

**UNITED STATES
SECURITIES AND EXCHANGE COMMISSION**

Washington, D.C. 20549

FORM 10-Q

(Mark One)

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

For the quarterly period ended March 31, 2014

OR

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

For the transition period from _____ to _____

Commission file number: 000-50549

GTx, Inc.

(Exact name of registrant as specified in its charter)

Delaware

(State or other jurisdiction of incorporation or organization)

62-1715807

(I.R.S. Employer Identification No.)

175 Toyota Plaza

7th Floor

Memphis, Tennessee

(Address of principal executive offices)

38103

(Zip Code)

(901) 523-9700

(Registrant's telephone number, including area code)

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

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 No

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

Accelerated filer

Non-accelerated filer

Smaller reporting company

(Do not check if a smaller reporting company)

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

As of May 8, 2014, 75,161,437 shares of the registrant's Common Stock were outstanding.

PART I — FINANCIAL INFORMATION

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PART I: FINANCIAL INFORMATION

ITEM 1. FINANCIAL STATEMENTS

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

	<u>March 31,</u> <u>2014</u>	<u>December 31,</u> <u>2013</u>
	<u>(unaudited)</u>	
ASSETS		
Current assets:		
Cash and cash equivalents	\$ 22,432	\$ 14,529
Short-term investments	5,345	200
Prepaid expenses and other current assets	1,278	442
Total current assets	29,055	15,171
Property and equipment, net	86	112
Intangible and other assets, net	652	322
Total assets	<u>\$ 29,793</u>	<u>\$ 15,605</u>
LIABILITIES AND STOCKHOLDERS’ EQUITY		
Current liabilities:		
Accounts payable	\$ 619	\$ 808
Accrued expenses and other current liabilities	3,894	3,759
Total current liabilities	4,513	4,567
Other long-term liabilities	195	354
Commitments and contingencies		
Stockholders’ equity:		
Common stock, \$0.001 par value: 120,000,000 shares authorized at March 31, 2014 and December 31, 2013; 75,161,437 and 63,185,389 shares issued and outstanding at March 31, 2014 and December 31, 2013, respectively	75	63
Additional paid-in capital	489,357	465,981
Accumulated deficit	(464,347)	(455,360)
Total stockholders’ equity	25,085	10,684
Total liabilities and stockholders’ equity	<u>\$ 29,793</u>	<u>\$ 15,605</u>

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

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

	Three Months Ended March 31,	
	2014	2013
Expenses:		
Research and development expenses	\$ 6,360	\$ 9,614
General and administrative expenses	2,629	3,023
Total expenses	8,989	12,637
Loss from operations	(8,989)	(12,637)
Other income, net	2	55
Net loss	<u>\$ (8,987)</u>	<u>\$ (12,582)</u>
Net loss per share:		
Basic and diluted	<u>\$ (0.14)</u>	<u>\$ (0.20)</u>
Weighted average shares outstanding:		
Basic and diluted	<u>66,512,069</u>	<u>62,864,140</u>

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

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

	Three Months Ended March 31,	
	2014	2013
Cash flows from operating activities:		
Net loss	\$ (8,987)	\$ (12,582)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	33	150
Share-based compensation	2,215	814
Directors' deferred compensation	32	42
Changes in assets and liabilities:		
Prepaid expenses and other assets	(1,169)	(913)
Accounts payable	(189)	(607)
Accrued expenses and other liabilities	(22)	(827)
Net cash used in operating activities	<u>(8,087)</u>	<u>(13,923)</u>
Cash flows from investing activities:		
Purchase of property and equipment	(4)	(29)
Purchase of short-term investments, held to maturity	(5,145)	(1,225)
Proceeds from maturities of short-term investments, held to maturity	—	3,185
Net cash (used in) provided by investing activities	<u>(5,149)</u>	<u>1,931</u>
Cash flows from financing activities:		
Net proceeds from the issuance of common stock and warrants	21,141	—
Payments on capital lease and financed equipment obligations	(2)	(2)
Proceeds from exercise of employee stock options	—	86
Net cash provided by financing activities	<u>21,139</u>	<u>84</u>
Net increase (decrease) in cash and cash equivalents	7,903	(11,908)
Cash and cash equivalents, beginning of period	14,529	48,044
Cash and cash equivalents, end of period	<u>\$ 22,432</u>	<u>\$ 36,136</u>

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

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

1. Business and Basis of Presentation

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 POWER 1 (platinum plus taxane chemotherapy) and POWER 2 (platinum plus non-taxane chemotherapy) 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 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 (“EU”) 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, may be 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 has initiated seven Phase 1 clinical studies that are typically required for submission purposes and have submitted a pediatric investigational plan, or PIP, to the EMA, which is necessary for submission of a MAA. The Company has retained experts in both the United States and the EU to work with its internal team to explore the option of submitting a MAA for enobosarm 3 mg for the prevention and treatment of muscle wasting in patients with advanced NSCLC treated with platinum plus taxane chemotherapy, 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. The Company is evaluating options for further development of enobosarm 3 mg. Any further development would be subject to the Company’s 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.

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 non-metastatic castration resistant prostate cancer.

The Company has experienced significant recurring operating losses since its inception and has limited funds. The Company expects that its current cash resources, together with interest income thereon, will be sufficient to fund the completion of its ongoing Phase 2 clinical trials of enobosarm 9 mg and GTx-758, the completion of its

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

Phase 1 clinical studies of enobosarm 3 mg and the continuation of activities required for the potential submission of a 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. However, the Company will need to raise substantial additional capital in the near term to complete the activities that would be required to submit a MAA and to sustain its operations through and beyond the second quarter of 2015.

Basis of Presentation

The accompanying unaudited condensed financial statements reflect, in the opinion of management, all adjustments (consisting of normal recurring adjustments) necessary for a fair presentation of GTx’s financial position, results of operations and cash flows for each period presented in accordance with accounting principles generally accepted in the United States for interim financial information and with the instructions to Form 10-Q and Article 10 of Regulation S-X. Accordingly, information and footnote disclosures normally included in financial statements prepared in accordance with accounting principles generally accepted in the United States have been condensed or omitted from the accompanying condensed financial statements. These interim condensed financial statements should be read in conjunction with the audited financial statements and related notes thereto, which are included in the Company’s Annual Report on Form 10-K for the year ended December 31, 2013. Operating results for the three months ended March 31, 2014 are not necessarily indicative of the results that may be expected for the entire fiscal year ending December 31, 2014.

Use of Estimates

The preparation of condensed financial statements in conformity with accounting principles generally accepted in the United States requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities, the disclosure of contingent assets and liabilities at the date of the condensed financial statements, and the reported amounts of revenues and expenses during the reporting period. Actual amounts and results could differ from those estimates.

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 discovery activities and is focusing its research and development activities on the ongoing clinical development of the Company's current product candidates.

