Piggyback Registrations.

(i) If the Company proposes to register any of its securities under the Securities Act as contemplated by Section 2.2 and such securities are to be distributed in an underwritten offering through one or more underwriters, the Company shall, if requested by any Holders pursuant to Section 2.2, use its reasonable best efforts to arrange for such underwriters to include on the same terms and conditions that apply to the other sellers in such registration all the Registrable Securities to be offered and sold by such Holders among the securities of the Company to be distributed by such underwriters in such registration.

(ii) If the managing underwriter or underwriters of any proposed underwritten offering including Registrable Securities pursuant to Section 2.2 informs the Company and each Participating Holder and the RRA Holder that, in its or their opinion, the number of securities which the Participating Holders and the RRA Holder intend to include in such offering exceeds the number which can be sold in such offering without being likely to have a significant adverse effect on the price, timing or distribution of the securities offered or the market for the securities offered, then the securities to be included in such registration shall be allocated *pro rata* among the Participating Holders and/or RRA Holder that have requested to participate in such registration based on the relative number of Registrable Securities requested by each Participating Holder and/or RRA Holder, as applicable, to be included in such underwritten offering.

(ii) Notwithstanding anything in this Agreement to the contrary, the provisions of this Section 2.6(b) may be amended or waived (either generally or in a particular instance, either retroactively or prospectively and either for a specified period of time or indefinitely), with the written consent of (i) the Company and (ii) the holders holding at least sixty percent (60%) of the then outstanding Piggyback Registrable Securities; *provided, however*, that if any such waiver or amendment effected pursuant to this Section 2.6(b)(iii) materially and adversely affects the rights of the Holders and does not materially and adversely affect the rights of the RRA Holder in the same manner, then such waiver or amendment shall require the consent of the holders of a majority-in-interest of the then outstanding Registrable Securities; *provided further* that if any such waiver or amendment effected pursuant to this Section 2.6(b)(iii) materially and adversely affects the rights of the RRA Holder and does not materially and adversely affect the rights of the Holders in the same manner, then such waiver or amendment shall require the consent of the RRA Holder.

(c) Participation in Underwritten Registrations. Subject to the provisions of Section 2.6(a)(ii) and Section 2.6(b)(ii) above, no Person may participate in any underwritten offering hereunder unless such Person (i) agrees to sell such Person's securities on the basis provided in any underwriting arrangements approved by the Persons entitled to approve such arrangements and (ii) completes and executes all questionnaires, powers of attorney, indemnities, underwriting agreements and other documents required under the terms of such underwriting arrangements and all applicable securities laws. The Participating Holders shall be parties to such underwriting

agreement, which underwriting agreement shall (i) contain such representations and warranties by, and the other agreements on the part of, the Company to and for the benefit of such Participating Holders as are customarily made by issuers to selling stockholders in secondary underwritten public offerings and (ii) provide that any or all of the conditions precedent to the obligations of such underwriters under such underwriting agreement also shall be conditions precedent to the obligations of such Participating Holders. Any such Participating Holder shall not be required to make any representations or warranties to or agreements with the Company or the underwriters in connection with such underwriting agreement other than representations, warranties or agreements regarding such Participating Holder, such Participating Holder's title to the Registrable Securities, such Participating Holder's authority to sell the Registrable Securities, such Participating Holder's intended method of distribution, absence of liens with respect to the Registrable Securities, enforceability of the applicable underwriting agreement as against such Participating Holder, receipt of all consents and approvals with respect to the entry into such underwriting agreement and the sale of such Registrable Securities and any other representations required to be made by such Participating Holder under applicable law, rule or regulation, and the aggregate amount of the liability of such Participating Holder in connection with such underwriting agreement shall not exceed such Participating Holder's net proceeds from such underwritten offering.

(d) Clear Market. With respect to any underwritten offerings of Registrable Securities by the Holders, the Company agrees not to, and shall not be obligated to, effect any public sale or distribution, or to file any Registration Statement covering any of its equity securities or any securities convertible into or exchangeable or exercisable for such securities, during the period not to exceed ten (10) days prior and sixty (60) days following the effective date of such offering (or such lesser period that the managing underwriters in any underwritten offering permit). Notwithstanding the foregoing, the Company may effect the registration of (A) equity securities and/or options or other rights in respect thereof solely registered on Form S-4 or Form S-8 (or successor form) or (B) shares of equity securities and/or options or other rights in respect thereof to be offered to directors, employees, consultants, customers, lenders or vendors of the Company or its Subsidiaries or in connection with dividend reinvestment plans.

2.7 Indemnification.

(a) Indemnification by the Company. The Company will indemnify and hold harmless each Purchaser and its officers, directors, members, employees and agents, successors and assigns, and each other person, if any, who controls such Purchaser within the meaning of the Securities Act, against any losses, claims, damages or liabilities, joint or several, to which they may become subject under the Securities Act or otherwise, insofar as such losses, claims, damages or liabilities (or actions in respect thereof) arise out of or are based upon: (i) any untrue statement or alleged untrue statement of any material fact contained in any Registration Statement, any preliminary Prospectus or final Prospectus, or any amendment or supplement thereof or any omission or alleged omission to state a material fact required to be stated therein or necessary to make the statements therein not misleading, in light of the circumstances in which they were made; (ii) any "Blue Sky" application or other document executed by the Company specifically for that purpose or based upon written information furnished by the Company filed in any state or other jurisdiction in order to qualify any or all of the Registrable Securities under the securities laws thereof (any such application, document or information herein called a "Blue Sky Application"); (iii) the omission or alleged omission to state in a Blue Sky Application a material fact required to be stated therein or necessary to make the statements therein not misleading, in light of the circumstances in which they were made; (iv) any violation by the Company or its agents of any rule or regulation promulgated under the Securities Act applicable to the Company or its agents and relating to action or inaction required of the Company in connection with such registration; or (v) any failure to register or qualify the Registrable Securities included in any such Registration Statement in any state where the Company or its agents has affirmatively undertaken or agreed in writing that the Company will undertake such registration or qualification on a Purchaser's behalf and will reimburse such Purchaser, and each such officer, director or member and each such controlling person for any legal or other expenses reasonably incurred by them in connection with investigating or defending any such loss, claim, damage, liability or action; provided, however, that the Company will not be liable in any such case if and to the extent that any such loss, claim, damage or liability arises out of or is based upon an untrue statement or alleged untrue statement or omission or alleged omission so made in conformity with information furnished by such Purchaser or any such controlling person in writing specifically for use in such Registration Statement or Prospectus.

(b) Indemnification by the Purchasers. Each Purchaser agrees, severally but not jointly, to indemnify and hold harmless, to the fullest extent permitted by law, the Company, its directors, officers, employees, stockholders and each person who controls the Company (within the meaning of the Securities Act) against any losses, claims, damages, liabilities and expense (including reasonable attorney fees) resulting from any untrue statement or alleged

untrue statement of a material fact or any omission or alleged omission of a material fact required to be stated in the Registration Statement or Prospectus or preliminary Prospectus or amendment or supplement thereto or necessary to make the statements therein not misleading, in light of the circumstances in which they were made, to the extent, but only to the extent that such untrue statement or alleged untrue statement or omission or alleged omission is contained in any information furnished in writing by such Purchaser to the Company specifically for inclusion in such Registration Statement or Prospectus or amendment or supplement thereto. In no event shall the liability of a Purchaser be greater in amount than the dollar amount of the proceeds (net of all expense paid by such Purchaser in connection with any claim relating to this Section 2 and the amount of any damages such Purchaser has otherwise been required to pay by reason of such untrue statement or omission) received by such Purchaser upon the sale of the Registrable Securities included in the Registration Statement giving rise to such indemnification obligation.

(c) Conduct of Indemnification Proceedings. Any Person entitled to indemnification hereunder shall (i) give prompt notice to the indemnifying party of any claim with respect to which it seeks indemnification and (ii) permit such indemnifying party to assume the defense of such claim with counsel reasonably satisfactory to the indemnified party (provided, however, that such indemnified party shall, at the expense of the indemnifying party, be entitled to counsel of its own choosing to monitor such defense); provided that, subject to the preceding sentence, any Person entitled to indemnification hereunder shall have the right to employ separate counsel and to participate in the defense of such claim, but the fees and expenses of such counsel shall be at the expense of such Person unless (a) the indemnifying party has agreed to pay such fees or expenses, or (b) the indemnifying party shall have failed to assume the defense of such claim and employ counsel reasonably satisfactory to such person or (c) in the reasonable judgment of any such Person, based upon written advice of its counsel, a conflict of interest exists between such person and the indemnifying party with respect to such claims (in which case, if the Person notifies the indemnifying party in writing that such Person elects to employ separate counsel at the expense of the indemnifying party, the indemnifying party shall not have the right to assume the defense of such claim on behalf of such Person); and provided, further, that the failure of any indemnified party to give notice as provided herein shall not relieve the indemnifying party of its obligations hereunder, except to the extent that such failure to give notice shall materially adversely affect the indemnifying party in the defense of any such claim or litigation. It is understood that the indemnifying party shall not, in connection with any proceeding in the same jurisdiction, be liable for fees or expenses of more than one separate firm of attorneys at any time for all such indemnified parties. No indemnifying party will, except with the consent of the indemnified party, consent to entry of any judgment or enter into any settlement that does not include as an unconditional term thereof the giving by the claimant or plaintiff to such indemnified party of a release from all liability in respect of such claim or litigation.

(d) Contribution. If for any reason the indemnification provided for in the preceding paragraphs (a) and (b) is unavailable to an indemnified party or insufficient to hold it harmless, other than as expressly specified therein, then the indemnifying party shall contribute to the amount paid or payable by the indemnified party as a result of such loss, claim, damage or liability in such proportion as is appropriate to reflect the relative fault of the indemnified party and the indemnifying party, as well as any other relevant equitable considerations. No Person guilty of fraudulent misrepresentation within the meaning of Section 11(f) of the Securities Act shall be entitled to contribution from any Person not guilty of such fraudulent misrepresentation. In no event shall the contribution obligation of a holder of Registrable Securities be greater in amount than the dollar amount of the proceeds (net of all expenses paid by such holder in connection with any claim relating to this Section 2 and the amount of any damages such holder has otherwise been required to pay by reason of such untrue or alleged untrue statement or omission or alleged omission) received by it upon the sale of the Registrable Securities giving rise to such contribution obligation.

3. Miscellaneous.

3.1 Governing Law; Jurisdiction. This Agreement shall be governed by, and construed in accordance with, the internal laws of the State of Delaware without regard to the choice of law principles thereof. Each of the parties hereto irrevocably submits to the exclusive jurisdiction of the state and federal courts located in the State of Delaware for the purpose of any suit, action, proceeding or judgment relating to or arising out of this Agreement and the transactions contemplated hereby. Service of process in connection with any such suit, action or proceeding may be served on each party hereto anywhere in the world by the same methods as are specified for the giving of notices under this Agreement. Each of the parties hereto irrevocably consents to the jurisdiction of any such court in any such suit, action or proceeding and to the laying of venue in such court. Each party hereto irrevocably waives any objection to the laying of venue of any such suit, action or proceeding brought in such courts and irrevocably waives any claim that any such suit, action or proceeding brought in any such court has been brought in an inconvenient forum.

3.2 Successors and Assigns. Except as otherwise expressly provided herein, the provisions hereof shall inure to the benefit of, and be binding upon, the successor and assigns of the parties hereto (other than the rights of any Holder under Section 2 hereof, which shall not be assignable and shall not inure to the benefit of any successor or assign of a Holder). The Company may not assign its rights or obligations hereunder except with the prior written consent of each Holder. Each Holder may assign their respective rights hereunder in the manner and to the Persons permitted under the Purchase Agreement, except as specified above.

3.3 Entire Agreement; Amendment. This Agreement and the other Transaction Documents constitute the full and entire understanding and agreement between the parties with regard to the subjects hereof and thereof. Any previous agreements among the parties relative to the specific subject matter hereof are superseded by this Agreement. Neither this Agreement nor any provision hereof may be amended, changed, waived, discharged or terminated other than by a written instrument signed by the party against who enforcement of any such amendment, change, waiver, discharge or termination is sought.

3.4 Notices. All notices and other communications provided for or permitted hereunder shall be made as set forth in Section 7.3 of the Purchase Agreement.

3.5 Severability. If any term, provision, covenant or restriction of this Agreement is held by a court of competent jurisdiction to be invalid, illegal, void or unenforceable, the remainder of the terms, provisions, covenants and restrictions set forth herein shall remain in full force and effect and shall in no way be affected, impaired or invalidated, and the parties hereto shall use their commercially reasonable efforts to find and employ an alternative means to achieve the same or substantially the same result as that contemplated by such term, provision, covenant or restriction. It is hereby stipulated and declared to be the intention of the parties that they would have executed the remaining terms, provisions, covenants and restrictions without including any of such that may be hereafter declared invalid, illegal, void or unenforceable.

3.6 Headings. The headings herein are for convenience only, do not constitute a part of this Agreement and shall not be deemed to limit or affect any of the provisions hereof.

3.7 Counterparts. This Agreement may be executed in two or more counterparts, all of which when taken together shall be considered one and the same agreement and shall become effective when counterparts have been signed by each party and delivered to each other party, it being understood that the parties need not sign the same counterpart. In the event that any signature is delivered by facsimile transmission or by e-mail delivery of a ".pdf" format data file, such signature shall create a valid and binding obligation of the party executing (or on whose behalf such signature is executed) with the same force and effect as if such facsimile or ".pdf" signature page were an original thereof.

3.8 Delays or Omissions. It is agreed that no delay or omission to exercise any right, power or remedy accruing to any party upon any breach or default of any other party under this Agreement shall impair any such right, power or remedy, nor shall it be construed to be a waiver of any such breach or default, or any acquiescence therein, or of any similar breach or default thereafter occurring; nor shall any waiver of any single breach or default

be deemed a waiver of any other breach or default theretofore or thereafter occurring. It is further agreed that any waiver, permit, consent or approval of any kind or character of any breach or default under this Agreement, or any waiver of any provisions or conditions of this Agreement must be in writing and shall be effective only to the extent specifically set forth in writing, and that all remedies, either under this Agreement, by law or otherwise, shall be cumulative and not alternative.

3.9 Consents. Any permission, consent, or approval of any kind or character under this Agreement shall be in writing and shall be effective only to the extent specifically set forth in such writing.

3.10 SPECIFIC PERFORMANCE. THE PARTIES HERETO AGREE THAT IRREPARABLE DAMAGE WOULD OCCUR IN THE EVENT THAT ANY OF THE PROVISIONS OF THIS AGREEMENT WERE NOT PERFORMED IN ACCORDANCE WITH ITS SPECIFIC INTENT OR WERE OTHERWISE BREACHED. IT IS ACCORDINGLY AGREED THAT THE PARTIES SHALL BE ENTITLED TO AN INJUNCTION OR INJUNCTIONS, WITHOUT BOND, TO PREVENT OR CURE BREACHES OF THE PROVISIONS OF THIS AGREEMENT AND TO ENFORCE SPECIFICALLY THE TERMS AND PROVISIONS HEREOF, THIS BEING IN ADDITION TO ANY OTHER REMEDY TO WHICH THEY MAY BE ENTITLED BY LAW OR EQUITY, AND ANY PARTY SUED FOR BREACH OF THIS AGREEMENT EXPRESSLY WAIVES ANY DEFENSE THAT A REMEDY IN DAMAGES WOULD BE ADEQUATE.

3.11 Construction of Agreement. No provision of this Agreement shall be construed against either party as the drafter thereof.

3.12 Section References. Unless otherwise stated, any reference contained herein to a Section or subsection refers to the provisions of this Agreement.

3.13 Variations of Pronouns. All pronouns and all variations thereof shall be deemed to refer to the masculine, feminine, or neuter, singular or plural, as the context in which they are used may require.

[Remainder of Page Intentionally Left Blank; Signature Pages Follow]

IN WITNESS WHEREOF, the parties have caused this Registration Rights Agreement to be duly executed and delivered by their proper and duly authorized officers as of the day and year first written above.

GTx, INC.

By: /s/ Marc S. Hanover
Name: Marc S. Hanover
Title: President and Chief Operating Officer

[Signature Page to Registration Rights Agreement]

IN WITNESS WHEREOF, the parties have caused this Registration Rights Agreement to be duly executed and delivered by their proper and duly authorized officers as of the day and year first written above.