Cash, Cash Equivalents and Short-term Investments

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

At March 31, 2014 and December 31, 2013, short-term investments consisted of Federal Deposit Insurance Corporation insured certificates of deposit with original maturities of greater than three months and less than one year. As the Company has the positive intent and ability to hold the certificates of deposit 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

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determined using third-party pricing sources, which generally utilize observable inputs, such as interest rates and maturities of similar assets.

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 March 31, 2014 and December 31, 2013, net of the valuation allowance, the net deferred tax assets were reduced to zero. Income taxes are described more fully in Note 9 to the Company's financial statements included in the Company's Annual Report on Form 10-K for the year ended December 31, 2013.

Other Income, net

Other income, net consists of foreign currency transaction gains and losses associated with conducting clinical trials in foreign countries, interest earned on the Company's cash, cash equivalents and short-term investments, interest expense, and other non-operating income or expense.

FARESTON® Revenue Recognition

Although the Company sold its rights and certain assets related to FARESTON® effective September 30, 2012, the Company retained 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 March 31, 2014 and December 31, 2013, the Company's accrual for product returns, was \$454 and \$918, respectively. Of these amounts, \$189 and \$332 have been included in "Other long-term liabilities" in the condensed balance sheet at March 31, 2014 and December 31, 2013, respectively, and represents the portion of the Company's product returns accrual estimated to be payable after one year. The accrual for product returns decreased during the current period due to the closure of the return period for a portion of the previously sold inventory.

Subsequent Events

The Company has evaluated all events or transactions that occurred after March 31, 2014 up through the date the condensed financial statements were issued. Other than as set forth below, there were no material recognizable or nonrecognizable subsequent events during the period evaluated.

In April 2014, the Company announced that Mitchell S. Steiner, M.D., the Company's Vice Chairman and Chief Executive Officer and a co-founder of the Company, was leaving the Company to pursue other business interests. Dr. Steiner resigned from his roles as Chief Executive Officer and Vice Chairman of the Board of Directors at the Company, and as a member of the Board of Directors, effective April 3, 2014. In connection with Dr. Steiner's resignation, Marc S. Hanover was appointed as the Company's interim Chief Executive Officer and was elected to the Board to serve the remainder of Dr. Steiner's term on the Board until the annual meeting of GTx shareholders in 2015. Also in connection with Dr. Steiner's resignation, the Company entered into a severance agreement with Dr. Steiner, pursuant to which Dr. Steiner received severance benefits of (i) twelve months of base salary continuation payments, which totals \$452; (ii) a payout of accrued vacation of \$12; and (iii) continued healthcare coverage through the earliest to occur of (a) December 2014, (b) the date he becomes eligible for group health insurance coverage through a new employer or (c) the date Dr. Steiner ceases to be eligible for COBRA continuation coverage, which is estimated to cost the Company \$19. As a result of these severance benefits, the

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Company will recognize cash severance related expenses of \$483 during the second quarter of 2014. Additionally, all of Dr. Steiner's outstanding unvested stock options were vested and became immediately exercisable on April 13, 2014. The Company extended the post-termination exercise period of all of his stock options until the earlier to occur of (i) April 13, 2019 or (ii) the expiration of the term of a particular stock option grant. As a result of the modification of Dr. Steiner's options, the Company will recognize a one-time, noncash net compensation expense of \$215 during the second quarter of 2014, which reflects the aggregate incremental fair value associated with the modifications of \$359, partially offset by the reversal of \$144 of previously recognized share-based compensation expense for Dr. Steiner's unvested options.

In connection with Mr. Hanover's appointment as the Company's interim Chief Executive Officer, the Board approved the grant of a stock option under its 2013 Equity Incentive Plan ("2013 EIP") to purchase 500,000 shares of common stock. In accordance with the terms of the 2013 EIP, the option was granted at a price equal to the fair market value of the stock on the date of grant and has a term of ten years from the date of grant and vests in five equal annual installments. All of Mr. Hanover's previously granted options and a portion of the options granted on April 3, 2014 are subject to accelerated vesting if Mr. Hanover is involuntarily terminated or resigns due to a material demotion, as defined in the amended employment agreement between Mr. Hanover and the Company, within six months of a new Chief Executive Officer becoming employed by the Company. Additionally, if either of these events were to occur, the post-termination exercise period for all of his outstanding vested stock options would be extended until the earlier of (i) five years from his date of termination or resignation on account of a material demotion or (ii) the expiration of the term of a particular stock option grant in accordance with the terms of his amended employment agreement.

On May 7, 2014, the Company filed a Certificate of Amendment to the Company's Restated Certificate of Incorporation with the Secretary of State 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 120,000,000 shares to 200,000,000 shares. The foregoing amendment was approved by the Company's stockholders at the Company's 2014 Annual Meeting of Stockholders held on May 6, 2014.

2. Share-Based Compensation

Share-based payments include stock option grants and restricted stock units ("RSU's") under the Company's stock option and equity incentive plans and deferred compensation arrangements for the Company's 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. The Company's share-based compensation plans are described more fully in Note 3 to the Company's financial statements included in the Company's Annual Report on Form 10-K for the year ended December 31, 2013.

The following table summarizes share-based compensation expense included within the condensed statements of operations for the three months ended March 31, 2014 and 2013:

	Three Months Ended March 31,	
	2014	2013
Research and development expenses	\$ 1,380	\$ 342
General and administrative expenses	867	514
Total share-based compensation	<u>\$ 2,247</u>	<u>\$ 856</u>

Share-based compensation expense recorded as general and administrative expense for the three months ended March 31, 2014 and 2013 included share-based compensation expense related to deferred compensation arrangements for the Company's non-employee directors of \$32 and \$42, respectively.

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The Company uses the Black-Scholes-Merton option pricing valuation model ("Black-Scholes Model") to value stock options. The expected life of options is determined by calculating the average of the vesting term and the contractual term of the options. The expected price volatility is based on the Company's historical stock price volatility. 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 the Company has not made any dividend payments and has 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.

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.

The fair value of options granted was estimated using the following assumptions for the periods presented:

	Three Months Ended March 31,	
	2014	2013
Expected price volatility	87.9%	74.4%
Risk-free interest rate	2.3%	1.1%
Weighted average expected life in years	6.5 years	6.5 years

The following is a summary of stock option transactions for all of the Company's stock option and equity incentive plans since the Company's most recent fiscal year end:

Number of Shares	Weighted Average Exercise Price Per
------------------	--

		Share
Options outstanding at December 31, 2013	6,445,342	\$ 6.58
Options granted	2,000	1.74
Options forfeited or expired	(231,533)	11.85
Options exercised	—	—
Options outstanding at March 31, 2014	6,215,809	6.38

At March 31, 2014 and December 31, 2013, the Company had 1,225,000 unvested RSUs with a weighted average grant date fair value per share of \$1.87.

3. 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, unvested restricted stock units and common stock warrants.

Weighted average potential shares of common stock of 10,382,571 and 6,680,423 for the three months ended March 31, 2014 and 2013, respectively, were excluded from the calculations of diluted loss per share as inclusion of the potential shares would have had an anti-dilutive effect on the net loss per share for the periods.

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

4. Common Stock

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 its common stock per unit for gross proceeds of \$21,272. 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.

The warrants, which have a one year term expiring on March 6, 2015, have a per share exercise price of \$1.67 that is payable only in cash. The Company assessed whether the warrants require accounting as derivatives. The Company determined that the warrants were indexed to the Company's own stock. As such, the Company has concluded the warrants meet the scope exception for determining whether the instruments require accounting as derivatives and are classified in stockholders' equity. The fair value of the warrants was estimated at \$4,478 using the Black-Scholes Model with the following assumptions: expected volatility of 67%, risk free interest rate of 0.12%, expected life of one year and no dividends. The proceeds of the sale of the private placement were allocated to the common stock and warrants based upon their relative fair values.

5. 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 was 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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ITEM 2. MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

The following discussion should be read in conjunction with the condensed financial statements and the notes thereto included in Part 1, Item 1 of this Quarterly Report on Form 10-Q.

Forward-Looking Information

This Quarterly Report on Form 10-Q contains forward-looking statements. The forward-looking statements are contained principally in the sections entitled "Management's Discussion and Analysis of Financial Condition and Results of Operations" and "Risk Factors." 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 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 Quarterly Report on Form 10-Q in greater detail in the section entitled “Risk Factors” under Part II, 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 Quarterly Report on Form 10-Q. You should read this Quarterly Report on Form 10-Q and the documents that we incorporate by reference in and have filed as exhibits to this Quarterly Report on Form 10-Q, 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

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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.

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.

In April 2014, we announced that Mitchell S. Steiner, our Vice Chairman and Chief Executive Officer, or CEO, and a co-founder of the Company, was leaving the Company to pursue other business interests. Dr. Steiner resigned from his roles as CEO and Vice Chairman of the Board of Directors at the Company effective April 3, 2014. Marc S. Hanover, a co-founder of the Company, was named interim CEO by the Company’s Board. Mr. Hanover was also elected by the Board to fill Dr. Steiner’s remaining term as a Class II director until the annual meeting of shareholders in 2015. Mr. Hanover has served as President and Chief Operating Officer of GTx since our inception in September 1997. Also in April 2014, we announced that James T. Dalton, our Chief Scientific Officer, notified us of his decision to resign from GTx effective August 31, 2014. Our Board of Directors has approved the Company entering into a consulting agreement with Dr. Dalton following his resignation from the Company.

We remain committed to the implementation of our strategy, which includes pursuing a marketing authorization application, or MAA, in the European Union, or EU, for enobosarm 3 mg for the prevention and treatment of muscle wasting in patients with advanced non-small cell lung cancer, or NSCLC, treated with platinum plus taxane chemotherapy. We are evaluating options for further development of enobosarm 3 mg in the United States. We are also completing our Phase 2 clinical trials of enobosarm 9 mg to treat androgen receptor and estrogen receptor positive advanced breast cancer and GTx-758 as a secondary hormonal treatment for men with castration-resistant prostate cancer.

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 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 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 may potentially be 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 2014 to review and discuss the results of the POWER trials to determine an appropriate path forward for submitting a MAA in the EU 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, may be sufficient to support the submission of a MAA to the EMA seeking marketing authorization for enobosarm 3

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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. 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 a 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. We have initiated seven Phase 1 clinical studies that are typically required for submission purposes and have submitted a pediatric investigational plan, or PIP, to the EMA, which is necessary for submission of a MAA. We have retained experts in both the US and the EU to work with our internal team to explore the option of submitting a MAA after completion of the Phase 1 studies and acceptance of the PIP by the EMA's Pediatric Committee.

In our meeting with the United States Food and Drug Administration, or 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. We are evaluating options for further development of enobosarm 3 mg in the US. Any further development 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.

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. Moreover, they may also be metabolized to estrogens that could stimulate tumor growth. We believe that enobosarm, by targeting the androgen receptor, or AR, selectively in estrogen receptor, or ER, positive breast cancer, has the potential to provide clinical benefit to women whose metastatic breast cancer is progressing by treating their disease while minimizing the unwanted masculinizing side effects associated with steroidal androgens, and unlike steroidal 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 we have determined that the study will 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 early in the third 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 non-metastatic 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.

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We are currently conducting a Phase 2 open-label clinical trial in men who have developed metastatic or non-metastatic 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 trial is evaluating 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. Based on the safety and efficacy data observed in the 125 mg cohort and there being no unexpected side effects observed in the first ten metastatic patients enrolled in the 250 mg cohort, enrollment of the 250 mg cohort was opened to individuals with metastatic or non-metastatic CRPC. The study is ongoing and, subject to meeting our current enrollment projections, data from all patients in the study is expected in the first quarter of 2015.

Financial Highlights

Our net loss for the three months ended March 31, 2014 was \$9.0 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 March 31, 2014, we had cash, cash equivalents and short-term investments of \$27.8 million compared to \$14.7 million at December 31, 2013. 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 expiring on March 6, 2015, 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.

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 into the second quarter of 2015. We expect that our current cash resources, together with interest income thereon, will be sufficient to fund the completion of our ongoing Phase 2 clinical trials of enobosarm 9 mg and GTx-758, the completion of our Phase 1 clinical studies of enobosarm 3 mg and the continuation of activities required for the potential submission of a 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. However, we will need to raise substantial additional capital in the near term to complete the activities that would be required to submit a MAA and to sustain our operations through and beyond the second quarter of 2015. In addition, we have based these estimates on our current business plan and our

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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, we will need to raise substantial additional funding in order to complete the activities that would be required to submit a MAA to the EMA or 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 are conducting to support a potential MAA submission to the EMA for enobosarm 3 mg, including any additional Phase 3 clinical trials we may have to conduct to seek approval from any regulatory authorities for enobosarm 3 mg. If we are unable to raise additional funds in the near term to fund our operations through and beyond the second quarter of 2015, we would be required to, among other things, make further reductions in our workforce, eliminate our ongoing clinical trials, discontinue the development of enobosarm and/or GTx-758, liquidate all or a portion of our assets, and/or seek protection under the provisions of the U.S. Bankruptcy Code, all of which would have a material adverse effect on our business and stock price. In addition, the accompanying financial statements do not include any adjustments or charges that might be necessary should we be unable to continue as a going concern, such as charges related to impairment of our assets, the recoverability and classification of assets or the amounts and classification of liabilities or other similar adjustments.