J.R. HYDE, III

By: /s/ J.R. Hyde, III

[Signature Page to Registration Rights Agreement]

IN WITNESS WHEREOF, the parties have caused this Registration Rights Agreement to be duly executed and delivered by their proper and duly authorized officers as of the day and year first written above.

THE PYRAMID PEAK FOUNDATION

By: /s/ Andrew R. McCarroll
Name: Andrew R. McCarroll
Title: Secretary

[Signature Page to Registration Rights Agreement]

NEITHER THIS WARRANT, NOR THE SECURITIES ISSUABLE UPON EXERCISE OF THIS WARRANT (COLLECTIVELY, THE "SECURITIES"), HAVE BEEN REGISTERED UNDER THE SECURITIES ACT OF 1933, AS AMENDED (THE "SECURITIES ACT"), OR UNDER ANY STATE SECURITIES OR BLUE SKY LAWS. THE SECURITIES ARE SUBJECT TO RESTRICTIONS ON TRANSFERABILITY AND RESALE AND MAY NOT BE OFFERED, SOLD, PLEDGED, HYPOTHECATED OR OTHERWISE TRANSFERRED EXCEPT AS PERMITTED UNDER THE SECURITIES ACT AND APPLICABLE STATE SECURITIES OR BLUE SKY LAWS, PURSUANT TO REGISTRATION OR QUALIFICATION OR EXEMPTION THEREFROM. INVESTORS SHOULD BE AWARE THAT THEY MAY BE REQUIRED TO BEAR THE FINANCIAL RISKS OF THIS INVESTMENT FOR AN INDEFINITE PERIOD OF TIME. THE ISSUER OF THE SECURITIES MAY REQUIRE AN OPINION OF COUNSEL IN FORM AND SUBSTANCE REASONABLY SATISFACTORY TO THE ISSUER TO THE EFFECT THAT ANY PROPOSED TRANSFER IS IN COMPLIANCE WITH THE SECURITIES ACT AND ANY APPLICABLE STATE SECURITIES OR BLUE SKY LAWS. THIS WARRANT IS SUBJECT TO THE TRANSFER RESTRICTIONS SET FORTH HEREIN AND IN AN INVESTORS RIGHT AGREEMENT, DATED AS OF MARCH 3, 2014, COPIES OF WHICH ARE AVAILABLE WITH THE SECRETARY OF THE ISSUER.

GTx, INC.

FORM OF COMMON STOCK WARRANT

Warrant No. CSW-[·] Date of Issuance: March 6, 2014

GTx, Inc., a Delaware corporation (the "Company"), hereby certifies that, for value received, [·], a [·], or its registered assign (the "Holder"), is entitled to purchase from the Company [·] shares (as adjusted from time to time as provided in Section 12) of common stock, par value $0.001 per share, of the Company (the "Common Stock") (each such share, a "Warrant Share" and all such shares, the "Warrant Shares"), at an exercise price determined pursuant to Section 3 (the "Exercise Price"), at any time and from time to time from and after the date hereof through and including the date that is one (1) year following the date of issuance set forth above (the "Expiration Date"), and subject to the following terms and conditions:

1. Purchase Agreement. This Common Stock Warrant (this "Warrant") is one of a series of Warrants (collectively, the "Warrants") issued by the Company in connection with that certain Securities Purchase Agreement, entered into on March 3, 2014 (the "Purchase Agreement"), by and among the Company and Holder and certain other Purchasers, and is subject to, and the Company and the Holder shall be bound by, all the applicable terms, conditions and provisions of the Purchase Agreement.

2. Definitions. In addition to the terms defined elsewhere in this Warrant, capitalized terms that are not otherwise defined herein shall have the meanings assigned to such terms in the Purchase Agreement.

3. Exercise Price. This Warrant may be exercised for a price per Warrant Share equal to $1.67, subject to adjustment from time to time pursuant to Section 12 (the "Exercise Price").

4. Registration of Warrant. The Company shall register this Warrant, upon records to be maintained by the Company for that purpose (the "Warrant Register"), in the name of the record Holder hereof from time to time. The Company may deem and treat the Holder of this Warrant as the absolute owner hereof for the purpose of any exercise hereof or any distribution to the Holder, and for all other purposes, absent actual notice to the contrary.

5. Transfer of Warrant.

(a) No Holder may, directly or indirectly, sell, exchange, assign or otherwise transfer all or any portion of this Warrant without the prior written consent of the Company; provided that (i) a Holder that is a natural person may transfer all or a portion of this Warrant to one or more trusts for the benefit of such Holder, such Holder's spouse, a lineal descendant of such Holder or such Holder's parents, the spouse of any such descendant or

a lineal descendant of any such spouse and (ii) a Holder that is a Person other than a natural person may transfer all or a portion of the Warrant to an Affiliate of such Holder.

(b) Subject to the Holder's appropriate compliance with the restrictive legend on this Warrant and the transfer restrictions set forth herein, the Company shall register the transfer of any portion of this Warrant in the Warrant Register, upon surrender of this Warrant, with the Form of Assignment substantially in the form attached hereto as Attachment B duly completed and signed, to the Company at its address specified herein. Upon any such registration or transfer, a new Warrant to purchase Common Stock, in substantially the form of this Warrant (any such new Warrant, a "New Warrant"), evidencing the portion of this Warrant so transferred shall be issued to the transferee and a New Warrant evidencing the remaining portion of this Warrant not so transferred, if any, shall be issued to the transferring Holder. The acceptance of the New Warrant by the transferee thereof shall be deemed the acceptance by such transferee of all of the rights and obligations of a holder of a Warrant.

6. Exercise and Duration of Warrants. This Warrant shall be exercisable by the registered Holder at any time and from time to time on or after the date hereof to and including the Expiration Date. At 6:30 p.m., New York City time, on the Expiration Date, the portion of this Warrant not exercised prior thereto shall be and become void and of no value.

7. Delivery of Warrant Shares.

(a) To effect conversions hereunder, the Holder shall not be required to physically surrender this Warrant unless the aggregate number of Warrant Shares represented by this Warrant is being exercised. Upon delivery of an Exercise Notice substantially in the form attached hereto as Attachment A (an "Exercise Notice") to the Company at its address for notice determined as set forth herein, and upon payment of the applicable Exercise Price multiplied by the number of Warrant Shares that the Holder intends to purchase hereunder, the Company shall promptly (but in no event later than five (5) trading days after the Date of Exercise (as defined below)) issue and deliver, or cause its transfer agent to issue and deliver, to the Holder a certificate for the Warrant Shares issuable upon such exercise registered in the name of the Holder or its designee. A "Date of Exercise" means the date on which the Holder shall have delivered to the Company: (i) an Exercise Notice, appropriately completed and duly signed, and (ii) payment of the Exercise Price (by certified or official bank check, intra-bank account transfer or wire transfer) for the number of Warrant Shares so indicated by the Holder to be purchased.

(b) If by the fifth trading day after a Date of Exercise the Company fails to deliver the required number of Warrant Shares in the manner required pursuant to Section 7(a), the Holder will have the right to rescind such exercise.

(c) The Company's obligations to issue and deliver Warrant Shares in accordance with the terms hereof are absolute and unconditional, irrespective of any action or inaction by the Holder to enforce the same, any waiver or consent with respect to any provision hereof, the recovery of any judgment against any Person or any action to enforce the same, or any setoff, counterclaim, recoupment, limitation or termination, or any breach or alleged breach by the Holder or any other Person of any obligation to the Company or any violation or alleged violation of law by the Holder or any other Person, and irrespective of any other circumstance which might otherwise limit such obligation of the Company to the Holder in connection with the issuance of Warrant Shares. Nothing herein shall limit a Holder's right to pursue any other remedies available to it hereunder, at law or in equity, including a decree of specific performance and/or injunctive relief with respect to the Company's failure to timely deliver certificates representing shares of Common Stock upon exercise of the Warrant as required pursuant to the terms hereof; provided, however, under no circumstances shall the Company be required to settle the Warrant by cash payment.

8. Charges, Taxes and Expenses. Issuance and delivery of certificated or uncertificated shares of Common Stock upon exercise of this Warrant shall be made without charge to the Holder for any issue or transfer tax, withholding tax, transfer agent fee, or other incidental tax or expense in respect of the issuance of such shares, all of which taxes and expenses shall be paid by the Company; provided, however, that the Company shall not be

required to pay any tax which may be payable in respect of any transfer involved in the registration of any certificates for Warrant Shares or Warrants in a name other than that of the Holder. The Holder shall be responsible for all other tax liability that may arise as a result of holding or transferring this Warrant or receiving Warrant Shares upon exercise hereof.

9. Replacement of Warrant. If this Warrant is mutilated, lost, stolen or destroyed, the Company shall issue or cause to be issued in exchange and substitution for and upon cancellation hereof, or in lieu of and substitution for this Warrant, a new Warrant, but only upon receipt of evidence reasonably satisfactory to the Company of such loss, theft or destruction and customary and reasonable indemnity (which shall not include a surety bond), if requested. Applicants for a new warrant under such circumstances shall also comply with such other reasonable regulations and procedures and pay such other reasonable third-party costs as the Company may prescribe. If a new warrant is requested as a result of a mutilation of this Warrant, then the Holder shall deliver this mutilated Warrant to the Company as a condition precedent to the Company's obligation to issue the new warrant.

10. Reservation of Warrant Shares. The Company covenants that it will at all times reserve and keep available out of the aggregate of its authorized but unissued and otherwise unreserved Common Stock, solely for the purpose of enabling it to issue Warrant Shares upon exercise of this Warrant as herein provided, the number of Warrant Shares which are then issuable and deliverable upon the exercise of this entire Warrant, free from Liens or any other contingent purchase rights of persons other than the Holder (taking into account the adjustments and restrictions of Section 12). The Company covenants and warrants that all Warrant Shares so issuable and deliverable shall, upon issuance and the payment of the applicable Exercise Price in accordance with the terms hereof, be duly and validly authorized, issued and fully paid and non-assessable

11. Notice of Certain Corporate Action. In case the Company shall propose (a) to offer to the holders of its Common Stock rights to subscribe for or to purchase any shares of Common Stock or shares of stock of any class or any other securities, rights or options, or (b) to effect any reclassification of its Common Stock (other than a reclassification involving only the subdivision, or combination, of outstanding shares of Common Stock), or (c) to effect any capital reorganization, or (d) to effect any Fundamental Transaction (as defined below), or (e) to effect the liquidation, dissolution or winding up of the Company or (f) to offer to the holders generally of its Common Stock the right to have their shares of Common Stock repurchased or redeemed or otherwise acquired by the Company, or (g) to take any other action which would require the adjustment of the Exercise Price and/or the number of Warrant Shares issuable upon exercise of this Warrant, then in each such case (but without limiting the provisions of Section 12), the Company shall give to the Holder, a notice of such proposed action, which shall specify the date on which a record is to be taken for purposes of such dividend, distribution of offer of rights, or the date on which such reclassification, reorganization, Fundamental Transaction, liquidation, dissolution, or winding up is to take place and the date of participation therein by the holders of Common Stock, if any such date is to be fixed and shall also set forth such facts with respect thereto as shall be reasonably necessary to indicate the effect of such action on the Common Stock. Such notice shall be so given at least ten (10) Business Days prior to the record date for determining holders of the Common Stock for purposes of participating in or voting on such action, or at least ten (10) Business Days prior to the date of the taking of such proposed action or the date of participation therein by the holders of Common Stock, whichever shall be the earlier. Such notice shall specify, in the case of any subscription or repurchase rights, the date on which the holders of Common Stock shall be entitled thereto. Such notice shall also state whether the action in question or the record date is subject to the effectiveness of a registration statement under the Securities Act or to a favorable vote of security holders, if either is required, and the adjustment in Exercise Price and/or number of Warrant Shares issuable upon exercise of this Warrant as a result of such reorganization, reclassification, Fundamental Transaction or other action, to the extent then determinable. No such notice shall be given if the Company reasonably determines that the giving of such notice would require disclosure of material information which the Company has a bona fide purpose for preserving as confidential or the disclosure of which would not be in the best interests of the Company.

12. Certain Adjustments. The number of Warrant Shares issuable upon exercise of this Warrant is subject to adjustment from time to time as set forth in this Section 12.

(a) Stock Dividends and Splits. If the Company, at any time while this Warrant is outstanding: (i) pays a stock dividend or otherwise makes a distribution or distributions on shares of its Common Stock or any other equity or equity equivalent securities payable in shares of Common Stock (which, for avoidance of doubt, shall not include any shares of Common Stock issued by the Company upon exercise of any Warrants), (ii) subdivides outstanding shares of Common Stock into a larger number of shares, (iii) combines (including by way of reverse stock split) outstanding shares of Common Stock into a smaller number of shares, or (iv) issues by reclassification of shares of Common Stock any shares of capital stock of the Company; then in each such case (A) the Exercise Price will be adjusted by multiplying the Exercise Price then in effect by a fraction, the numerator of which equals the number of shares of Common Stock outstanding immediately prior to such event (excluding treasury shares, if any), and the denominator of which equals the number of shares of Common Stock outstanding immediately after such event (excluding treasury shares, if any), and (B) the number of Warrant Shares issuable hereunder shall be concurrently adjusted by multiplying such number by the reciprocal of such fraction. Such adjustments will take effect (i) if a record date shall have been

fixed for determining the stockholders or security holders, as applicable, of the Company entitled to receive such dividend, distribution or issuance by reclassification, as the case may be, immediately after such record date, (ii) otherwise, immediately after the effective date of such dividend, distribution, subdivision, combination, or issuance by reclassification, as the case may be.

(b) Fundamental Transaction. If, at any time while this Warrant is outstanding, (i) the Company, directly or indirectly, in one or a series of related transactions, (A) effects any merger or consolidation of the Company with or into another Person, (B) effects any sale, lease, license, assignment, transfer, conveyance or other disposition of all or substantially all of its assets, (C) effects any reclassification, reorganization or recapitalization of the Common Stock or any compulsory share exchange pursuant to which the Common Stock is effectively converted into or exchanged for other securities, cash or property (except for issuances by reclassification contemplated by Section 12(a)(iv)), or (D) consummates a stock or share purchase agreement or other business combination (including a reorganization, recapitalization, spin-off or scheme of arrangement) with another Person or group of Persons whereby such other Person or group acquires more than fifty percent (50%) of the outstanding shares of Common Stock (not including any shares of Common Stock held by the other Person or group making or party to, or associated or affiliated with the other Persons making or party to, such stock or share purchase agreement or other business combination), or (ii) any, direct or indirect, purchase offer, tender offer or exchange offer (whether by the Company or another Person or group of Persons) is completed pursuant to which holders of Common Stock are permitted to sell, tender or exchange their shares for other securities, cash or property (each transaction or series of transactions referred to in clause (i) or (ii) above, a "Fundamental Transaction"); then, the Company shall provide to the Holder twenty (20) days advance written notice of such Fundamental Transaction, and this Warrant shall terminate unless exercised (if exercisable) prior to the date such Fundamental Transaction.

(c) Notice of Adjustment. Upon any adjustment of the Exercise Price, and from time to time upon the request of the Holder, the Company shall furnish to the Holder the Exercise Price resulting from such adjustment or otherwise in effect and the number of Warrant Shares then available for purchase under this Warrant, setting forth in reasonable detail the method of calculation and the facts upon which such calculation is based.

13. No Fractional Shares. No fractional shares of Common Stock will be issued in connection with any exercise of this Warrant. In lieu of any fractional shares which would otherwise be issuable, the Company shall pay the Holder an amount of cash equal to the product of such fraction multiplied by the closing price of one share of Common Stock as reported on the principal trading market for the Common Stock on the Date of Exercise.

14. No Impairment. The Company shall not by any action including, without limitation, amending its Certificate of Incorporation, any reorganization, transfer of assets, consolidation, merger, dissolution, issue or sale of securities or any other voluntary action, avoid or seek to avoid the observance or performance of any of the terms of this Warrant, but shall at all times in good faith assist in the carrying out of all such terms and in the taking of all such action, as may be necessary or appropriate to protect the rights of the Holder against impairment. Without limiting the generality of the foregoing, the Company shall take all such action as may be necessary or appropriate in order that the Company may validly issue fully paid and nonassessable shares of Common Stock upon the exercise of this Warrant at the then Exercise Price therefor.