While we have been able to fund our operations to date, we do not currently have any commitments for future external funding, other than the above mentioned warrants which may or may not be exercised by the warrant holders. 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. 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 by the uncertainty regarding our ability to obtain approval of enobosarm 3 mg in the EU in the absence of additional Phase 3 development of enobosarm 3 mg and the terms of any such approval. Our ability to raise additional funds and the terms upon which we are able to raise such funds may also be adversely affected by the uncertainties regarding our financial condition, the sufficiency of our capital resources and recent and potential future management turnover. As a result of these and other factors, we cannot be certain that additional funding will be available on acceptable terms, or at all.

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 and will be primarily focused on the continued clinical development of enobosarm and GTx-758.

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

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 evaluating options for further development of this program in the US.
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 from all subjects is expected early in the third quarter of 2014.
GTx-758 Secondary hormonal therapy in men with metastatic or non-metastatic 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 currently enrolling the 250 mg cohort in both metastatic and non-metastatic CRPC.

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.

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 condensed financial statements, which have been prepared in accordance with accounting principles generally accepted in the United States of America for interim financial statements. 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 condensed 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 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.

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While our significant accounting policies are more fully described in Note 2 to our financial statements appearing in our Annual Report on Form 10-K for the year ended December 31, 2013 filed with the SEC, 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. 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.

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 recognize 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 share-based payment awards 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.

The following table summarizes share-based compensation expense included within the condensed statements of operations for the three months ended March 31, 2014 and 2013:

	Three Months Ended March 31,	
	2014	2013
	(in thousands)	
Research and development expenses	\$ 1,380	\$ 342
General and administrative expenses	867	514
Total share-based compensation	\$ 2,247	\$ 856

Share-based compensation expense recorded in the condensed statement of operations as general and administrative expense for the three months ended March 31, 2014 and 2013 included share-based compensation

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expense related to deferred compensation arrangements for our non-employee directors of \$32,000 and \$42,000, respectively. At March 31, 2014, the total compensation cost related to non-vested stock options not yet recognized was approximately \$3.1 million with a weighted average expense recognition period of 1.58 years. At March 31, 2014, the total compensation cost related to non-vested RSUs not yet recognized was approximately \$577,000 with a weighted average expense recognition period of two months.

FARESTON® Revenue Recognition

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 March 31, 2014 and December 31, 2013, our accrual for product returns, was \$454,000 and \$918,000, respectively. The accrual for product returns decreased during the current period due to the closure of the return period for a portion of the previously sold inventory.

Results of Operations

Three Months Ended March 31, 2014 and 2013

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	Three Months Ended March 31,	
		2014	2013
		(in thousands)	
Enobosarm 3 mg			
Prevention and treatment of muscle wasting in patients with advanced non-small cell lung cancer	SARM	\$ 3,987	\$ 5,978
Enobosarm 9 mg			
Treatment of women with AR positive and ER positive metastatic breast cancer	SARM	863	323
Capesaris®			
Secondary hormonal therapy in men with metastatic and non-metastatic CRPC	Selective ER alpha agonist	1,462	1,714
Other research and development		48	1,599
Total research and development expenses		\$ 6,360	\$ 9,614

Research and development expenses decreased to \$6.4 million for the three months ended March 31, 2014 from \$9.6 million for the three months ended March 31, 2013. Research and development expenses related to enobosarm

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decreased as the last patients completed the POWER 1 and POWER 2 Phase 3 clinical trials for enobosarm 3 mg in May 2013. This was partially offset by activities related to a potential MAA submission, including the initiation of seven Phase 1 clinical trials, during the first quarter of 2014. 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. Research and development expenses related to GTx-758 decreased during the three months ended March 31, 2014 related to the ongoing Phase 2 clinical trial to evaluate GTx-758 as secondary hormonal therapy in men with metastatic CRPC due to the timing of patient activities and related management expenses as this trial was initiated in the third quarter of 2012.

“Other research and development” expenses include the cost of personnel, supplies and facilities associated with preclinical and discovery research and development activities and has decreased from the prior year comparable period as 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.

General and Administrative Expenses

General and administrative expenses decreased 13% to \$2.6 million for the three months ended March 31, 2014 from \$3.0 million for the three months ended March 31, 2013, which was due primarily to the workforce reduction implemented in October 2013, decreased legal expenses as the prior period included the preparation of new equity incentive plans and more intellectual property activities, and other costs saving measures we implemented related to the workforce reduction. These decreases were partially offset by increases due to expenses related to cash bonuses, stock options modifications, and stock option and RSU grants made to the employees as part of our efforts to retain the essential employees continuing with us following the October 2013 workforce reduction.

Liquidity and Capital Resources

At March 31, 2014, we had cash, cash equivalents and short-term investments of \$27.8 million, compared to \$14.7 million at December 31, 2013. Net cash used in operating activities was \$8.1 million and \$13.9 million for the three months ended March 31, 2014 and 2013, respectively, and resulted primarily from funding our operations.

Net cash used in investing activities was \$5.1 million for the three months ended March 31, 2014 and resulted primarily from the purchase of short-term investments. Net cash provided by investing activities was \$1.9 million for the three months ended March 31, 2013 and resulted from the maturities of short-term investments of \$3.2 million partially offset by the purchase of short-term investments of \$1.2 million.

Net cash provided by financing activities was \$21.1 million for the three months ended March 31, 2014 and reflects proceeds from the issuance of common stock and warrants, partially offset by payments on capital lease obligations. Net cash provided by financing activities was \$84,000 for the three months ended March 31, 2013 and was provided primarily from proceeds from the exercise of employee stock options partially offset by payments on capital lease and financed equipment obligations.

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 into the second quarter of 2015. We expect that our current cash resources, together with interest income thereon, will be sufficient to fund the completion of our ongoing Phase 2 clinical trials of enobosarm 9 mg and GTx-758, the completion of our Phase 1 clinical studies of enobosarm 3 mg and the continuation of activities required for the potential submission of a 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. However, we will need to raise substantial additional capital in the near term to complete the activities that would be required to submit a MAA and to sustain our operations through and beyond the second quarter of 2015. In addition, we have based these estimates on our current business plan and our 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, we will need to raise substantial additional funding in order to complete the activities that would be required to submit a MAA to the EMA or 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 are conducting to support a potential MAA submission to

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the EMA for enobosarm 3 mg, including any additional Phase 3 clinical trials we may have to conduct to seek approval from any regulatory authorities for enobosarm 3 mg.

Our estimate of the period of time through which our financial resources will be adequate to support our projected operating requirements is a forward-looking statement and involves risks and uncertainties, and actual results could vary as a result of a number of factors, including the factors discussed under Part II, 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 and commercialization activities, we are unable to estimate with certainty the amounts of increased capital outlays and operating expenditures associated with our anticipated future clinical trials, other research and development activities, and potential commercialization activities. 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.

While we have been able to fund our operations to date, we do not currently have any commitments for future external funding, other than the above mentioned warrants which may or may not be exercised by the warrant holders. 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 in the near term to fund our operations through and beyond the second quarter of 2015, we would be required to, among other things, make further reductions in our workforce, eliminate our ongoing clinical trials, discontinue the development of enobosarm and/or GTx-758, liquidate all or a portion of our assets, and/or seek protection under the provisions of the U.S. Bankruptcy Code, all of which would have a material adverse effect on our business and stock price. In addition, the accompanying financial statements do not include any adjustments or charges that might be necessary should we be unable to continue as a going concern, such as charges related to impairment of our assets, the recoverability and classification of assets or the amounts and classification of liabilities or other similar adjustments.

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

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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 by the uncertainty regarding our ability to obtain approval of enobosarm 3 mg in the EU in the absence of additional Phase 3 development of enobosarm 3 mg and the terms of any such approval. Our ability to raise additional funds and the terms upon which we are able to raise such funds may also be adversely affected by the uncertainties regarding our financial condition, the sufficiency of our capital resources and recent and potential future management turnover. As a result of these and other factors, we cannot be certain that additional funding will be available on acceptable terms, or at all.

ITEM 3. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK

During the three months ended March 31, 2014, there were no material changes to our market risk disclosures as set forth in Part II, Item 7A of our Annual Report on Form 10-K for the year ended December 31, 2013.

ITEM 4. CONTROLS AND PROCEDURES

We maintain disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) of the Securities and 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.

There were no changes in our internal control over financial reporting during the first quarter of 2014 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting. Effective December 31, 2013, our then Vice President, Chief Financial Officer and Treasurer resigned from GTx. During the first quarter of 2014, we reassigned certain responsibilities related to our key controls as a result of this resignation. However, we do not believe that these changes materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

PART II: OTHER INFORMATION

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.

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We have marked with an asterisk (*) those risks described below that reflect substantive changes from the risks described under Part I, Item 1A “Risk Factors” included in our Annual Report on Form 10-K filed with the Securities and Exchange Commission on March 12, 2014.

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 March 31, 2014, we had an accumulated deficit of \$464.3 million. Our net loss for the three months ended March 31, 2014 was \$9.0 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 may submit a marketing authorization application, or MAA, to the European Medicines Agency, or EMA, seeking marketing approval of enobosarm 3 mg in the European Union, or EU, for the more narrow indication of the prevention and treatment of muscle wasting in patients with advanced 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 a 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 a 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 EU, 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.

We are evaluating options for further development of enobosarm 3 mg in the United States. 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 are unable to raise additional capital in the near term to fund our operations through and beyond the second quarter of 2015 and to continue as a going concern, if we and/or any potential collaborators are unable to

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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 in the near term and may be unable to raise capital when needed, which would force us to delay, reduce or eliminate our product development programs and could cause us to discontinue our operations.*

We will need to raise substantial additional capital in the near term 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 EU 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.

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 into the second quarter of 2015. We expect that our current cash resources, together with interest income thereon, will be sufficient to fund the completion of our ongoing Phase 2 clinical trials of enobosarm 9 mg and GTx-758, the completion of our Phase 1 clinical studies of enobosarm 3 mg and the continuation of activities required for the potential submission of a 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. However, we will need to raise substantial additional capital in the near term to complete the activities that would be required to submit a MAA and to sustain our operations through and beyond the second quarter of 2015 and to continue as a going concern. In addition, we have based these estimates on our current business plan and our 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, we will need to raise substantial additional funding in order to complete the activities that would be required to submit a MAA to the EMA or 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 are conducting to support a potential MAA submission to the EMA for enobosarm 3 mg, including any additional Phase 3 clinical trials 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:

- 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

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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 in the near term to fund our operations through and beyond the second quarter of 2015 and to continue as a going concern, we would be required to, among other things, make further reductions in our workforce, eliminate our ongoing clinical trials, discontinue the development of enobosarm and/or GTx-758, liquidate all or a portion of our assets, and/or seek protection under the provisions of the U.S. Bankruptcy Code, all of which would have a material adverse effect on our business and stock price. In addition, the accompanying financial statements do not include any adjustments or charges that might be necessary should we be unable to continue as a going concern, such as charges related to impairment of our assets, the recoverability and classification of assets or the amounts and classification of liabilities or other similar adjustments.

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 by the uncertainty regarding our ability to obtain approval of enobosarm 3 mg in the EU in the absence of additional Phase 3 development of enobosarm 3 mg and the terms of any such approval. Our ability to raise additional funds and the terms upon which we are able to raise such funds may also be adversely affected by the uncertainties regarding our financial condition, the sufficiency of our capital resources and recent and potential future management turnover. As a result of these and other factors, we cannot be certain that additional funding will be available on acceptable terms, or at all.

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 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 may submit a MAA to the EMA for the marketing approval of enobosarm 3 mg in the EU for the more narrow

indication of the prevention and treatment of muscle wasting in patients with advanced NSCLC treated with platinum plus taxane chemotherapy, 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 a 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. Also, we are evaluating options for further

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development of enobosarm 3 mg in the United States. 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. While we may 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 would approve a MAA, which could result in the requirement that we conduct additional clinical studies or our ceasing further development of enobosarm program. Also we are evaluating options for further development of enobosarm 3 mg in the United States. There can be no assurance that we and the FDA would agree on any further development of enobosarm 3 mg. 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:

- 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

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- 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 both our ability to obtain additional funding and 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:

- 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.

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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 1 and Phase 2 clinical trials that we are currently conducting, and any additional Phase 3 clinical trials we may have to conduct to seek approval from any regulatory authority for enobosarm 3 mg is subject to our ability to raise substantial additional funds. There can be no assurances that we will be successful in obtaining additional funding in any event. If we are not able to raise substantial additional capital in the near term, 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;
- 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.

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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 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

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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 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

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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

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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 may submit a MAA to the EMA for the marketing approval of enobosarm 3 mg in the EU for the more narrow indication of the prevention and treatment of muscle wasting in patients with advanced NSCLC treated with platinum plus taxane chemotherapy, 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 a 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. Also, we are evaluating options for further development of enobosarm 3 mg in the United States. There can be no assurance that we would reach agreement with the FDA on any further development for enobosarm 3 mg and we would in any event be required to obtain substantial additional funding in order to commence any such Phase 3 program. 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 approvals.

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 EU, or other countries. See the section entitled "Business — Government Regulation" under Part 1, Item 1 of our Annual Report on Form 10-K, filed with the SEC on March 12, 2014, for additional information regarding risks associated with marketing approval, as well as risks related to potential post-approval requirements.

Risks Related to Commercialization

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If we submit a MAA to the EMA for the marketing approval of enobosarm 3 mg in the EU 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 marketing approval is obtained, we anticipate that the commercial prospects for enobosarm 3 mg could be diminished as a result of this more limited product indication. Additionally, any 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

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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 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 EU, 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 EU 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.