15. No Rights as a Stockholder; Notice to Holder. Nothing contained in this Warrant shall be construed as conferring upon the Holder the right to vote or to consent or to receive notice as a stockholder in respect of any meeting of stockholders for the election of directors of the Company or any other matter, or any rights whatsoever as a stockholder of the Company.

16. Warrant Agent. The Company shall serve as warrant agent under this Warrant. Upon thirty (30) days notice to the Holder, the Company may appoint a new warrant agent. Any corporation into which the Company or any new warrant agent may be merged or any corporation resulting from any consolidation to which the Company or any new warrant agent shall be a party or any corporation to which the Company or any new warrant agent transfers substantially all of its corporate trust or stockholders services business shall be a successor warrant agent under this Warrant without any further act. Any such successor warrant agent shall promptly cause notice of its succession as warrant agent to be mailed (by first class mail, postage prepaid) to the Holder at the Holder's last address as shown on the Warrant Register.

17. Miscellaneous.

(a) Notices. Any and all notices or other communications or deliveries hereunder (including any Exercise Notice) shall be in writing and shall be deemed given and effective on the earliest of (i) the date of transmission, if such notice or communication is delivered via facsimile at the facsimile number pursuant to this Section 17(a) prior to 5:30 p.m. (New York City time) on a trading day, (ii) the next trading day after the date of transmission, if such notice or communication is delivered via facsimile at the facsimile number specified pursuant to this Section 17(a) on a day that is not a trading day or later than 5:30 p.m. (New York City time) on any trading day, (iii) the second trading day following the date of mailing, if sent by nationally recognized overnight courier service to the street address specified pursuant to this Section 17(a), or (iv) upon actual receipt by the party to whom such notice is required to be given. The addresses for such communications shall be as follows:

(i) if to the Company, to:

GTx, Inc.
175 Toyota Plaza
7th Floor
Memphis Tennessee
Attn: Principal Financial Officer or Chief Legal Officer
Facsimile: (901) 844-8075

with a copy to (which shall not constitute notice to the Company):

Cooley LLP
3175 Hanover Street
Palo Alto, CA 94304
Attn: Chadwick L. Mills
Facsimile: (650) 849-7400

(ii) if to the Holder, to the address, facsimile number or email or street address appearing on the Warrant Register (which shall initially be the facsimile number and email and street address set forth for the initial Holder in the Purchase Agreement);

or to such other address, facsimile number or email address as the Company or the Holder may provide to the other in accordance with this Section 17(a).

(b) Assignment. Subject to the restrictions on transfer described herein, the rights and obligations of the Company and the Holder shall be binding upon, and inure to the benefit of, the successors, assigns, heirs, administrators and transferees of the parties. The Company shall not have the right directly or indirectly to assign or

transfer this Warrant without the prior written consent of the Holder, which may be withheld in the Holder's sole discretion, or as part of a Fundamental Transaction.

(c) No Third Party Beneficiaries. Nothing in this Warrant shall be construed to give to any Person other than the Company and the Holder any legal or equitable right, remedy or cause of action under this Warrant.

(d) Amendments; Waiver. This Warrant may be amended only in writing signed by the Company and the Holder, and any amendment so effected shall amend each Warrant issued pursuant to the Purchase Agreement and be binding upon each holder of such Warrants (provided, however, that any such amendment that adversely affects any holder or class of holders of such Warrants in a manner that does not apply uniformly to all holders of such Warrants, as applicable, shall require the written consent of such adversely affected holders or class). Any provision of this Warrant may be waived, but only if in writing by the party against whom enforcement of any such waiver is sought. No waiver of any default with respect to any provision, condition or requirement of this Warrant shall be deemed to be a continuing waiver in the future or a waiver of any subsequent default or a waiver of any other provision, condition or requirement hereof, nor shall any delay or omission of either party to exercise any right hereunder in any manner impair the exercise of any such right.

(e) Governing Law. This Warrant shall be governed by and construed in accordance with the laws of the State of Delaware, without regard to principles of conflict of laws.

(f) Severability. If one or more provisions of this Warrant are held to be unenforceable under applicable law in any respect, such provision shall be excluded from this Warrant and the balance of this Warrant shall be construed and interpreted as if such provision were so excluded and shall be enforceable in accordance with its remaining terms.

[*Remainder of Page Intentionally Left Blank; Signature Page Follows*]

IN WITNESS WHEREOF, the Company has caused this Warrant to be duly executed by its authorized officer as of the date first indicated above.

GTx, INC.

By: _____
Name: Marc S. Hanover
Title: President and Chief Operating Officer

[Signature Page — Warrant]

ATTACHMENT A

EXERCISE NOTICE

To GTx, Inc.:

The undersigned hereby irrevocably elects to purchase shares (the "Shares") of common stock, par value $0.001 per share ("Common Stock"), of GTx, Inc., a Delaware corporation, pursuant to Warrant No. CSW-[•], originally issued on March 6, 2014 (the "Warrant"). The undersigned elects to utilize the following manner of exercise:

Shares:

o Full Exercise of Warrant
o Partial Exercise of Warrant (in the amount of Shares)
Exercise Price: $
Manner of Exercise:

o Certified or Official Bank Check

o Intra-Bank Account Transfer
o Wire Transfer

[Please issue a new Warrant for the unexercised portion of the attached Warrant in the name of the [undersigned]/[the undersigned's nominee as is specified below].]

Date: _____
Full Name of Holder*: _____
Signature of Holder or Authorized Representative: _____
Name and Title of Authorized Representative†: _____
Additional Signature of Holder (if jointly held): _____
Social Security or Tax Identification Number: _____
Address of Holder: _____

Full Name of Nominee of Holder†: _____
Address of Nominee of Holder†: _____

* Must conform in all respects to name of holder as specified on the face of the Warrant.

† If applicable.

ATTACHMENT B

FORM OF ASSIGNMENT

[*To be completed and signed only upon transfer of Warrant*]

FOR VALUE RECEIVED, the undersigned hereby sells, assigns and transfers unto [·] the right represented by the attached Common Stock Warrant to purchase [·] shares of Common Stock of GTx, Inc., a Delaware corporation (the "Company"), to which the Warrant relates and appoints [·] as attorney to transfer said right on the books of the Company with full power of substitution in the premises.

Date: _____
Full Name of Holder*: _____
Signature of Holder or Authorized Representative: _____
Name and Title of Authorized Representative†: _____
Additional Signature of Holder (if jointly held): _____
Address of Holder: _____

Full Name of Transferee: _____
Address of Transferee: _____

In the presence of: _____

* Must conform in all respects to name of holder as specified on the face of the Warrant.

† If applicable.

CONFIDENTIAL SEPARATION AGREEMENT AND GENERAL RELEASE

This Confidential Separation Agreement and General Release ("**Agreement**" or **"Release"**) is entered into by and between Mark E. Mosteller ("**Employee**") and GTx, Inc. ("**GTx**" or the "**Company**").

A. Employee was employed by GTx from August 6, 2001 until December 31, 2013 **(date of "Separation from Service")** at which time his/her employment was terminated due to a reduction in force.

B. Though it has no obligation to do so, GTx desires to provide certain severance benefits to Employee in consideration of a final and complete resolution, with prejudice, of any and all matters between them relating to Employee's employment with GTx, the terms and conditions of that employment, and the termination of that employment.

NOW, THEREFORE, the Parties, in consideration of the above and the agreements and covenants herein, agree as follows:

1. **Payment.** As consideration for Employee's execution of this Agreement, Employee shall be entitled to receive the severance benefits set forth below (collectively, the "Severance Benefits"):

1.1. GTx shall pay Employee a gross amount equal to (i) Two Hundred Forty Thousand, Six Hundred Forty-Two and 75/100 dollars ($240,642.75**)** ("Amount"), equivalent to nine (9) months of Employee's base salary, and (ii) Fourteen Thousand Dollars (the "Additional Amount"), to partially offset the costs attributable to the loss of certain Employee benefits for a period of six (6) months.. The Amount and the Additional Amount, less applicable taxes and withholdings required by law, will be paid by check or direct deposit to Employee's bank account payable to Mark E. Mosteller within thirty (30) days following Employee's Separation from Service. GTx will issue Employee an IRS Form W-2 for the Amount. Employee expressly understands and agrees that (i) GTx shall not be required to make any further payment, for any reason whatsoever, to him/her or on his/her behalf regarding any claim or right whatsoever which might possibly be asserted by him/her, and (ii) GTx, by entering into this Agreement, in no way admits that it treated him/her unlawfully or unfairly in any way. Employee acknowledges that this Agreement is not an admission of liability or fault by GTx, by whom liability and fault are expressly denied. Employee acknowledges that in the absence of this Agreement he/she has no entitlement to the Amount, the Additional Amount or any other consideration outlined herein.

1.2. GTx agrees that all currently unvested outstanding stock options previously granted to Employee, as described in Exhibit A attached hereto and made a part hereof, will immediately vest and become exercisable as of December 31, 2013. Additionally, the post-termination exercise period applicable to all of Employee's outstanding vested stock options will be extended for a period of two (2) years from his date of Separation from Service through, and including, December 31, 2015, subject in each case to the earlier expiration of the original term of the applicable stock option grant.

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2. **Release.** In consideration of the Severance Benefits in Section 1 and other good and valuable consideration, the receipt and sufficiency of which Employee hereby acknowledges, Employee releases and forever discharges GTx, its affiliates, and GTx's and its affiliates' members, officers, directors, shareholders, employees, successors, parents, agents, attorneys, insurers and assigns ("**Released Parties**"), from any and all claims, demands, obligations, or liabilities for injuries, death, losses and damages, whether personal, property or economic, whether now known or unknown, in any way arising out of or related to his/her employment with GTx, the terms and conditions of that employment, and the termination of employment, from the beginning of time up to and including the time of the signing this Agreement.

Employee represents that he/she has not filed or caused to be filed any lawsuit, complaint, or charge with respect to any claim this Agreement purports to waive, and he/she promises never to file or prosecute any lawsuit, complaint, claim for damages, or charge based on such claims. This provision will not apply to non-waivable charges or claims brought before a governmental agency. With respect to any such non-waivable claims, however, Employee agrees to waive his/her right (if any) to any monetary or other recovery, (including but not limited to reinstatement) should a governmental agency or other party pursue claims on his/her behalf, either individually or as part of any class or collective action, except where expressly prohibited by law, including the amount, if any, awarded to him/her by the Securities Exchange Commission ("SEC") under Section 922 of the Dodd-Frank Act for providing original and independent information to the SEC which leads to the successful recovery of fines and penalties by the SEC in excess of $1 Million. Employee understands that the claims he/she is releasing may arise under various laws and under any possible legal, equitable, statutory, common law, or tort theory, including, but not limited to:

2.1 Anti-discrimination statutes, such as the Age Discrimination in Employment Act ("**ADEA**") (which prohibits age discrimination in employment); Title VII of the Civil Rights Act of 1964 (which prohibits discrimination or harassment based on race, color, national origin, religion, or sex); the Equal Pay Act (which prohibits paying men and women unequal pay for equal work); the Americans With Disabilities Act, and the Rehabilitation Act of 1973 (which prohibits discrimination based on disability); 42 USC Section 1981 (which prohibits discrimination based on race); the Genetic Information Nondiscrimination Act of 2008 (which prohibits discrimination based on an employee's genetic information); the Tennessee Handicapped Discrimination Act, § 8-50-103, *et seq*. (which prohibits discrimination based solely upon any physical, mental or visual handicap of the applicant, or because such person uses a guide dog); the Tennessee Human Rights Act, § 4-21-101, *et seq*. (which prohibits discrimination based on race, creed, color, religion, sex, age or national origin); and any other federal, state, or local law prohibiting employment discrimination, harassment, or retaliation of any kind.

2.2 Other laws, such as the Family and Medical Leave Act of 1993 ("FMLA"), which requires employers to provide leaves of absence under certain circumstances; the Worker Adjustment and Retraining Notification Act ("WARN"), which requires that advance notice be given of certain work force reductions; the Tennessee Plant Closings and Reduction in Operations statute; the Fair Credit Reporting Act; the Occupational Safety and Health Act; any federal, state, or local laws restricting an employer's right to terminate employees, or otherwise

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regulating employment; whistleblowing laws; the Sarbanes-Oxley Act, which prohibits retaliation against employees/whistleblowers (including those who report or cause to be reported and those who assist in investigations) concerning alleged violations pertaining to certain types of fraud, including securities

fraud, involving public companies; any federal, state, or local laws enforcing express or implied employment contracts or requiring employers to deal with employees fairly or in good faith; and any wage payment and collection law.

2.3 Tort and contract claims, such as claims for wrongful or constructive discharge, retaliatory discharge, negligence, physical or personal injury, emotional distress, fraud, fraud in the inducement, negligent misrepresentation, defamation, invasion of privacy, interference with contract or with prospective economic advantage, breach of oral, express or implied contract, breach of covenants of good faith and fair dealing, and similar or related claims.

2.4 Other released claims, include, without limitation, claims: (i) under the Employee Retirement Income Security Act of 1974; (ii) for compensation, stock options, bonuses, or lost wages; (iii) in any way related to design or administration of any employee benefit program; (iv) for severance or similar benefits or for post-employment health or group insurance benefits; (v) for fees, costs, or expenses of any attorneys who represent or have represented Employee; or (vi) any other state, federal or local laws relating to employment.

2.5 Unknown claims: Employee understands that he/she is releasing the Released Parties from claims that he/she may not know about as of the date hereof and that this is his/her knowing and voluntary intent even though someday he/she might learn that some or all of the facts he/she currently believes to be true are untrue and even though he/she might then regret having signed this Agreement. Employee is expressly assuming that risk and agrees that this Agreement shall remain effective in all respects in any such case. Employee expressly waives all rights he/she might have under any law that is intended to protect him/her from waiving unknown claims, and Employee understands the significance of doing so. However, nothing in this Release prevents or waives Employee's right to challenge the validity of this Release under the ADEA as amended by the Older Workers Benefit Protection Act or otherwise.

3. **Representations/Warranties.** Employee represents, warrants, and covenants that he/she:
(i) has not sold, assigned or transferred any claim he/she is purporting to release, nor attempted to do so;
(ii) has the full legal authority to enter into this Agreement for himself/herself and his/her estate and requires no approval of anyone else;
(iii) relied on, or had the opportunity to obtain, the advice of attorneys of his/her choice concerning legal and tax consequences;
(iv) has completely read this Agreement, and/or had it explained to Employee by his/her attorney, if any; and
(v) fully understands and voluntarily accepts the terms of the Agreement.

4. **FMLA and FLSA Rights Honored.** Employee acknowledges that he/she has received all of the leave from work for family and/or personal medical reasons and/or other benefits to which he/she believes he/she is entitled under GTx's policy and FMLA; that Employee has no pending request for FMLA leave; that GTx has not mistreated Employee in any way because of

any illness or injury to Employee or any member of his/her family; and that Employee has received all monetary compensation, including hourly wages, salary and/or overtime compensation, to which he/she believes he/she is entitled under the Fair Labor Standards Act ("FLSA").

5. **ADEA Release Requirements Satisfied.** Employee understands that this Agreement has to meet certain requirements to validly release any ADEA claims Employee might have, and Employee represents and warrants that all such requirements have been satisfied. ***GTx hereby advises Employee that before signing this Agreement, he/she may take forty-five (45) days to consider this Agreement***. Employee acknowledges that: (i) he/she took advantage of as much of this period to consider this Agreement as he/she wished before signing; (ii) he/she carefully read this Agreement; (iii) he/she fully understands it; (iv) he/she entered into this Agreement knowingly and voluntarily (free from fraud, duress, coercion, or mistake of fact); (v) this Agreement is in writing and is understandable; (vi) in this Agreement, he/she waives current ADEA claims; (vii) he/she has not waived future ADEA claims that may arise after the date of execution of this Agreement; (viii) he/she is receiving valuable consideration in exchange for execution of this Agreement that he/she would not otherwise be entitled to receive; (ix) GTx hereby advises Employee in writing to discuss this Agreement with his/her attorney (at his/her own expense) prior to execution, and he/she has done so to the extent he/she deemed appropriate; and (x) he/she received with this Agreement, GTx's Cumulative Information Disclosure Notice Concerning Reduction In Force.

6. **Agreement on Condition of Employment, and Confidentiality Agreement.**

6.1 Agreement on Condition of Employment: Employee agrees that the Agreement on Condition of Employment which Employee signed on March 1, 2013 shall survive this Agreement and shall remain in full force and effect. Employee hereby reconfirms, acknowledges and ratifies all obligations he/she has to GTx, including those set forth in the "Agreement on Condition of Employment."