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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;
- 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 non-metastatic 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

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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

Management transition creates uncertainties and could harm our business.*

We have recently had significant changes in executive leadership, and more could occur. Effective December 31, 2013, Mark Mosteller resigned as our Chief Financial Officer. In connection with Mr. Mosteller's resignation, Marc S. Hanover, who was then serving as our President and Chief Operating Officer, was appointed as our acting principal financial officer and Jason T. Shackelford, who was then serving as our Corporate Controller and Director of Accounting, was appointed as our principal accounting officer. On April 3, 2014, Mitchell S. Steiner resigned as our Vice Chairman and Chief Executive Officer. On April 3, 2014, Mr. Hanover was appointed as our interim Chief Executive Officer. Upon the appointment of Mr. Hanover as interim Chief Executive Officer, Mr. Hanover ceased to perform the duties of our principal financial officer, which duties were assigned to Mr. Shackelford. Additionally, James T. Dalton, who notified us that he is resigning as our Chief Scientific Officer, will terminate his employment with us effective August 31, 2014. However, we anticipate retaining Dr. Dalton as a consultant to GTx following his resignation as a company employee.

As a result of the recent changes in our management team, Messrs. Hanover and Shackelford have taken on substantially more responsibility for the management of our business and of our financial reporting which has resulted in greater workload demands and could divert their attention away from certain key areas of our business. For instance, Mr. Hanover has taken on the role of interim Chief Executive Officer in addition to his role as our President and Chief Operating Officer, positions that were previously occupied by two persons. In addition, because Messrs. Hanover and Shackelford are serving as interim Chief Executive Officer and acting principal financial and accounting officer, respectively, it is possible that they could be replaced in those positions when permanent replacements are identified by the Board, and any transition period could create additional diversions for us and our employees, including for Messrs. Hanover and Shackelford. Also, while we expect that we will retain Dr. Dalton as a consultant to GTx following his employment end date, we will no longer have regular access to Dr. Dalton's key scientific expertise, which could materially and adversely impact our product candidate development efforts. Disruption to our organization as a result of executive management transition may have a detrimental impact on our ability to implement our strategy and could have a material adverse effect on our business, financial condition and results of operations.

Changes to company strategy, which can often times occur with the appointment of new executives, can create uncertainty, may negatively impact our ability to execute quickly and effectively, and may ultimately be unsuccessful. In addition, executive leadership transition periods are often difficult as the new executives gain detailed knowledge of our operations, and friction can result from changes in strategy and management style. Management transition inherently causes some loss of institutional knowledge, which can negatively affect strategy and execution. Until we integrate new personnel, and unless they are able to succeed in their positions, we may be

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unable to successfully manage and grow our business, and our results of operations and financial condition could suffer as a result.

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, 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.

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. In addition, since our October 2013 workforce reduction, our former Chief Executive Officer, former Chief Financial Officer and current Chief Scientific Officer have either resigned or notified us of their intent to resign. Primarily as a result of our October 2013 workforce reduction, only 32 employees remained as employees of GTx as of March 31, 2014. 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. Further, to the extent we experience additional management transition, competition for top management is high and it may take many months to find a candidate that meets our requirements. If we are unable to attract and retain qualified management personnel, our business could suffer.

If we are able to raise sufficient additional funds necessary to continue as a going concern and to pursue the development of our product candidates, 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.*

If we are able to raise sufficient additional funds necessary to continue as a going concern and to pursue the development of our product candidates, 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. As of March 31, 2014, we had only 32 employees.

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 to 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

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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;
- uncertainties created by our recent and potential future management turnover;
- 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

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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 31, 2014, our executive officers, directors and holders of 5% or more of our outstanding common stock, including their affiliated or associated entities, held approximately 64.3% 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 May 5, 2014 was \$1.45 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

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to replace or remove our current management by making it more difficult for stockholders to replace members of our Board of Directors. Because our Board of Directors is responsible for appointing the members of our management team, these provisions could in turn affect any attempt by our stockholders to replace current members of our management team. These provisions include:

- a classified Board of Directors;
- a prohibition on actions by our stockholders by written consent;
- the ability of our Board of Directors to issue preferred stock without stockholder approval, which could be used to institute a “poison pill” that would work to dilute the stock ownership of a potential hostile acquirer, effectively preventing acquisitions that have not been approved by our Board of Directors; and
- limitations on the removal of directors.

Moreover, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, which prohibits a person who owns in excess of 15% of our outstanding voting stock from merging or combining with us for a period of three years after the date of the transaction in which the person acquired in excess of 15% of our outstanding voting stock, unless the merger or combination is approved in a prescribed manner. Finally, these provisions establish advance notice requirements for nominations for election to our Board of Directors or for proposing matters that can be acted upon at stockholder meetings. These provisions would apply even if the offer may be considered beneficial by some stockholders.

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 March 31, 2014, the average daily trading volume of our common stock on The NASDAQ Global Market was 849,464 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 March 31, 2014, we had 75,161,437 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.

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ITEM 6. EXHIBITS

The exhibits listed on the accompanying Exhibit Index are filed or incorporated by reference (as stated therein) as part of this Quarterly Report on Form 10-Q.

SIGNATURES

Pursuant to the requirements 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.

Date: May 12, 2014

By: /s/ Marc S. Hanover
Marc S. Hanover, President, Chief Operating Officer
and interim Chief Executive Officer
(Principal Executive Officer)

Jason T. Shackelford, Corporate Controller,
Senior Director of Accounting and
Acting Principal Financial and Accounting Officer
(Principal Financial and Accounting Officer)

[Table of Contents](#)**EXHIBIT INDEX**

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	Certificate of Amendment of Restated Certificate of Incorporation of GTx, Inc.	8-K	000-50549	3.3	05/09/2014
3.4	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 between Registrant and 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	10-K	000-50549	4.5	03/12/2014
4.6	Registration Rights Agreement among Registrant, J.R. Hyde, III and The Pyramid Peak Foundation, dated March 6, 2014	10-K	000-50549	4.6	03/12/2014
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-K	000-50549	4.7	03/12/2014
10.1+	2014 Compensation Information for Registrant's Executive Officers	—	—	—	—
10.2+	Severance Agreement, made effective as of April 3, 2014, between Mitchell S. Steiner, M.D. and the Registrant	—	—	—	—
10.3+	Amendment to Amended and Restated Employment Agreement, effective as of April 3, 2014, between Registrant and Marc S. Hanover	—	—	—	—
31.1+	Certification of Principal Executive Officer, as required by Rule 13a-14(a) or Rule 15d-14(a)	—	—	—	—
31.2+	Certification of Principal Financial Officer, as required by Rule 13a-14(a) or Rule 15d-14(a)	—	—	—	—
32.1+	Certification of Principal 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	—	—	—	—

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+ Filed herewith

(1) This certification accompanies the Form 10-Q 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-Q), irrespective of any general incorporation language contained in such filing.