6.2 Confidentiality Agreement: Employee agrees that the Confidentiality Agreement which Employee signed on August 6, 2001 as a prospective employee shall survive this Agreement and shall remain in full force and effect. Employee hereby reconfirms, acknowledges and ratifies all obligations he/she has to GTx, including those set forth in the "Confidentiality Agreement."

6.3 Return**:** Except for such equipment as GTx shall have agreed in writing with Employee that Employee shall not be required to return to GTx, Employee represents that with this Agreement, he/she has returned all GTx property in his/her possession without exception and in whatsoever form including without limitation Information, Proprietary Information and Items, all property, things, documents, lab notebooks, reports, results, any and all documents with GTx information, records, emails, documents in electronic form, keys, pass cards, passwords, credit cards, computers, cell phones, handheld devices, disks, and other media of any kind relating to GTx and/or its customers, prospects and/or vendors, and any copies, in whole or part, whether or not prepared by him/her, all of which are the sole and exclusive property of GTx.

6.4 Release Confidentiality: Except for such information as the Company shall disclose in its public filings, Employee shall not disclose the terms of this Agreement or the Severance Benefits to anyone other than his/her spouse and his/her attorney or financial advisors and, even then, only if they agree to maintain the confidentiality thereof. Such person's disclosure of such information to any third party is a violation of this Agreement by Employee. This section does not prohibit disclosure of the terms of this Agreement or the Severance Benefits to the extent necessary to enforce this Agreement or to the extent otherwise legally required.

7. **Review & Revocation.**

7.1 Review: ***Before executing this Agreement, Employee may take 45 days to consider this Agreement***. Employee acknowledges and agrees that his/her waiver of rights under this Agreement is knowing and voluntary and complies in full with all criteria of the regulations promulgated under the Age Discrimination in Employment Act, the Older Workers Benefit Protection Act, Title VII of the Civil Rights Act of 1964, and any and all federal, state and local laws, regulations, and orders. ***GTx hereby advises Employee in writing to consult with an attorney prior to executing this Agreement***. In the event that Employee executes this Agreement prior to the expiration of the 45-day period, he/she acknowledges that his/her execution was knowing and voluntary and not induced in any way by GTx or any other person.

7.2 Revocation**:** For a period of 7 days following his/her execution of this Agreement, Employee may revoke this Release. If he/she wishes to revoke this Release, he/she must revoke in writing delivered by hand or confirmed facsimile prior to the end of the 7th day of the revocation period to **Debbie Ellis, HR Director, 175 Toyota Plaza, Suite 700, Memphis, Tennessee 38103 (901) 844-8076**(fax), or the revocation will not be effective. If Employee timely revokes this Agreement, all provisions hereof will be null and void, including the Severance Benefits in Section 1 above. If Employee does not advise **Debbie Ellis** in writing that he/she revokes this Release within 7 days of his/her execution of it, this Release shall be forever enforceable. The 8th day following Employee's execution of this Agreement shall be the Effective Date of this Release. ***This Agreement is not effective or enforceable until the revocation period has expired.***

8. **Governing Law**. Except to the extent governed by federal law, this Agreement shall be governed by the laws of and deemed to have been executed in the State of Tennessee, without reference to conflict of law principles. This Agreement shall be deemed to be that negotiated and approved by both Parties and no rule of strict construction shall be applied against either party.

9. **Entire Agreement/Severability**. Subject to Sections 6.1 and 6.2 herein, this Agreement contains the entire agreement of and supersedes all prior discussions, negotiations, or agreements between the Parties. The Parties have not relied on any promise, representation, or warranty not expressly set forth herein. In the event that any word, phrase, sentence or provision violates any applicable statute, ordinance, or rule of law in any applicable jurisdiction, such provision shall be ineffective to the extent of such violation without invalidating any other provisions herein. The Parties agree that each term of this Agreement is contractual and not merely a recital.

10. **Successors**: Employee agrees that this Agreement binds all of his/her heirs, administrators, representatives, executors, successors, attorneys and assigns, and will inure to the benefit of all Released Parties and their respective heirs, administrators, representatives, executors, successors, and assigns.

11. **Waiver**: The failure of any party hereto to enforce at any time any of the provisions of this Agreement shall in no way be construed to be a waiver of any such provision or the right of any party thereafter to enforce each and every such provision. No waiver of any breach of this Agreement shall be held to be a waiver of any other or subsequent breach.

12. **Counterparts**. This Agreement may be signed in two counterparts, each of which shall be deemed an original when signed and shall constitute the same instrument. The Parties agree that signatures that are faxed, or scanned or sent by electronic mail, shall be considered original signatures for purposes of executing this Agreement.

Employee acknowledges that he/she carefully read this Agreement, he/she understands completely its contents, he/she understands the significance and consequences of signing it, and he/she intends to be legally bound by its terms. Employee acknowledges that he/she was given forty-five (45) days to consider executing this Agreement and that he/she has been advised in writing to review this Agreement with legal counsel prior to signing it. Employee certifies that he/she has agreed to and signed this Agreement voluntarily and as his/her own free will, act, and deed, and for full and sufficient consideration.

IN WITNESS WHEREOF, each of the Parties have executed on the dates set forth below.

Dated: December 20, 2013

/s/ Mark E. Mosteller
Employee

Dated: December 20, 2013

GTx, INC.

/s/ Henry P. Doggrell, Vice President, Chief Legal Officer
Name, Title

EXHIBIT A

GTx, Inc.

PERSONNEL SUMMARY
AS OF 11/30/2013
Current
Report Type: All
ID is equal to 000096

Name	ID	Grant Number	Grant Date	Plan/Type	Shares	Price	Exercised/ Released	Vested	Cancelled	Unvested	Outstanding/ Unreleased	Exercisable/ Releasable
Mosteller, Mark E	000096	0000125	8/6/2001	2000/NQ	25,500	$ 6.7800	2,500	2,500	23,000	0	0	0

0000126	4/11/2002	2001/NQ	17,000	$ 6.7800	0	0	17,000	0	0	0
0000127	8/1/2003	2002/NQ	17,000	$ 6.2400	17,000	17,000	0	0	0	0
0000128	9/1/2003	2002/NQ	25,500	$ 6.2400	0	0	25,500	0	0	0
0000129	7/28/2004	2001/NQ	10,000	$ 8.9000	0	10,000	0	0	10,000	10,000
0000130	7/27/2005	2002/NQ	25,000	$ 10.8600	0	25,000	0	0	25,000	25,000
0000941	1/1/2013	2004/NQ	55,000	$ 4.2000	0	0	0	55,000	55,000	0
0000296	1/1/2007	2004/NQ	18,400	$ 17.8400	0	18,400	0	0	18,400	18,400
0000380	1/1/2008	2004/NQ	25,000	$ 14.3500	0	25,000	0	0	25,000	25,000
0000509	1/1/2009	2004/NQ	25,000	$ 16.8400	0	16,667	0	8,333	25,000	16,667
0000634	1/1/2010	2004/NQ	35,000	$ 4.2000	0	21,000	0	14,000	35,000	21,000
0000777	1/1/2011	2004/NQ	35,000	$ 2.6500	0	14,000	0	21,000	35,000	14,000
00000864	1/1/2012	2004/NQ	35,000	$ 3.3600	0	7,000	0	28,000	35,000	7,000
0000295	1/1/2007	2002/NQ	6,600	$ 17.8400	0	6,600	0	0	6,600	6,600
Name: Mosteller, Mark E			355,000		19,500	163,167	65,500	126,333	270,000	143,667
	TOTALS		355,000		19,500	163,167	65,500	126,333	270,000	143,667

Exhibit 10.31

2014 Compensation Information for Registrant's Executive Officers

The table below provides information regarding the base salary of each executive officer of GTx, Inc. (the "Company"), effective as of January 1, 2014:

Executive Officer	Title	2014 Annual Base Salary ($)
Mitchell S. Steiner	Chief Executive Officer and Vice-Chairman of the Board	452,088
Marc S. Hanover	President and Chief Operating Officer	393,317
James T. Dalton	Vice President, Chief Scientific Officer	375,000
Henry P. Doggrell	Vice President, Chief Legal Officer and Secretary	363,576

A description of the retention benefits and arrangements provided to the Company's executive officers is included under Item 5.02 of the Company's Current Report on Form 8-K, filed with the Securities and Exchange Commission on October 1, 2013, and is incorporated by reference herein.

SECURITIES PURCHASE AGREEMENT

This Securities Purchase Agreement (this "Agreement") is dated as of March 3, 2014, between GTx, Inc., a Delaware corporation (the "Company"), and each purchaser identified on Exhibit A hereto (each, including its successors and assigns, a "Purchaser" and collectively, the "Purchasers").

WHEREAS, on the terms and subject to the conditions set forth in this Agreement and pursuant to Section 4(2) of the Securities Act of 1933, as amended (the "Securities Act"), and Rule 506 promulgated thereunder, the Company desires to issue and sell to each Purchaser, and each Purchaser, severally and not jointly, desires to purchase from the Company, securities of the Company as more fully described in this Agreement.

NOW, THEREFORE, IN CONSIDERATION of the mutual covenants contained in this Agreement, and for other good and valuable consideration, the receipt and adequacy of which are hereby acknowledged, the Company and each Purchaser agree as follows:

ARTICLE 1
DEFINITIONS

1.1 Definitions. In addition to the terms defined elsewhere in this Agreement, for all purposes of this Agreement, the following terms have the meanings set forth in this Section 1.1:

"Action" means any action, suit, inquiry, notice of violation, proceeding or investigation before or by any court, arbitrator, governmental or administrative agency or regulatory authority (federal, state, county, local or foreign), including without limitation the Commission.

"Affiliate" means any Person that, directly or indirectly through one or more intermediaries, controls or is controlled by or is under common control with a Person, as such terms are used in and construed under Rule 405 under the Securities Act.

"Agreement" shall have the meaning ascribed to such term in the preamble.

"Auditors" shall have the meaning ascribed to such term in Section 3.11.

"Benefit Plans" shall have the meaning ascribed to such term in Section 3.3.

"Board of Directors" means the board of directors of the Company.

"Business Day" means any day except any Saturday, any Sunday, any day which is a federal legal holiday in the United States or any day on which banking institutions in the State of New York are authorized or required by law or other governmental action to close.

"Closing" shall have the meaning ascribed to such term in Section 2.2.

"Closing Date" shall have the meaning ascribed to such term in Section 2.2.

"Commission" means the United States Securities and Exchange Commission.

"Common Stock" means the common stock of the Company, par value $0.001 per share, and any other class of securities into which such securities may hereafter be reclassified or changed.

"Common Stock Equivalents" means any securities of the Company which would entitle the holder thereof to acquire at any time Common Stock, including, without limitation, any debt, preferred stock, right, option, warrant or other instrument that is at any time convertible into or exercisable or exchangeable for, or otherwise entitles the holder thereof to receive, Common Stock.

"Company" shall have the meaning ascribed to such term in the preamble.

"Derivative Transaction" shall have the meaning ascribed to such term in Section 4.2.

"Disclosure Schedules" shall have the meaning ascribed to such term in ARTICLE III.

"Effective Date" means the earliest of the date that (a) the initial Registration Statement has been declared effective by the Commission, (b) all of the Registrable Securities have been sold pursuant to Rule 144 or may be sold pursuant to Rule 144 without the requirement for the Company to be in compliance with the current public information required under Rule 144 and without volume or manner-of-sale restrictions or (c) following the one year anniversary of the Closing Date provided that a holder of Registrable Securities is not an Affiliate of the Company, all of the Registrable Securities may be sold pursuant to an exemption from registration under Section 4(1) of the Securities Act without volume or manner-of-sale restrictions and Company counsel has delivered to such holders a standing written unqualified opinion that resales may then be made by such holders of the Registrable Securities pursuant to such exemption which opinion shall be in form and substance reasonably acceptable to such holders.

"Environmental Laws" shall have the meaning ascribed to such term in Section 3.17.

"ERISA" shall have the meaning ascribed to such term in Section 3.17.

"Evaluation Date" shall have the meaning ascribed to such term in Section 3.10.

"Except as disclosed in the SEC Reports" shall be construed to mean only those matters that are reasonably apparent and fairly disclosed in the SEC Reports (excluding any disclosures set forth in any risk factor section and in any section relating to forward-looking statements to the extent they are

cautionary, predictive or forward-looking). For purposes of this definition, "SEC Reports" shall only include SEC Reports filed with or furnished to the Commission since January 1, 2012.

"Exchange Act" means the Securities Exchange Act of 1934, as amended, and the rules and regulations of the Commission promulgated thereunder.

"FCPA" means the Foreign Corrupt Practices Act of 1977, as amended.

"FDA" means the U.S. Food and Drug Administration.

"GAAP" shall have the meaning ascribed to such term in Section 3.8.

"Intellectual Property" means (i) worldwide patents, patent applications, invention disclosures and other rights of invention, filed with any governmental authority, and all reissues, divisions, renewals, extensions, provisionals, continuations and continuations-in-part thereof and all reexamined patents or other applications or patents claiming the benefit of the filing date of any of the foregoing; (ii) worldwide (A) registered trademarks and service marks and registrations and applications for such registrations, and (B) unregistered trademarks and service marks, trade names, fictitious business names, corporate names, trade dress, logos, product names and slogans, including any common law rights; in each case together with the goodwill associated therewith; (iii) worldwide (A) registered copyrights in published or unpublished works, mask work rights and similar rights, including rights created under Sections 901-914 of Title 17 of the United States Code, mask work registrations, and copyright applications for registration, including any renewals thereof, and (B) any unregistered copyrightable works and other rights of authorship in published or unpublished works; (iv) worldwide (A) internet domain names; (B) website content; (C) telephone numbers; and (D) moral rights and publicity rights; (v) any computer program or other software (irrespective of the type of hardware for which it is intended), including firmware and other software embedded in hardware devices, whether in the form of source code, assembly code, script, interpreted language, instruction sets or binary or object code (including compiled and executable programs), including any library,

component or module of any of the foregoing, including, in the case of source code, any related images, videos, icons, audio or other multimedia data or files, data files, and header, development or compilations tools, scripts, and files, and (vi) worldwide confidential or proprietary information or trade secrets, including technical information, inventions and discoveries (whether or not patentable and whether or not reduced to practice) and improvements thereto, know-how, processes, discoveries, developments, designs, techniques, plans, schematics, drawings, formulae, preparations, assays, surface coatings, diagnostic systems and methods, patterns, compilations, databases, database schemas, specifications, technical data, inventions, concepts, ideas, devices, methods, and processes; and includes any rights to exclude others from using or appropriating any Intellectual Property Rights, including the rights to sue for or assets claims against and remedies against past, present or future infringements or misappropriations of any or all of the foregoing and rights of priority and protection of interests therein, and any other proprietary, intellectual property or other rights relating to any or all of the foregoing anywhere in the world.

"Legend Removal Date" shall have the meaning ascribed to such term in Section 5.1(c).

"Lien" means any deed of trust, mortgage, pledge, hypothecation, assignment, deposit or preferential arrangement, right of first refusal, charge, encumbrance, lien, statutory lien of any kind or nature (including landlord's, warehousemen's, carriers', mechanics', suppliers', materialmen's, repairmen's or other like liens), or other security agreement or security interest of any kind or nature whatsoever, including any conditional sale or other title retention agreement and any capital or financing lease having substantially the same economic effect as any of the foregoing, but excluding any non-exclusive license of intellectual property and any restriction imposed under applicable securities laws.

"Material Adverse Effect" means a material adverse effect on (i) the assets, liabilities, results of operations, condition (financial or otherwise), business, or prospects of the Company taken as a whole, or (ii) the ability of the Company to perform its obligations under the Transaction Documents.

"Nasdaq" means The NASDAQ Stock Market, LLC.

"Person" means an individual or corporation, partnership, trust, incorporated or unincorporated association, joint venture, limited liability company, joint stock company, government (or an agency or subdivision thereof) or other entity of any kind.

"Price Per Unit" shall have the meaning ascribed to such term in Section 2.1.

"Principal Purchasers" means The Pyramid Peak Foundation and J.R. Hyde, III and their respective successors and assigns.

"Purchaser Party" shall have the meaning ascribed to such term in Section 5.7.

"Purchasers" shall have the meaning ascribed to such term in the preamble.

"Registration Rights Agreement" means the Registration Rights Agreement, dated as of the Closing Date, among the Company and the Purchasers, in the form of Exhibit B attached hereto.

"Registration Statement" means a registration statement meeting the requirements set forth in the Registration Rights Agreement and covering the resale by the Purchasers of the Shares and the Warrant Shares.

"Rule 144" means Rule 144 promulgated by the Commission pursuant to the Securities Act, as such Rule may be amended or interpreted from time to time, or any similar rule or regulation hereafter adopted by the Commission having substantially the same purpose and effect as such Rule.

"Rule 506(d) Related Party" shall have the meaning ascribed to such term in Section 4.2.

"SEC Reports" shall have the meaning ascribed to such term in Section 3.7.

"Securities" means the Units, Shares, the Warrants and the Warrant Shares.

"Securities Act" means the Securities Act of 1933, as amended, and the rules and regulations of the Commission promulgated thereunder.

"Shares" shall have the meaning ascribed to such term in Section 2.1.

"Trading Day" means a day on which the principal Trading Market is open for trading.

"Trading Market" means any of the following markets or exchanges on which the Common Stock is listed or quoted for trading on the date in question: the NYSE MKT, the Nasdaq Capital Market, the Nasdaq Global Market, the Nasdaq Global Select Market, the New York Stock Exchange (or any successors to any of the foregoing).

"Transaction Documents" means this Agreement, the Warrants, the Registration Rights Agreement, all exhibits and schedules thereto and hereto and any other documents or agreements executed in connection with the transactions contemplated hereunder.

"Transfer Agent" means Computershare Trust Company, N.A., the current transfer agent of the Company, with a mailing address of 250 Royall Street, Canton, Massachusetts 02021, and a telephone number of 1-877-282-1168, and any successor transfer agent of the Company.

"Units" shall have the meaning ascribed to such term in Section 2.1.

"Unit Purchase Price" shall have the meaning ascribed to such term in Section 2.1.

"Voting Commitment" shall have the meaning ascribed to such term in Section 4.2.

"Warrant Purchase Price" shall have the meaning ascribed to such term in Section 2.1.

"Warrant Shares" shall have the meaning ascribed to such term in Section 2.1.

"Warrants" shall have the meaning ascribed to such term in Section 2.1.

ARTICLE 2
PURCHASE AND SALE

2.1 Purchase and Sale. Upon the terms and subject to the conditions set forth in this Agreement, the Company shall issue and sell to the Purchasers, and the Purchasers shall, severally and not jointly, purchase from the Company an aggregate of 11,976,048 immediately separable Units (each a "Unit" and collectively, the "Units") in the amounts set forth opposite their respective names on Exhibit A, at a price per Unit equal to $ $1.77625001336 (the "Price Per Unit" and the purchase price for the Units, the "Unit Purchase Price"), each Unit consisting of one share of Common Stock (each a "Share" and collectively, the "Shares") and a warrant (each a "Warrant' and collectively, the "Warrant") to purchase 0.85 of a share of Common Stock (collectively, the "Warrant Shares").

2.2 Closing. The Company agrees to issue and sell to the Purchasers and, in consideration of and in express reliance upon the representations, warranties, covenants, terms and conditions of this Agreement, the Purchasers agree, severally and not jointly, to purchase the Units. The closing of the purchase and sale of the Units (the "Closing") shall take place at the offices of Cooley LLP located at 3175 Hanover Street, Palo Alto, California, three Business Days following the satisfaction or waiver of the conditions set forth in Section 2.4, or at such other time and place or on such date as the Principal Purchasers and the Company may agree upon (such date is

hereinafter referred to as the "Closing Date"). At the Closing, the Unit Purchase Price shall be paid by the applicable Purchasers in cash, by wire transfer of immediately available funds, to an account previously designated in writing by the Company against the issuance by the Company of the Units, and the Company shall instruct its transfer agent to deliver to the Purchasers, via physical certificate, the Shares comprising the Units such Purchasers are purchasing hereunder and shall deliver the Warrants to the Purchasers.

2.3 Deliveries.

(a) On or prior to the Closing Date, the Company shall deliver or cause to be delivered to each Purchaser the following:

(i) this Agreement duly executed by the Company;

(ii) the Registration Rights Agreement duly executed by the Company;

(iii) a copy of the irrevocable instructions to the Transfer Agent instructing the Transfer Agent to deliver to such Purchaser, on an expedited basis, a certificate evidencing the number of Shares set forth opposite such Purchaser's name on Exhibit A hereto, registered in the name of such Purchaser;

(iv) Warrants substantially in the form attached hereto as Exhibit C, registered in the name of such Purchaser to purchase up to a number of shares of Common Stock set forth opposite their respective names on Exhibit A hereto (such Warrant certificates to be delivered as promptly as practicable after the Closing Date but no in no event more than five Trading Days after the Closing Date);

(v) the Company shall have delivered a Certificate, executed on behalf of the Company by its Chief Executive Officer and its acting Chief Financial Officer, dated as of the Closing Date, certifying to the fulfillment of the conditions specified in subsections (i), (ii), (iv), (v), (vi) and

(vii) of Section 2.4(b);

(vi) the Company shall have delivered a Certificate, executed on behalf of the Company by its Secretary, dated as of the Closing Date, certifying the resolutions adopted by the Board of Directors of the Company approving the transactions contemplated by this Agreement and the other Transaction Documents and the issuance of the Securities, certifying the current versions of the Certificate of Incorporation and Bylaws of the Company and certifying as to the signatures and authority of persons signing the Transaction Documents and related documents on behalf of the Company;

(vii) the Company shall have requested and caused Cooley LLP, counsel for the Company, to have furnished to the Purchasers, a customary legal opinion reasonably satisfactory to the Purchasers; and

(viii) A Nasdaq Listing of Additional Shares notification form.

(b) On or prior to the Closing Date, each Purchaser shall deliver or cause to be delivered to the Company the following:

(i) this Agreement duly executed by such Purchaser;

(ii) the Registration Rights Agreement duly executed by such Purchaser; and

(iii) the Unit Purchase Price by wire transfer to the account specified by the Company.

2.4 Closing Conditions.

(a) The obligations of the Company hereunder with respect to any Purchaser in connection with the Closing are subject to the following conditions being met:

(i) the accuracy in all material respects on the Closing Date of the representations and warranties of such Purchaser contained herein (unless as of a specific date therein in which case they shall be accurate as of such date);

(ii) all obligations, covenants and agreements of such Purchaser required to be performed at or prior to the Closing Date shall have been performed in all material respects;

(iii) the delivery by such Purchaser of the items set forth in Section 2.3(b) of this Agreement; and

(iv) Nasdaq shall have raised no objection to the consummation of the transactions contemplated by the Transaction Documents in the absence of stockholder approval of such transactions.

(b) The respective obligations of the Purchasers hereunder in connection with the Closing are subject to the following conditions being met:

(i) the representations and warranties made by the Company in ARTICLE III hereof qualified as to materiality shall be true and correct as of the date hereof and the Closing Date, except to the extent any such representation or warranty expressly speaks as of an earlier date, in which case such representation or warranty shall be true and correct as of such earlier date, and, the representations and warranties made by the Company in ARTICLE III hereof not qualified as to materiality shall be true and correct in all material respects as of the date hereof and the Closing Date, except to the extent any such representation or warranty expressly speaks as of an earlier date, in which case such representation or warranty shall be true and correct in all material respects as of such earlier date;

(ii) all obligations, covenants and agreements of the Company required to be performed at or prior to the Closing Date, whether under this Agreement or the other Transaction Documents, shall have been performed in all material respects;

(iii) the delivery by the Company of the items set forth in Section 2.3(a) of this Agreement;

(iv) the Company shall have obtained any and all consents, permits, approvals, registrations and waivers necessary or appropriate for consummation of the purchase and sale of the Units and the consummation of the other transactions contemplated by the Transaction Documents, all of which shall be in full force and effect, except for such that could not reasonably be expected to have a Material Adverse Effect;

(v) no judgment, writ, order, injunction, award or decree of or by any court, or judge, justice or magistrate, including any bankruptcy court or judge, or any order of or by any governmental authority, shall have been issued, and no action or proceeding shall have been instituted by any governmental authority, enjoining or preventing the consummation of the transactions contemplated hereby or in the other Transaction Documents;

(vi) no stop order or suspension of trading shall have been imposed by Nasdaq, the Commission or any other governmental or regulatory body with respect to public trading in the Common Stock; and

(vii) Nasdaq shall have raised no objection to the consummation of the transactions contemplated by the Transaction Documents in the absence of stockholder approval of such transactions.

(viii) there shall have been no Material Adverse Effect with respect to the Company since the date hereof.

ARTICLE 3
REPRESENTATIONS AND WARRANTIES OF THE COMPANY

The Company hereby represents and warrants to the Purchasers that, except as otherwise disclosed to the Purchasers:

3.1 Organization, Good Standing and Qualification. The Company is a corporation duly organized, validly existing and in good standing under the laws of the jurisdiction of its incorporation and has all requisite corporate power and authority to carry on its business as now conducted and to own its properties. The Company is duly qualified to do business as a foreign corporation and is in good standing in each jurisdiction in which the conduct of its business or its ownership or leasing of property makes such qualification or leasing necessary unless the failure to so qualify has not had and could not reasonably be expected to have a Material Adverse Effect.

3.2 Authorization; Enforcement. The Company has all corporate right, power and authority to enter into the Transaction Documents and to consummate the transactions contemplated hereby and thereby. All corporate action on the part of the Company, its directors and stockholders necessary for the authorization, execution, delivery and performance of the Transaction Documents by the Company, the authorization, sale, issuance and delivery of the Securities contemplated herein and the performance of the Company's obligations hereunder and thereunder has been taken. The Transaction Documents have been (or upon delivery will have been) duly executed and delivered by the Company and constitute the legal, valid and binding obligation of the Company, enforceable against the Company in accordance with their terms, except: (i) as limited by general equitable principles and applicable bankruptcy, insolvency, reorganization, moratorium and other laws of general application affecting enforcement of creditors' rights generally, (ii) as limited by laws relating to the availability of specific performance, injunctive relief or other equitable remedies and (iii) insofar as indemnification and contribution provisions may be limited by applicable law

3.3 Capitalization. The authorized capital stock of the Company consists of 120,000,000 shares of Common Stock, of which 63,185,389 shares are outstanding as of the date hereof (prior to the issuance of the Units). All of the issued and outstanding shares of the Company's capital stock have been duly authorized and validly issued and are fully paid and nonassessable. Except (i) for options to purchase Common Stock or other equity awards (including restricted stock units) issued to employees and members of the Board of Directors pursuant to the equity compensation plans or arrangements disclosed in the SEC Reports, (ii) shares of capital stock issuable and reserved for issuance pursuant to securities exercisable for, or convertible into or exchangeable for any shares of capital stock of the Company disclosed in the SEC Reports, and (iii) as contemplated by this Agreement, there are no existing options, warrants, calls, preemptive (or similar) rights, subscriptions or other rights, agreements, arrangements or commitments of any character obligating the Company to issue, transfer or sell, or cause to be issued, transferred or sold, any shares of the capital stock of, or other equity interests in, the Company or any securities convertible into or exchangeable for such shares of capital stock or other equity interests, and there are no outstanding contractual obligations of the Company to repurchase, redeem or otherwise acquire any shares of its capital stock or other equity interests. The issue and sale of the Units will not result in the right of any holder of Company securities to adjust the exercise, conversion or exchange price under such securities.

3.4 Issuance; Reservation of Shares. The issuance of the Shares has been duly and validly authorized by all necessary corporate and stockholder action, and the Shares, when issued and paid for pursuant to this Agreement, will be validly issued, fully paid and non-assessable, and shall be free and clear of all encumbrances and restrictions (other than as provided in the Transaction Documents). The issuance of the Warrants has been duly and validly authorized by all necessary corporate and stockholder action, and the Warrant Shares, when issued upon the due exercise of the Warrants, will be validly issued, fully paid and nonassessable, and shall be free and clear of all encumbrances (other than as provided in the Transaction Documents). The Company has reserved, and will reserve,

at all times that the Warrants remain outstanding, such number of shares of Common Stock sufficient to enable the full exercise of the then outstanding Warrants.

3.5 No Conflicts. The execution, delivery and performance of the Transaction Documents by the Company and the issuance and sale of the Securities will not conflict with or result in a breach or violation of any of the terms and provisions of, or constitute a default under (i) the Company's Certificate of Incorporation or the Company's Bylaws, both as in effect on the date hereof (true and complete copies of which have been made available to the Purchasers through the EDGAR system), or (ii)(a) any statute, rule, regulation or order of any governmental agency or body or any court, domestic or foreign, having jurisdiction over the Company or any of its respective assets or properties, or (b) except for any such conflict, breach, violation or default that would not reasonably be expected to have a Material Adverse Effect, any material agreement or instrument to which the Company is a party or by which the Company is bound or to which any of their respective assets or properties is subject.

3.6 Filings, Consents and Approvals. The Company is not required to obtain any consent, waiver, authorization or order of, give any notice to, or make any filing or registration with, any court or other federal, state, local or other governmental authority or other Person in connection with the execution, delivery and performance by the Company of the Transaction Documents, other than filings that have been made, or will be made, or consents that have been obtained, or will be obtained, pursuant to the rules and regulations of Nasdaq, including a Nasdaq Listing of Additional Shares notification form, applicable state securities laws and post-sale filings pursuant to applicable state and federal securities laws which the Company undertakes to file or obtain within the applicable time periods.

3.7 SEC Reports. The Company has filed all reports, schedules, forms, statements and other documents required to be filed by the Company under the Securities Act and the Exchange Act, including pursuant to Section 13(a) or 15(d) thereof, since January 1, 2012 (the foregoing materials, including the exhibits thereto and documents incorporated by reference therein, being collectively referred to herein as the "SEC Reports") on a timely basis or has received a valid extension of such time of filing and has filed any such SEC Reports prior to the expiration of any such extension. As of their respective dates, the SEC Reports complied in all material respects with the requirements of the Securities Act and the Exchange Act, as applicable, and none of the SEC Reports, when filed, contained any untrue statement of a material fact or omitted to state a material fact required to be stated therein or necessary in order to make the statements therein, in the light of the circumstances under which they were made, not misleading. The Company has not received any letters of comment from the staff of the Commission that have not been satisfactorily resolved as of the date hereof.

3.8 Financial Statements. The financial statements of the Company included in the SEC Reports comply in all material respects with applicable accounting requirements and the rules and regulations of the Commission with respect thereto as in effect at the time of filing. Such financial statements have been prepared in accordance with United States generally accepted accounting principles applied on a consistent basis during the periods involved ("GAAP"), except as may be otherwise specified in such financial statements or the notes thereto and except that unaudited financial statements may not contain all footnotes required by GAAP, and fairly present in all material respects the financial position of the Company as of and for the dates thereof and

the results of operations and cash flows for the periods then ended, subject, in the case of unaudited statements, to normal, immaterial, year-end audit adjustments.

3.9 Material Changes; Undisclosed Events, Liabilities or Developments. Since the date of the latest audited financial statements included within the SEC Reports, except as specifically disclosed in a subsequent SEC Report filed prior to the date hereof: (i) there has been no event, occurrence or development that has had or that could reasonably be expected to have a Material Adverse Effect, (ii) the Company has not incurred any liabilities (contingent or otherwise) other than (A) trade payables and accrued expenses incurred in the ordinary course of business consistent with past practice and (B) liabilities not required to be reflected in the Company's financial statements pursuant to GAAP or disclosed in filings made with the Commission, (iii) the Company has not altered its method of accounting or changed its principal registered public accounting firm, (iv) the Company has not

declared or made any dividend or distribution of cash or other property to its stockholders or purchased, redeemed or made any agreements to purchase or redeem any shares of its capital stock and (v) the Company has not issued any equity securities, except pursuant to existing Company equity compensation plans. The Company does not have pending before the Commission any request for confidential treatment of information.