2014 Compensation Information for Registrant's Executive Officers

The following provides information regarding the base salary and certain other compensatory arrangements applicable to each "named executive officer" (as defined under applicable securities laws) of GTx, Inc. (the "Company") who service continued as of January 1, 2014, and to its other executive officer:

Executive Officer	Title	2014 Annual Base Salary \$(1)
Mitchell S. Steiner(2)	Chief Executive Officer and Vice-Chairman of the Board (Former)	452,088
Marc S. Hanover(3)	Interim Chief Executive Officer, President and Chief Operating Officer	393,317
James T. Dalton(4)	Vice President, Chief Scientific Officer	375,000
Henry P. Doggrell	Vice President, Chief Legal Officer and Secretary	363,576
Jason T. Shackelford(5)	Corporate Controller and Director of Accounting (Principal Financial and Accounting Officer)	178,010

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- (1) Effective January 1, 2014.
- (2) Dr. Steiner resigned from his positions as the Company's Chief Executive Officer and Vice-Chairman of the Board, effective April 3, 2014. A description of the severance agreement the Company entered into with Dr. Steiner in connection with his resignation is included under Item 5.02 of the Company's Current Report on Form 8-K, filed with the Securities and Exchange Commission on April 4, 2014, and is incorporated by reference herein.
- (3) Mr. Hanover was appointed as our interim Chief Executive Officer, effective April 3, 2014. A description of the compensatory arrangements provided to Mr. Hanover in connection with his appointment as the Company's interim Chief Executive Officer is included under Item 5.02 of the Company's Current Report on Form 8-K, filed with the Securities and Exchange Commission on April 4, 2014, and is incorporated by reference herein. There was no modification to Mr. Hanover's 2014 annual base salary in connection with his appointment as interim Chief Executive Officer.
- (4) Dr. Dalton notified the Company of his decision to resign from his employment with GTx, effective August 31, 2014.
- (5) Mr. Shackelford was appointed as our acting Principal Financial Officer, effective April 3, 2014 and as our acting Principal Accounting Officer, effective December 31, 2013. Effective May 12, 2014, Mr. Shackelford's annual base salary was increased to \$210,000.

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.

April 1, 2014

Mitchell S. Steiner, M.D.
 GTx, Inc.
 175 Toyota Plaza, 7th Floor
 Memphis, TN 38103

Re: Severance Agreement

Dear Mitch:

This letter sets forth the substance of the severance agreement (the “**Agreement**”) between you and GTx, Inc. (the “**Company**”).

1. Transition/Separation. You hereby resign from your position as Chief Executive Officer, and the Company hereby accepts such resignation, effective as of April 3, 2014. You also hereby resign from your position as a director on the Company’s Board of Directors (the “**Board**”), effective as of April 3, 2014, and the Company hereby accepts such resignation. Your last day of work with the Company and your employment termination date will be April 13, 2014 (the “**Separation Date**”). Between now and the Separation Date (the “**Transition Period**”), you agree to assist in the transition of your job duties as requested. During the Transition Period, you will not be expected to perform full-time work (unless requested to do so in connection with the transition of your duties); however, you will continue to receive your regular compensation and benefits.

2. Accrued Salary and Vacation. The Company will pay you all accrued salary, and all accrued and unused vacation earned through the Separation Date, subject to standard payroll deductions and withholdings, no later than twenty-one (21) days after the Separation Date.

3. Severance. Although the Company is not otherwise obligated to provide you with any severance benefits, if you sign the Separation Date Release attached as **Exhibit A** on the Separation Date, and allow the release set forth therein to become effective, then the Company will provide you with the following severance benefits:

(a) Severance Payments. The Company will pay you twelve (12) months of your base salary in effect as of the Separation Date (which is equal to \$452,088), less applicable taxes and withholdings, payable over the twelve (12) month period running from April 14, 2014, paid on the same payroll schedule on which current employees are paid (the “**Severance Payments**”).

(b) Health Care Continuation Coverage. If you timely elect continued coverage under COBRA, the Company will pay the COBRA premiums to continue your coverage (including coverage for eligible dependents, if applicable) (the “**COBRA Premiums**”) through the period (the “**COBRA Premium Period**”) starting on the Separation Date and ending on the earliest to occur of: (i) December 31, 2014; (ii) the date you become eligible for group health insurance coverage through a new employer; or (iii) the date you cease to be

eligible for COBRA continuation coverage for any reason, including plan termination. If you are obligated to make the COBRA payments directly to the insurance carrier, upon submission of proof of such payments to the Company, the Company will reimburse you such amounts. In the event you become covered under another employer’s group health plan or otherwise cease to be eligible for COBRA during the COBRA Premium Period, you must immediately notify the Company of such event. Notwithstanding the foregoing, if the Company determines, in its sole discretion, that it cannot pay the COBRA Premiums without a substantial risk of violating applicable law (including, without limitation, Section 2716 of the Public Health Service Act), the Company instead shall pay to you, on the first day of each calendar month, a fully taxable cash payment equal to the applicable COBRA premiums for that month (including premiums for you and your eligible dependents who have elected and remain enrolled in such COBRA coverage), subject to applicable tax withholdings, for the remainder of the COBRA Premium Period, which you may, but are not obligated to, use toward the cost of COBRA premiums.

(c) Life Insurance. The Company will assign to you the life insurance policies that it obtained for your benefit.

(d) Stock Options. You were granted options (the “**Options**”) in five (5) separate grants to purchase an aggregate of 525,000 shares of the Company’s common stock, as more particularly set forth in the Personal Grant Status sheet attached herewith as Attachment 1. As of the Separation Date, vesting of all Options will be accelerated such that all unvested Options will become immediately vested. You will be able to exercise these vested Options at any time following the Separation Date until the earliest of: (i) five (5) years after the Separation Date; or (ii) the expiration of the term of each of the Options. Except as expressly set forth in this paragraph, all of your rights and obligations with respect to the Options will be as set forth in each of the stock option agreements, grant notices, and applicable plan documents executed by you and the Company at the time of each such grant of Options.

4. Other Compensation or Benefits. You acknowledge that, except as expressly provided in this Agreement, you will not receive any additional compensation, severance or benefits after the Separation Date, including, but not limited to, any benefits under your Employment Agreement, dated February 14, 2013, and your Retention Benefit Agreement, dated October 1, 2013 (collectively, the “**Employment Agreements**”).

5. Expense Reimbursements. You agree that, within ten (10) days after the Separation Date, you will submit your final documented expense reimbursement statement reflecting all business expenses you incurred through the Separation Date, if any, for which you seek reimbursement. The Company will reimburse you for these expenses pursuant to its regular business practice.