3.10 Internal Controls. The Company maintains a system of internal control over financial reporting (as such term is defined in Rule 13a-15(f) of the Exchange Act) that complies with the requirements of the Exchange Act and has been designed by the Company's principal executive officer and principal financial officer, or under their supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles; the Company's internal control over financial reporting is effective and the Company is not aware of any material weaknesses in its internal control over financial reporting; there has been no fraud, whether or not material, that involves management or other employees who have a significant role in the Company's internal control over financial reporting; since the date of the latest audited financial statements included or incorporated by reference in the Company's SEC Reports, there has been no change in the Company's internal control over financial reporting that has materially affected, or is reasonably likely to materially affect, the Company's internal control over financial reporting. The Company maintains disclosure controls and procedures (as such term is defined in Rule 13a-15(e) of the Exchange Act) that comply with the requirements of the Exchange Act; such disclosure controls and procedures have been designed to ensure that material information relating to the Company is made known to the Company's principal executive officer and principal financial officer by others within those entities; such disclosure controls and procedures are effective. The Company is in compliance in all material respects with all applicable provisions of the Sarbanes-Oxley Act of 2002 and the rules and regulations promulgated by the Commission thereunder (the "Sarbanes-Oxley Act")..

3.11 Accountant. To the Company's knowledge, Ernst & Young LLP (the "Auditor"), which has expressed its opinion with respect to the Company's financial statements as of December 31, 2012 and 2011, respectively, and included in the SEC Reports (including the related notes), is an independent registered public accounting firm as required by the Act and the Public Company Accounting Oversight Board (United States). Ernst & Young LLP have not been engaged by the Company to perform any "prohibited activities" (as defined in Section 10A of the Exchange Act).

3.12 Litigation. Except as set forth in the SEC Reports, there is not pending or, to the knowledge of the Company, threatened or contemplated, any action, suit or proceeding to which the Company is a party or of which any property or assets of the Company is the subject before or by any court or governmental agency, authority or body, or any arbitrator, which, individually or in the aggregate, could reasonably be expected to result in any Material Adverse Effect. There are no current or pending legal, governmental or regulatory actions, suits or proceedings that are required to be described in the SEC Reports that have not been so described.

3.13 Tax Matters. The Company has filed all federal, state, local and foreign income and franchise tax returns required to be filed or has requested extensions thereof (except in any case in which the failure so to file would not have a Material Adverse Effect), except as set forth in the SEC reports and has paid all taxes required to be paid by it and any other assessment, fine or penalty levied against it, to the extent that any of the foregoing is due and payable, except for any such assessment, fine or penalty that is currently being contested in good faith or as would not have a Material Adverse Effect, except as set forth in or contemplated in the SEC Reports.

3.14 Insurance. The Company maintains in full force and effect insurance coverage that is customary for comparably situated companies for the business being conducted and properties owned or leased by the Company, and the Company reasonably believes such insurance coverage to be adequate against all liabilities, claims and risks against which it is customary for comparably situated companies to insure.

3.15 Environmental Matters. The Company (A) is in compliance with any and all applicable federal, state, local and foreign laws, rules, regulations, decisions and orders relating to the protection of human health and safety, the environment or hazardous or toxic substances or wastes, pollutants or contaminants (collectively,

"Environmental Laws"); (B) has received and is in material compliance with all permits, licenses or other approvals required of it under applicable Environmental Laws to conduct its business; and (C) has not received notice of any actual or potential liability for the investigation or remediation of any disposal or release of hazardous or toxic substances or wastes, pollutants or contaminants, except in any such case for any such failure to comply, or failure to receive required permits, licenses or approvals, or liability as would not, individually or in the aggregate, have a Material Adverse Effect.

3.16 Labor Relations. The Company (A) is in compliance, in all material respects, with any and all applicable foreign, federal, state and local laws, rules, regulations, treaties, statutes and codes promulgated by any and all governmental authorities (including pursuant to the Occupational Health and Safety Act) relating to the protection of human health and safety in the workplace ("Occupational Laws"); (B) has received all material permits, licenses or other approvals required of it under applicable Occupational Laws to conduct their business as currently conducted; and (C) is in compliance, in all material respects, with all terms and conditions of such permits, licenses or approvals. No action, proceeding, revocation proceeding, writ, injunction or claim is pending or, to the Company's knowledge, threatened against the Company relating to Occupational Laws, and the Company does not have knowledge of any material facts, circumstances or developments relating to its operations or cost accounting practices that could reasonably be expected to form the basis for or give rise to such actions, suits, investigations or proceedings. Each employee benefit plan, within the meaning of Section 3(3) of the Employee Retirement Income Security Act of 1974, as amended ("ERISA"), that is maintained, administered or contributed to by the Company or any of its affiliates for employees or former employees of the Company and has been maintained in material compliance with its terms and the requirements of any applicable statutes, orders,

rules and regulations, including but not limited to, ERISA and the Internal Revenue Code of 1986, as amended (the "Code"). No prohibited transaction, within the meaning of Section 406 of ERISA or Section 4975 of the Code, has occurred with respect to any such plan excluding transactions effected pursuant to a statutory or administrative exemption; and for each such plan that is subject to the funding rules of Section 412 of the Code or Section 302 of ERISA, no "accumulated funding deficiency" as defined in Section 412 of the Code has been incurred, whether or not waived, and the fair market value of the assets of each such plan (excluding for these purposes accrued but unpaid contributions) exceeds the present value of all benefits accrued under such plan determined using reasonable actuarial assumptions.

3.17 Certificates, Authorities and Permits. The Company holds, and is operating in compliance in all material respects with, all registrations, approvals, certificates, authorizations and permits of any governmental authority or self-regulatory body required for the conduct of its business as described in the SEC Reports, including without limitation, all such registrations, approvals, certificates, authorizations and permits required by the FDA or any other federal, state, local or foreign agencies or bodies engaged in the regulation of pharmaceuticals or biohazardous substances or materials; and the Company has not received notice of any revocation or modification of any such registration, approval, certificate, authorization and permit or has reason to believe that any such registration, approval, certificate, authorization and permit will not be renewed in the ordinary course that could lead to, the withdrawal, revocation, suspension, modification or termination of any such registration, approval, certificate, authorization or permit, which, singly or in the aggregate, if the subject of an unfavorable decision, ruling or finding, could result in a Material Adverse Effect.

3.18 Title to Assets. The Company has good and marketable title to all property (whether real or personal) described in the SEC Reports as being owned by it, in each case free and clear of all liens, claims, security interests, other encumbrances or defects except as described in the SEC Reports, and except those that could not, individually or in the aggregate, reasonably be expected to have a Material Adverse Effect. The property held under lease by the Company is held under valid, subsisting and enforceable leases with only such exceptions with respect to any particular lease as do not interfere in any material respect with the conduct of the business of the Company.

3.19 Intellectual Property. The Company owns, possesses, or can acquire on reasonable terms, all Intellectual Property necessary for the conduct of its business as now conducted or as described in the SEC Reports to be conducted in all material respects, except as such failure to own, possess, or acquire such rights would not have a Material Adverse Effect. Except as set forth in the SEC Reports, (A) to the knowledge of the Company, there is no infringement, misappropriation or violation by third parties of any such Intellectual Property, except as such

infringement, misappropriation or violation would not have a Material Adverse Effect; (B) there is no pending or, to the knowledge of the Company, threatened action, suit, proceeding or claim by others challenging the Company's rights in or to any such Intellectual Property, and the Company is unaware of any material facts which would form a reasonable basis for any such claim; (C) the Intellectual Property owned by the Company, and to the knowledge of the Company, the Intellectual Property licensed to the Company, have not been adjudged invalid or unenforceable, in whole or in part, and there is no pending or threatened action, suit, proceeding or claim by others challenging the validity or scope of any such Intellectual Property, and the Company is unaware of any material facts which would form a reasonable basis for any such claim; (D) to the Company's knowledge, there is no pending or threatened action, suit, proceeding or claim by others that the Company infringes, misappropriates or otherwise violates any Intellectual Property or other proprietary rights of others, the Company has not received any written notice of such claim and the Company is unaware of any other material fact which would form a reasonable basis for any such claim; and (E) to the Company's knowledge, no Company employee is obligated under any contract (including licenses, covenants or commitments of any nature) or other agreement, or subject to any judgment, decree or order of any court or administrative agency, that would interfere with the use of such employee's best efforts to promote the interest of the Company or that would conflict with the Company's business; none of the execution and delivery of this Agreement, the carrying on of the Company's business by the employees of the Company, and the conduct of the Company's business as proposed, will conflict with or result in a breach of terms, conditions, or provisions of, or constitute a default under, any contract, covenant or instrument under which any such employee is now obligated; and it is not and will not be necessary to use any inventions, trade secrets or proprietary information of any of its consultants, or its employees (or persons it currently intends to hire) made prior to their employment by the Company, except for technology that is licensed to or owned by the Company.

3.20 FDA and Related Matters. The Company and, to the Company's knowledge, others who perform services on the Company's behalf have been and are in compliance with all applicable federal, state, local and foreign laws, rules, regulations, standards, orders and decrees governing their respective businesses, including without limitation, all regulations promulgated by the FDA or any other federal, state, local or foreign agencies or bodies engaged in the regulation of pharmaceuticals or biohazardous substances or materials, except where noncompliance would not, singly or in the aggregate, have a Material Adverse Effect; and the Company has not received any notice citing action or inaction by the Company or others who perform services on the Company's behalf that would constitute non-compliance with any applicable federal, state, local or foreign laws, rules, regulations or standards excepting, however, such actions that have heretofore been resolved to the satisfaction of such governmental entity. The tests and preclinical and clinical studies conducted by or on behalf of the Company that are described in the SEC Reports were and, if still pending, are being, conducted in all material respects in accordance with experimental protocols, procedures and controls generally used by qualified experts in the preclinical and clinical study of new drugs, and laws and regulations; the descriptions of the tests and preclinical and clinical studies, and results thereof, conducted by or on behalf of the Company contained in the SEC Reports are accurate in all material respects; the Company has not received any written notice or correspondence from the FDA or any foreign, state or local governmental body exercising comparable authority or any Institutional Review Board or comparable authority requiring the termination, suspension, material modification or clinical hold of any tests or preclinical or clinical studies being conducted by or on behalf of the Company, which termination, suspension, material modification or clinical hold would reasonably be expected to have a Material Adverse Effect; and the Company has not received any written notices or correspondence from others concerning the termination, suspension, material modification or clinical hold of any tests or preclinical or clinical studies conducted by others on any active ingredient contained in the existing products of the Company or the products described in the SEC Reports as being under development, which termination, suspension, material modification or clinical hold would reasonably be expected to have a Material Adverse Effect.

3.21 Compliance with Nasdaq Continued Listing Requirements. The Company is, and has no reason to believe that it will not, upon the issuance of the Securities hereunder, continue to be, in compliance with the listing and maintenance requirements for continued listing on Nasdaq in all material respects. Assuming the representations and warranties of the Purchasers set forth in Section 4.2 are true and correct in all material respects, the consummation of the transactions contemplated by the Transaction Documents does not contravene the rules and regulations of Nasdaq. There are no proceedings pending or, to the Company's knowledge, threatened against the

Company relating to the continued listing of the Common Stock on Nasdaq and the Company has not received any notice of, nor to the Company's knowledge is there any basis for, the delisting of the Common Stock from Nasdaq.

3.22 Application of Takeover Protections. The Company and the Board of Directors have taken all necessary action, if any, in order to render inapplicable any control share acquisition, business combination, poison pill (including any distribution under a rights agreement) or other similar anti-takeover provision under the Company's certificate of incorporation (or similar charter documents) or the laws of its state of incorporation that would prevent the Purchasers or the Company from fulfilling their obligations or exercising their rights under the Transaction Documents, including without limitation as a result of the Company's issuance of the Securities and the Purchasers' ownership of the Securities and exercise in full of the Warrants.

3.23 Certain Fees. No brokerage or finder's fees or commissions are or will be payable by the Company to any broker, financial advisor or consultant, finder, placement agent, investment banker, bank or other Person with respect to the transactions contemplated by the Transaction Documents. The Purchasers shall have no obligation with respect to any fees or with respect to any claims made by or on behalf of other Persons for fees of a type contemplated in this Section that may be due in connection with the transactions contemplated by the Transaction Documents.

3.24 No Directed Selling Efforts or General Solicitation. Neither the Company nor any Person acting on its behalf has conducted any general solicitation or general advertising (as those terms are used in Regulation D) in connection with the offer or sale of any of the Securities.

3.25 No Integrated Offering. Neither the Company nor any of its Affiliates, nor any Person acting on its or their behalf has, directly or indirectly, made any offers or sales of any Company security or solicited any offers to buy any security, under circumstances that would adversely affect reliance by the Company on Section 4(2) for the exemption from registration for the transactions contemplated hereby or would require registration of the Shares under the Securities Act.

3.26 Private Placement. Assuming the accuracy of the Purchasers' representations and warranties set forth in ARTICLE IV, no registration under the Securities Act is required for the offer and sale of the Securities by the Company to the Purchasers as contemplated hereby.

3.27 Form S-3 Eligibility. The Company is eligible to register the resale of the Securities for resale by the Purchaser on Form S-3 promulgated under the Securities Act.

3.28 Investment Company. The Company is not and, after giving effect to the offering and sale of the Securities, will not be an "investment company," as such term is defined in the Investment Company Act of 1940, as amended.

3.29 Foreign Corrupt Practices. The Company, nor, to the best knowledge of the Company, any director, officer, agent, employee or other person associated with or acting on behalf of the Company has (A) used any corporate funds for any unlawful contribution, gift, entertainment or other unlawful expense relating to political activity; (B) made any direct or indirect unlawful payment to any foreign or domestic government official or employee from corporate funds; (C) violated or is in violation of any provision of the Foreign Corrupt Practices Act of 1977; or (D) made any bribe, rebate, payoff, influence payment, kickback or other unlawful payment.

3.30 Regulation M Compliance. The Company has not, and to its knowledge no one acting on its behalf has, (i) taken, directly or indirectly, any action designed to cause or to result in the stabilization or manipulation of the price of any security of the Company to facilitate the sale or resale of any of the Securities, (ii) sold, bid for, purchased, or paid any compensation for soliciting purchases of, any of the Securities, or (iii) paid or agreed to pay to any Person any compensation for soliciting another to purchase any other securities of the Company.

3.31 Disclosure. No representation or warranty by the Company in this Agreement and no statement contained in the SEC Reports or any certificate or other document furnished or to be furnished to the Purchasers pursuant to this Agreement contains any untrue statement of a material fact or omits to state a material fact necessary in order to make the statements contained therein, in light of the circumstances under which they were made, not misleading.

ARTICLE 4
REPRESENTATIONS AND WARRANTIES OF THE PURCHASERS

Each Purchaser, for itself and for no other Purchaser, hereby represents and warrants as of the date hereof and as of the Closing Date to the Company as follows (unless as of a specific date therein):

4.1 Organization; Authority. Such Purchaser is either an individual or an entity duly incorporated or formed, validly existing and in good standing under the laws of the jurisdiction of its incorporation or formation with full right, corporate, partnership, limited liability company or similar power and authority to enter into and to consummate the transactions contemplated by the Transaction Documents and otherwise to carry out its obligations hereunder and thereunder. The execution and delivery of the Transaction Documents and performance by such Purchaser of the transactions contemplated by the Transaction Documents have been duly authorized by all necessary corporate, partnership, limited liability company or similar action, as applicable, on the part of such Purchaser. Each Transaction Document to which it is a party has been duly executed by such Purchaser, and when delivered by such Purchaser in accordance with the terms hereof, will constitute the valid and legally binding obligation of such Purchaser, enforceable against it in accordance with its terms, except: (i) as limited by general equitable principles and applicable bankruptcy, insolvency, reorganization, moratorium and other laws of general application affecting enforcement of creditors' rights generally, (ii) as limited by laws relating to the availability of specific performance, injunctive relief or other equitable remedies and (iii) insofar as indemnification and contribution provisions may be limited by applicable law.

4.2 Purchaser Status.

(a) At the time such Purchaser was offered the Securities, it was, and as of the date hereof it is, and on each date on which it exercises the Warrants it will be, an "accredited investor" as defined in Rule 501 under the Securities Act. Such Purchaser is not a broker-dealer registered under Section 15 of the Exchange Act. Each Purchaser is acting alone in its determination as to whether to invest in the Securities. Each such Purchaser has delivered a questionnaire in form reasonably satisfactory to the Company with respect to the "bad actor" provisions of Rule 506(d) promulgated under the Securities Act. Each such Purchaser is not party to any voting agreements or similar arrangements with respect to the Securities, except the Registration

Rights Agreement, and that certain Amended and Restated Registration Rights Agreement dated as of August 7, 2003, as amended by that certain Waiver and Amendment Agreement, dated as of the date hereof. Each such Purchaser is not a member of a partnership, limited partnership, syndicate, or other group for the purpose of acquiring, holding, voting or disposing of the Securities, provided, that J.R. Hyde, III may be viewed as a group with those persons and entities as described on the Schedule 13D/A filed with the Commission by J.R. Hyde III on July 5, 2011. Each Purchaser represents and warrants that it (i) is not and will not become a party to (A) any agreement, arrangement or understanding with, and has not given any commitment or assurance to, any person or entity as to how such person, if serving as a director or if elected as a director of the Corporation, will act or vote on any issue or question (a "Voting Commitment") or (B) any Voting Commitment that could limit or interfere with such person's ability to comply, if serving as or elected as a director of the Company, with such person's fiduciary duties under applicable law; (ii) is not and will not become a party to any agreement, arrangement or understanding with any person or entity other than the corporation with respect to any direct or indirect compensation, reimbursement or indemnification in connection with service or action as a director of the Company.

(b) Each Purchaser has disclosed in writing to the Company a description of all Derivative Transactions (as defined below) by each such Purchaser in effect as of the date hereof, including the date of the transactions and the class, series and number of securities involved in, and the material economic terms of, such

Derivative Transactions. For purposes of this Agreement, a "Derivative Transaction" means any agreement, arrangement, interest or understanding entered into by, or on behalf or for the benefit of, any Purchaser or any of its affiliates or associates, whether record or beneficial: (w) the value of which is derived in whole or in part from the value of any class or series of shares or other securities of the Company, (x) which otherwise provides any direct or indirect opportunity to gain or share in any gain derived from a change in the value of securities of the Company, (y) the effect or intent of which is to mitigate loss, manage risk or benefit of security value or price changes, or (z) which provides the right to vote or increase or decrease the voting power of, such Purchaser, or any of its affiliates or associates, with respect to any securities of the Company, which agreement, arrangement, interest or understanding may include, without limitation, any option, warrant, debt position, note, bond, convertible security, swap, stock appreciation right, short position, profit interest, hedge, right to dividends, voting agreement, performance-related fee or arrangement to borrow or lend shares (whether or not subject to payment, settlement, exercise or conversion in any such class or series), and any proportionate interest of such Purchaser in the securities of the Company held by any general or limited partnership, or any limited liability company, of which such Purchaser is, directly or indirectly, a general partner or managing member.

4.3 General Solicitation. Such Purchaser is not purchasing the Securities as a result of any advertisement, article, notice or other communication regarding the Securities published in any newspaper, magazine or similar media or broadcast over television or radio or presented at any seminar or any other general solicitation or general advertisement.

4.4 Purchase Entirely for Own Account. The Securities to be received by such Purchaser hereunder will be acquired for such Purchaser's own account, not as nominee or agent, and not with a view to the resale or distribution of any part thereof in violation of the Securities Act, and such Purchaser has no present intention of selling, granting any participation in, or otherwise distributing the same in violation of the Securities Act without prejudice, however, to such Purchaser's right at all times to sell or otherwise dispose of all or any part of such Securities in compliance with applicable federal and state securities laws. Nothing contained herein shall be deemed a representation or warranty by such Purchaser to hold the Securities for any period of time.

4.5 Experience of Such Purchaser. Such Purchaser, either alone or together with its representatives, has such knowledge, sophistication and experience in business and financial matters so as to be capable of evaluating the merits and risks of the prospective investment in the Securities, and has so evaluated the merits and risks of such investment. Such Purchaser is able to bear the economic risk of an investment in the Securities and, at the present time, is able to afford a complete loss of such investment.

4.6 Disclosure of Information. Such Purchaser has had an opportunity to receive all information related to the Company requested by it and to ask questions of and receive answers from the Company regarding the Company, its business and the terms and conditions of the offering of the Securities. Such Purchaser acknowledges receipt of copies of the SEC Reports. Neither such inquiries nor any other due diligence investigation conducted by such Purchaser shall modify, limit or otherwise affect such Purchaser's right to rely on the Company's representations and warranties contained in this Agreement.

4.7 Interested Stockholders. Each Purchaser that is an "Interested Stockholder" (as such term is defined in Section 203 of the Delaware General Corporate Law) represents and warrants that it has been an Interested Stockholder for at least three years prior to the date hereof.

4.8 Restricted Securities. Such Purchaser understands that the Securities are "restricted securities" and have not been registered under the Securities Act and may not be offered, resold, pledged or otherwise transferred except (i) pursuant to an exemption from registration under the Securities Act or pursuant to an effective registration statement in compliance with Section 5 under the Securities Act and (ii) in accordance with all applicable securities laws of the states of the United States and other jurisdictions.

4.9 Commissions. No Person will have, as a result of the transactions contemplated by the Transaction Documents, any valid right, interest or claim against or upon the Company or a Purchaser for any

commission, fee or other compensation pursuant to any agreement, arrangement or understanding entered into by or on behalf of such Purchaser.

The Company acknowledges and agrees that the representations contained in ARTICLE IV shall not modify, amend or affect such Purchaser's right to rely on the Company's representations and warranties contained in this Agreement or any representations and warranties contained in any other Transaction Document or any other document or instrument executed and/or delivered in connection with this Agreement or the consummation of the transaction contemplated hereby.

ARTICLE 5
OTHER AGREEMENTS OF THE PARTIES

5.1 Transfer Restrictions.

(a) The Securities may only be disposed of in compliance with state and federal securities laws. In connection with any transfer of Securities other than pursuant to an effective registration statement under the Securities Act or Rule 144, to the Company or to an Affiliate of a Purchaser or in connection with a pledge as contemplated in Section 5.1(b), the Company may require the transferor thereof to provide to the Company an opinion of counsel selected by the transferor and reasonably acceptable to the Company, the form and substance of which opinion shall be reasonably satisfactory to the Company, to the effect that such transfer does not require registration of such transferred Securities under the Securities Act.

(b) The Purchasers agree to the imprinting, so long as is required by this Section 5.1, of a legend on any of the Securities in the following form:

THIS SECURITY HAS NOT BEEN REGISTERED WITH THE SECURITIES AND EXCHANGE COMMISSION OR THE SECURITIES COMMISSION OF ANY STATE IN RELIANCE UPON AN EXEMPTION FROM REGISTRATION UNDER THE SECURITIES ACT OF 1933, AS AMENDED (THE "SECURITIES ACT"), AND, ACCORDINGLY, MAY NOT BE OFFERED OR SOLD EXCEPT PURSUANT TO AN EFFECTIVE REGISTRATION STATEMENT UNDER THE SECURITIES ACT OR PURSUANT TO AN AVAILABLE EXEMPTION FROM, OR IN A TRANSACTION NOT SUBJECT TO, THE REGISTRATION REQUIREMENTS OF THE SECURITIES ACT AND IN ACCORDANCE WITH APPLICABLE STATE SECURITIES LAWS. THIS SECURITY MAY BE PLEDGED IN CONNECTION WITH A BONA FIDE MARGIN ACCOUNT.

(c) Certificates evidencing the Shares and Warrant Shares shall not contain any legend (including the legend set forth in Section 5.1(b) hereof), (i) while a registration statement (including the Registration Statement) covering the resale of such security is effective under the Securities Act, (ii) following any sale of such Shares or Warrant Shares pursuant to Rule 144, (iii) if such Shares or Warrant Shares are eligible for sale under Rule 144, without the requirement for the Company to be in compliance with the current public information required under Rule 144 as to such Shares and Warrant Shares and without volume or manner-of-sale restrictions, or (iv) if such legend is not required under applicable requirements of the Securities Act (including judicial interpretations and pronouncements issued by the staff of the Commission). The Company shall cause its counsel to issue a legal opinion to the Transfer Agent promptly after the Effective Date if required by the Transfer Agent to effect the removal of the legend hereunder. If all or any portion of a Warrant is exercised at a time when there is an effective registration statement to cover the resale of the Warrant Shares, or if such Shares or Warrant Shares may be sold under Rule 144 and the Company is then in compliance with the current public information required under Rule 144, or if the Shares or Warrant Shares may be sold under Rule 144 without the requirement for the Company to be in compliance with the current public information required under Rule 144 as to such Shares or Warrant Shares or if such legend is not otherwise required under applicable requirements of the Securities Act (including judicial interpretations and pronouncements issued by the staff of the Commission) then such Warrant Shares shall be issued free of all legends. The Company agrees that following the Effective Date or at such time as such legend is no longer required under this Section 5.1(c), it will, no later than three Trading Days following the delivery by a

Purchaser to the Company or the Transfer Agent of a certificate representing Shares or Warrant Shares, as the case may be, issued with a restrictive legend (such third Trading Day, the "Legend Removal Date"), deliver or cause to be delivered to such Purchaser a certificate representing such shares that is free from all restrictive and other legends. The Company may not make any notation on its records or give instructions to the Transfer Agent that enlarge the restrictions on transfer set forth in this Section 5.1. Certificates for Securities subject to legend removal hereunder shall be transmitted by the Transfer Agent to the Purchaser by crediting the account of the Purchaser's prime broker with the Depository Trust Company System as directed by such Purchaser.

5.2 Furnishing of Information; Public Information. Until the earliest of the time that (i) no Purchaser owns Securities or (ii) the Warrants have expired, the Company covenants to maintain the registration of the Common Stock under Section 12(b) of the Exchange Act and to timely file (or obtain extensions in respect thereof and file within the applicable grace period) all reports required to be filed by the Company after the date hereof pursuant to the Exchange Act.

5.3 Integration. The Company shall not sell, offer for sale or solicit offers to buy or otherwise negotiate in respect of any security (as defined in Section 2 of the Securities Act) that would be integrated with the offer or sale of the Securities in a manner that would require the registration under the Securities Act of the sale of the Securities or that would be integrated with the offer or sale of the Securities for purposes of the rules and regulations of Nasdaq such that it would require shareholder approval prior to the closing of such other transaction unless shareholder approval is obtained before the closing of such subsequent transaction. The Purchasers shall take no action to become a group such that any transactions contemplated by this Agreement would require shareholder approval prior to Closing.

5.4 Securities Laws Disclosure; Publicity. The Company shall (a) by 9:30 a.m. (New York City time) on the Trading Day immediately following the date hereof, issue a press release disclosing the material terms of the transactions contemplated hereby in a form reasonably satisfactory to the Principal Purchasers, and (b) file a Current Report on Form 8-K, including the Transaction Documents as exhibits thereto, with the Commission within the time required by the Exchange Act. The Company shall consult with the Principal Purchasers in issuing any other press releases with respect to the transactions contemplated hereby, and the Company shall not issue any such press release nor otherwise make any such public statement without the prior consent of the Principal Purchasers (which consent shall not unreasonably be withheld or delayed) or, with respect to the public disclosure of the identity of any Purchaser, the prior consent of such Purchaser, except if such disclosure is required by law, in which case the Company shall promptly provide the Purchasers with prior notice of such public statement or communication.

5.5 Shareholder Rights Plan. Solely to the extent that it would impair the ability of any Purchaser to receive Securities under the Transaction Documents, no claim will be made or enforced by the Company or, with the consent of the Company, any other Person, that any Purchaser is an "Acquiring Person" under any control share acquisition, business combination, poison pill (including any distribution under a rights agreement) or similar anti-takeover plan or arrangement in effect or hereafter adopted by the Company, or that any Purchaser could be deemed to trigger the provisions of any such plan or arrangement.

5.6 Use of Proceeds. The Company shall use the net proceeds from the sale of the Securities hereunder for funding operations or for working capital or other general corporate purposes.

5.7 Indemnification of Purchasers. Subject to the provisions of this Section 5.7, the Company will indemnify and hold each Purchaser and its directors, officers, shareholders, members, partners, employees and agents (and any other Persons with a functionally equivalent role of a Person holding such titles notwithstanding a lack of such title or any other title), each Person who controls such Purchaser (within the meaning of Section 15 of the Securities Act and Section 20 of the Exchange Act), and the directors, officers, shareholders, agents, members, partners or employees (and any other Persons with a

functionally equivalent role of a Person holding such titles notwithstanding a lack of such title or any other title) of such controlling persons (each, a "Purchaser Party") harmless from any and all losses, liabilities, obligations, claims, contingencies, damages, costs and expenses, including all judgments, amounts paid in settlements, court costs and reasonable attorneys' fees and costs of

investigation that any such Purchaser Party may suffer or incur as a result of or relating to (a) any breach of any of the representations, warranties, covenants or agreements made by the Company in this Agreement or in the other Transaction Documents or (b) any action instituted against the Purchaser Parties in any capacity, or any of them or their respective Affiliates, by any stockholder of the Company who is not an Affiliate of such Purchaser Parties, with respect to any of the transactions contemplated by the Transaction Documents (unless such action is based upon a breach of such Purchaser Party's representations, warranties or covenants under the Transaction Documents or any agreements or understandings such Purchaser Parties may have with any such stockholder or any violations by such Purchaser Parties of state or federal securities laws or any conduct by such Purchaser Parties which constitutes fraud, gross negligence, willful misconduct or malfeasance of such Purchaser Party). If any action shall be brought against any Purchaser Party in respect of which indemnity may be sought pursuant to this Agreement, such Purchaser Party shall promptly notify the Company in writing, and the Company shall have the right to assume the defense thereof with counsel of its own choosing reasonably acceptable to the Purchaser Party. Any Purchaser Party shall have the right to employ separate counsel in any such action and participate in the defense thereof, but the fees and expenses of such counsel shall be at the expense of such Purchaser Party except to the extent that (i) the employment thereof has been specifically authorized by the Company in writing, (ii) the Company has failed after a reasonable period of time to assume such defense and to employ counsel or (iii) in such action there is, in the reasonable opinion of counsel, a material conflict on any material issue between the position of the Company and the position of such Purchaser Party, in which case the Company shall be responsible for the reasonable fees and expenses of no more than one such separate counsel. The Company will not be liable to any Purchaser Party under this Agreement (y) for any settlement by a Purchaser Party effected without the Company's prior written consent, which shall not be unreasonably withheld or delayed; or (z) to the extent, but only to the extent that a loss, claim, damage or liability is attributable to any Purchaser Party's breach of any of the representations, warranties, covenants or agreements made by such Purchaser Party in this Agreement or in the other Transaction Documents. The indemnification required by this Section 5.7 shall be made by periodic payments of the amount thereof during the course of the investigation or defense, as and when bills are received or are incurred. The indemnity agreements contained herein shall be in addition to any cause of action or similar right of any Purchaser Party against the Company or others and any liabilities the Company may be subject to pursuant to law.

5.8 Reservation of Common Stock. The Company has reserved and the Company shall continue to reserve and keep available at all times, free of preemptive rights, a sufficient number of shares of Common Stock for the purpose of enabling the Company to issue Shares pursuant to this Agreement and Warrant Shares pursuant to any exercise of the Warrants.

5.9 Listing of Common Stock. The Company hereby agrees to use reasonable best efforts to maintain the listing or quotation of the Common Stock on the Trading Market on which it is currently listed, and, as promptly as practicable following the Closing, to secure the listing of all of the Shares and Warrant Shares on such Trading Market. The Company further agrees, if the Company applies to have the Common Stock traded on any other Trading Market, it will then include in such application all of the Shares and Warrant Shares, and will take such other action as is necessary to cause all of the Shares and Warrant Shares to be listed or quoted on such other Trading Market as promptly as possible. The Company will then take all action reasonably necessary to continue the listing or quotation and trading of its Common Stock on a Trading Market and will comply in all respects with the Company's reporting, filing and other obligations under the bylaws or rules of the Trading Market. The Purchasers and the Company agree to cooperate in good faith, if necessary, to restructure the transactions contemplated by the Transaction Documents such that they do not contravene the rules and regulations of Nasdaq; provided, however, that such restructuring does not impact the economic interests of the Purchasers contemplated by the Transaction Documents. Each Purchaser agrees to provide information reasonably requested by the Company to comply with this Section 5.9 and Section 3.24.

5.10 Equal Treatment of Purchasers. No consideration (including any modification of any Transaction Document) shall be offered or paid to any Person to amend or consent to a waiver or modification of any provision of this Agreement unless the same consideration is also offered to all of the parties to this Agreement. For clarification purposes, this provision constitutes a separate right granted to each Purchaser by the Company and negotiated separately by each Purchaser, and is intended for the Company to treat the Purchasers as a class and shall

not in any way be construed as the Purchasers acting in concert or as a group with respect to the purchase, disposition or voting of Securities or otherwise.

5.11 Form D; Blue Sky Filings. The Company agrees to timely file a Form D with respect to the Securities as required under Regulation D and to provide a copy thereof, promptly upon request of any Purchaser. The Company shall take such action as the Company shall reasonably determine is necessary in order to obtain an exemption for, or to qualify the Securities for, sale to the Purchasers at the Closing under applicable securities or "Blue Sky" laws of the states of the United States, and shall provide evidence of such actions promptly upon request of any Purchaser. Each Purchaser shall provide any information reasonably requested by the Company to comply with Section 5.11.

5.12 Acknowledgment of Dilution. The Company acknowledges that the issuance of the Securities may result in dilution of the outstanding shares of Common Stock, which dilution may be substantial under certain market conditions. The Company further acknowledges that its obligations under the Transaction Documents, including, without limitation, its obligation to issue the Shares and Warrant Shares pursuant to the Transaction Documents, are unconditional and absolute and not subject to any right of set off, counterclaim, delay or reduction, regardless of the effect of any such dilution or any claim the Company may have against any Purchaser and regardless of the dilutive effect that such issuance may have on the ownership of the other stockholders of the Company.

5.13 Other Actions. Except as otherwise set forth in this Agreement, from the date of this Agreement until the earlier to occur of the Closing or the termination of this Agreement in accordance with the terms hereof, the Company and the Purchasers shall not, and shall not permit any of their respective Affiliates to, take, or agree or commit to take, any action that would reasonably be expected to, individually or in the aggregate, prevent, materially delay or materially impede the consummation of the transactions contemplated by this Agreement.

ARTICLE 6
TERMINATION

6.1 Termination. The obligations of the Company, on the one hand, and the Purchasers, on the other hand, to effect the Closing shall terminate as follows:

(a) Upon the mutual written consent of the Company and the Purchasers;

(b) By the Company if any of the conditions set forth in Section 2.4(a) shall have become incapable of fulfillment, and shall not have been waived by the Company;

(c) By a Purchaser (with respect to itself only) if any of the conditions set forth in Section 2.4(b) shall have become incapable of fulfillment, and shall not have been waived by such Purchaser; or

(d) By either the Company or any Purchaser (with respect to itself only) if the Closing has not occurred on or prior to April 15, 2014; provided, however, that the Principal Purchasers may, in their sole discretion, extend such date to August 15, 2014, in the event shareholder approval is required;

provided, however, that, except in the case of clause (a) above, the party seeking to terminate its obligation to effect the Closing shall not then be in breach of any of its representations, warranties, covenants or agreements contained in this Agreement or the other Transaction Documents if such breach has resulted in the circumstances giving rise to such party's seeking to terminate its obligation to effect the Closing.

6.2 Notice of Termination; Effect of Termination. In the event of termination by the Company or any Purchaser of its obligations to effect the Closing pursuant to this ARTICLE VI, written notice thereof shall forthwith be given to the other Purchasers by the Company and the other Purchasers shall have the right to terminate their obligations to effect the Closing upon written notice to the Company and the other Purchasers. Nothing in this

ARTICLE VI shall be deemed to release any party from any liability for any breach by such party of the terms and provisions of this Agreement or the other Transaction Documents or to impair the right of any party to compel specific performance by any other party of its obligations under this Agreement or the other Transaction Documents.

ARTICLE 7
MISCELLANEOUS

7.1 Fees and Expenses. The parties hereto shall pay their own costs and expenses in connection herewith, including all attorneys' fees.

7.2 Entire Agreement. The Transaction Documents, together with the exhibits and schedules thereto, contain the entire understanding of the parties with respect to the subject matter hereof and thereof and supersede all prior agreements and understandings, oral or written, with respect to such matters, which the parties acknowledge have been merged into such documents, exhibits and schedules.

7.3 Notices. Any and all notices or other communications or deliveries required or permitted to be provided hereunder shall be in writing and shall be deemed given and effective on the earliest of: (a) the date of transmission, if such notice or communication is delivered via facsimile at the facsimile number set forth on the signature pages attached hereto at or prior to 5:30 p.m. (New York City time) on a Trading Day, (b) the next Trading Day after the date of transmission, if such notice or communication is delivered via facsimile at the facsimile number set forth on the signature pages attached hereto on a day that is not a Trading Day or later than 5:30 p.m. (New York City time) on any Trading Day, (c) the second (2nd) Trading Day following the date of mailing, if sent by U.S. nationally recognized overnight courier service or (d) upon actual receipt by the party to whom such notice is required to be given. The address for such notices and communications shall be as follows:

(i) if to the Company, to GTx, Inc., 175 Toyota Plaza, 7th Floor, Memphis, Tennessee 38103, Attention: Chief Legal Officer and Secretary, (facsimile: 901-271-8670), with a copy to Cooley LLP, 3175 Hanover Street, Palo Alto, California 94304 (facsimile: 650-849-740), Attention: Chadwick L. Mills; and

(ii) if to the Purchasers, to their respective addresses as set forth on Exhibit A attached hereto, with a copy to Matthew S. Heiter, Baker, Donelson, Bearman, Caldwell & Berkowitz, PC, 165 Madison Avenue, Suite 2000, Memphis, TN 38103; Fax: 901.577.0737.

7.4 Amendments; Waivers. No provision of this Agreement may be waived, modified, supplemented or amended except in a written instrument signed, in the case of an amendment, by the Company and the Principal Purchasers or, in the case of a waiver, by the party against whom enforcement of any such waived provision is sought. No waiver of any default with respect to any provision, condition or requirement of this Agreement shall be deemed to be a continuing waiver in the future or a waiver of any subsequent default or a waiver of any other provision, condition or requirement hereof, nor shall any delay or omission of any party to exercise any right hereunder in any manner impair the exercise of any such right.

7.5 Headings. The headings herein are for convenience only, do not constitute a part of this Agreement and shall not be deemed to limit or affect any of the provisions hereof.

7.6 Successors and Assigns. This Agreement shall be binding upon and inure to the benefit of the parties and their successors and permitted assigns. The Company may not assign this Agreement or any rights or obligations hereunder without the prior written consent of each Purchaser. With the consent of the Company which will not be unreasonably withheld, any Purchaser may assign any or all of its rights under this Agreement to any Person to whom such Purchaser assigns or transfers any Securities, provided, that a Purchaser may assign any or all rights under this Agreement to an Affiliate of such Purchaser without the consent of the Company, provided, further that such transferee agrees in writing to be bound, with respect to the transferred Securities, by the provisions of the Transaction Documents that apply to the "Purchasers."

7.7 No Third-Party Beneficiaries. This Agreement is intended for the benefit of the parties hereto and their respective successors and permitted assigns and is not for the benefit of, nor may any provision hereof be enforced by, any other Person, except as otherwise set forth in Section 5.7.

7.8 Governing Law; Jurisdiction. This Agreement shall be governed by, and construed in accordance with, the internal laws of the State of Delaware without regard to the choice of law principles thereof. Each of the parties hereto irrevocably submits to the exclusive jurisdiction of the state and federal courts located in the State of Delaware for the purpose of any suit, action, proceeding or judgment relating to or arising out of this Agreement and the transactions contemplated hereby. Service of process in connection with any such suit, action or proceeding may be served on each party hereto anywhere in the world by the same methods as are specified for the giving of notices under this Agreement. Each of the parties hereto irrevocably consents to the jurisdiction of any such court in any such suit, action or proceeding and to the laying of venue in such court. Each party hereto irrevocably waives any objection to the laying of venue of any such suit, action or proceeding brought in such courts and irrevocably waives any claim that any such suit, action or proceeding brought in any such court has been brought in an inconvenient forum.

7.9 WAIVER OF JURY TRIAL. IN ANY ACTION, SUIT, OR PROCEEDING IN ANY JURISDICTION BROUGHT BY ANY PARTY AGAINST ANY OTHER PARTY, THE PARTIES EACH KNOWINGLY AND INTENTIONALLY, TO THE GREATEST EXTENT PERMITTED BY APPLICABLE LAW, HEREBY ABSOLUTELY, UNCONDITIONALLY, IRREVOCABLY AND EXPRESSLY WAIVES FOREVER TRIAL BY JURY.

7.10 Survival. The representations and warranties contained herein shall survive the Closing and the delivery of the Securities.

7.11 Execution. This Agreement may be executed in two or more counterparts, all of which when taken together shall be considered one and the same agreement and shall become effective when counterparts have been signed by each party and delivered to each other party, it being understood that the parties need not sign the same counterpart. In the event that any signature is delivered by facsimile transmission or by e-mail delivery of a ".pdf" format data file, such signature shall create a valid and binding obligation of the party executing (or on whose behalf such signature is executed) with the same force and effect as if such facsimile or ".pdf" signature page were an original thereof.

7.12 Severability. If any term, provision, covenant or restriction of this Agreement is held by a court of competent jurisdiction to be invalid, illegal, void or unenforceable, the remainder of the terms, provisions, covenants and restrictions set forth herein shall remain in full force and effect and shall in no way be affected, impaired or invalidated, and the parties hereto shall use their commercially reasonable efforts to find and employ an alternative means to achieve the same or substantially the same result as that contemplated by such term, provision, covenant or restriction. It is hereby stipulated and declared to be the intention of the parties that they would have executed the remaining terms, provisions, covenants and restrictions without including any of such that may be hereafter declared invalid, illegal, void or unenforceable.

7.13 Rescission and Withdrawal Right. Notwithstanding anything to the contrary contained in (and without limiting any similar provisions of) any of the other Transaction Documents, whenever any Purchaser exercises a right, election, demand or option under a Transaction Document and the Company does not timely perform its related obligations within the periods therein provided, then such Purchaser may rescind or withdraw, in its sole discretion from time to time upon written notice to the Company, any relevant notice, demand or election in whole or in part without prejudice to its future actions and rights.

7.14 Remedies. In addition to being entitled to exercise all rights provided herein or granted by law, including recovery of damages, each of the Purchasers and the Company will be entitled to specific performance under the Transaction Documents. The parties agree that monetary damages may not be adequate compensation for any loss incurred by reason of any breach of obligations contained in the Transaction Documents and hereby agree

to waive and not to assert in any action for specific performance of any such obligation the defense that a remedy at law would be adequate.

7.15 Independent Nature of Purchasers' Obligations and Rights. The obligations of each Purchaser under any Transaction Document are several and not joint with the obligations of any other Purchaser, and no Purchaser shall be responsible in any way for the performance or non-performance of the obligations of any other Purchaser under any Transaction Document. Nothing contained herein or in any other Transaction Document, and no action taken by any Purchaser pursuant hereof or thereto, shall be deemed to constitute the Purchasers as a partnership, an association, a joint venture or any other kind of entity, or create a presumption that the Purchasers are in any way acting in concert or as a group with respect to such obligations or the transactions contemplated by the Transaction Documents. Each Purchaser shall be entitled to independently protect and enforce its rights, including, without limitation, the rights arising out of this Agreement or out of the other Transaction Documents, and it shall not be necessary for any other Purchaser to be joined as an additional party in any proceeding for such purpose. Each Purchaser has been represented by its own separate legal counsel in its review and negotiation of the Transaction Documents. The Company has elected to provide all Purchasers with the same terms and Transaction Documents for the convenience of the Company and not because it was required or requested to do so by any of the Purchasers.

[*Remainder of Page Intentionally Left Blank; Signature Pages Follow*]

IN WITNESS WHEREOF, the parties hereto have caused this Securities Purchase Agreement to be duly executed by their respective authorized signatories as of the date first indicated above.

GTx, INC.

By: /s/ Marc S. Hanover
Name: Marc S. Hanover
Title: President and Chief Operating Officer

IN WITNESS WHEREOF, the parties hereto have caused this Securities Purchase Agreement to be duly executed by their respective authorized signatories as of the date first indicated above.

J.R. HYDE, III

By: /s/ J.R. Hyde, III

IN WITNESS WHEREOF, the parties hereto have caused this Securities Purchase Agreement to be duly executed by their respective authorized signatories as of the date first indicated above.

THE PYRAMID PEAK FOUNDATION

By: /s/ Andrew R. McCarroll
Name: Andrew R. McCarroll
Title: Secretary

EXHIBIT A
SCHEDULE OF PURCHASERS

Purchaser	Units Purchased	Number of Shares of Common Stock Separable from Units Purchased	Number of Shares of Common Stock Exercisable from Warrants Separable from Units Purchased	Price Per Unit	Total Consideration
J.R. Hyde, III 17 West Pontotoc Ave., Suite 200 Memphis, TN 38103 (901) 685-3412	5,988,024	5,988,024	5,089,821	$ 1.77625001336	$ 10,636,227.71
The Pyramid Peak Foundation 6410 Poplar Ave. Ste 900 Memphis, Tennessee 38119	5,988,024	5,988,024	5,089,821	$ 1.77625001336	$ 10,636,227.71
TOTAL	11,976,048	11,976,048	10,179,642		$ 21,272,455.42

EXHIBIT B
FORM OF REGISTRATION RIGHTS AGREEMENT

EXHIBIT C
FORM OF WARRANT

Exhibit 23.1

Consent of Independent Registered Public Accounting Firm

We consent to the incorporation by reference in the following Registration Statements:

(1) Registration Statement (Form S-8 No. 333-188377) pertaining to the GTx, Inc. Directors' Deferred Compensation Plan, 2013 Non-Employee Director Equity Incentive Plan and 2013 Equity Incentive Plan,

(2) Registration Statements (Form S-8 Nos. 333-165507 and 333-149661) pertaining to the GTx, Inc. 2004 Equity Incentive Plan and the Amended and Restated 2004 Non-Employee Directors' Stock Option Plan,

(3) Registration Statement (Form S-8 No. 333-136527) pertaining to the GTx, Inc. Amended and Restated 2004 Non-Employee Directors' Stock Option Plan

(4) Registration Statement (Form S-8 No. 333-118882) pertaining to the GTx, Inc. Directors' Deferred Compensation Plan,

(5) Registration Statement (Form S-8 No. 333-112576) pertaining to the GTx, Inc. 2004 Equity Incentive Plan, 2004 Non-Employee Directors' Stock Option Plan, 2002 Stock Option Plan, 2001 Stock Option Plan, 2000 Stock Option Plan, and 1999 Stock Option Plan, and

(6) Registration Statement (Form S-3 No. 333-174396) of GTx, Inc. and in the related Prospectus

of our reports dated March 12, 2014, with respect to the financial statements of GTx, Inc. and the effectiveness of internal control over financial reporting of GTx, Inc. included in this Annual Report (Form 10-K) of GTx, Inc. for the year ended December 31, 2013.

/s/ Ernst & Young LLP

Memphis, Tennessee
March 12, 2014

EXHIBIT 31.1

CHIEF EXECUTIVE OFFICER CERTIFICATION

I, Mitchell S. Steiner, certify that:

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

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

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

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

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

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

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

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

5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of 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: March 12, 2014

/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, Marc S. Hanover, certify that:

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

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

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

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

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

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

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

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

5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of 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: March 12, 2014

/s/ Marc S. Hanover
Marc S. Hanover
President, Chief Operating Officer and
Acting Principal Financial Officer

EXHIBIT 32.1

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

In connection with the Annual Report of GTx, Inc. (the "Company") on Form 10-K for the year ended December 31, 2013, 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 Rule 13a-14(b) or Rule 15d-14(b) and 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.

Date: March 12, 2014

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

This certification accompanies the Form 10-K to which it relates, is not deemed filed with the Securities and Exchange Commission and is not to be incorporated by reference into any filing of the Company under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934 (whether made before or after the date of the Form 10-K), irrespective of any general incorporation language contained in such filing.

EXHIBIT 32.2

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

In connection with the Annual Report of GTx, Inc. (the "Company") on Form 10-K for the year ended December 31, 2013, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I, Marc S. Hanover, Principal Financial Officer of the Company certify, pursuant to Rule 13a-14(b) or Rule 15d-14(b) and 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.

Date: March 12, 2014

/s/ Marc S. Hanover
Marc S. Hanover
President, Chief Operating Officer and
Acting Principal Financial Officer

This certification accompanies the Form 10-K to which it relates, is not deemed filed with the Securities and Exchange Commission and is not to be incorporated by reference into any filing of the Company under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934 (whether made before or after the date of the Form 10-K), irrespective of any general incorporation language contained in such filing.