6. Return of Company Property. By the Separation Date, you agree to return to the Company all Company documents (and all copies thereof) and other Company property in your possession, including, but not limited to, Company files, notes, drawings, records, business plans and forecasts, financial information, specifications, computer-recorded information, tangible property (except such property as you and the Company agree should be retained by you), credit cards, entry cards, identification badges and keys; and, any materials of any kind that

contain or embody any proprietary or confidential information of the Company (and all reproductions thereof).

7. Proprietary Information Obligations. Both during and after your employment you acknowledge your continuing obligations not to use or disclose, without the Company's express authorization, any confidential or proprietary information of the Company, including such obligations set forth in the Employment Agreements.

8. Nondisparagement. You agree not to disparage the Company or the Company's officers, directors, employees, shareholders, parents, subsidiaries, affiliates, and agents, in any manner likely to be harmful to them or their business, business reputation or personal reputation; *provided that* you may respond accurately and fully to any question, inquiry or request for information when required by legal process.

9. No Voluntary Adverse Action; Cooperation. You agree that you will not voluntarily (except in response to legal compulsion) assist any person in bringing or pursuing any proposed or pending litigation, arbitration, administrative claim or other formal proceeding against the Company, its parent or subsidiary entities, affiliates, officers, directors, employees or agents. In addition, you agree to cooperate fully with the Company in connection with its actual or contemplated defense, prosecution, or investigation of any claims or demands by or against third parties, or other matters arising from events, acts, or failures to act that occurred during the period of your employment by the Company. Additionally, as an named inventor on many patents and patent applications owned or license by the Company, you agree to make yourself reasonably available to the Company and its officers, directors, employees or agents to assist the Company in its prosecution of its patent applications or in any action initiated by it to exercise its rights under such intellectual property or in any actions by others which requires the Company to defend such rights. Such cooperation includes, without limitation, making yourself available to the Company upon reasonable notice, without subpoena, to provide complete, truthful and accurate information in witness interviews, depositions, and trial testimony. The Company will reimburse you for reasonable out-of-pocket expenses you incur in connection with any such cooperation (excluding foregone wages) and will make reasonable efforts to accommodate your scheduling needs.

10. General. This Agreement constitutes the complete, final and exclusive embodiment of the entire agreement between you and the Company with regard to this subject matter. It is entered into without reliance on any promise or representation, written or oral, other than those expressly contained herein, and it supersedes any other such promises, warranties or representations. This Agreement may not be modified or amended except in a writing signed by both you and a duly authorized officer of the Company. This Agreement will bind the heirs, personal representatives, successors and assigns of both you and the Company, and inure to the benefit of both you and the Company, their heirs, successors and assigns. If any provision of this Agreement is determined to be invalid or unenforceable, in whole or in part, this determination will not affect any other provision of this Agreement and the provision in question will be modified by the court so as to be rendered enforceable to the fullest extent permitted by law, consistent with the intent of the parties. This Agreement will be deemed to have been entered into and will be construed and enforced in accordance with the laws of the State of Tennessee as applied to contracts made and to be performed entirely within Tennessee.

If this Agreement is acceptable to you, please sign below and return the original to me.

I wish you good luck in your future endeavors.

Sincerely,

GTX, INC.

By: /s/ J.R. Hyde, III
J.R. Hyde, III
Chairman of the Board of Directors

AGREED:

/s/ Mitchell S. Steiner
Mitchell S. Steiner, M.D.

April 3, 2014
Date

Exhibit A
Separation Date Release
(to be executed on the Separation Date)

In exchange for the severance benefits to be provided to me pursuant to my Severance Agreement with GTx, Inc. (the "Company") dated April 1, 2014, I hereby provide the following release.

I hereby generally and completely release the Company and its parents, subsidiaries, successors, predecessors, and affiliates, and each of their respective directors, officers, employees, stockholders, shareholders, agents, attorneys, insurers, and assigns, from any and all claims, liabilities and obligations, both known and unknown, that arise out of or are in any way related to events, acts, conduct, or omissions occurring at any time prior to and including the date I sign this release. This general release includes, but is not limited to: (a) all claims arising out of or in any way related to my employment

or the termination of that employment; (b) all claims related to my compensation or benefits, including salary, bonuses, commissions, vacation pay, expense reimbursements, severance pay, fringe benefits, stock, stock options, or any other ownership interests; (c) all claims for breach of contract, wrongful termination, and breach of the implied covenant of good faith and fair dealing; (d) all tort claims, including claims for fraud, defamation, emotional distress, and discharge in violation of public policy; and (e) all federal, state, provincial and local statutory claims, including claims for discrimination, harassment, retaliation, attorneys' fees, or other claims arising under the federal Civil Rights Act of 1964 (as amended), the federal Americans with Disabilities Act of 1990 (as amended), the federal Employee Retirement Income Security Act of 1974 (as amended), the Tennessee Handicapped Discrimination Act, and the Tennessee Human Rights Act.

Notwithstanding the foregoing, I understand that the following claims are not included in my release: (a) any rights or claims for indemnification I may have pursuant to any written indemnification agreement; the charter, bylaws, or operating agreements of the Company; or under applicable law; or (b) any rights which cannot be waived as a matter of law. In addition, I understand that nothing in this release prevents me from filing, cooperating with, or participating in any proceeding before the Equal Employment Opportunity Commission or the Department of Labor, except that I hereby waive my right to any monetary benefits in connection with any such claim, charge or proceeding.

I acknowledge that I am knowingly and voluntarily waiving and releasing any rights I may have under the federal Age Discrimination in Employment Act (as amended) ("ADEA"), and that the consideration for the waiver and release in the preceding paragraph hereof is in addition to anything of value to which I was already entitled. I further acknowledge that I have been advised by this writing, as required by the ADEA, that: (a) my waiver and release do not apply to any rights or claims that may arise after the date I sign this release; (b) I should consult with an attorney prior to signing this release (although I may choose voluntarily not do so); (c) I have had 21 days to consider this release; (d) I have seven days following the date I sign this release to revoke it by providing written notice to the Board of Directors; and (e) this release will not be effective until the date upon which the revocation period has expired, which will be the eighth day after I sign this release.

I hereby represent that I have been paid all compensation owed and for all hours worked; I have received all the leave and leave benefits and protections for which I am eligible pursuant to the Family and Medical Leave Act or otherwise; and I have not suffered any on-the-job injury for which I have not already filed a workers' compensation claim.

MITCHELL S. STEINER, MD

/s/ Mitchell S. Steiner

(Signature)

Date: April 13, 2014

Personnel Grant Status **GTx, Inc.**
 ID: 62-1715807
 175 Toyota Plaza, 7th Floor
 Memphis, TN 38103

AS OF 3/24/2014

Mitchell Steiner

STOCK OPTIONS

Number	Grant Date	Plan	Type	Granted	Price	Exercised	Vested	Cancelled	Unvested	Outstanding	Exercisable
00000859	1/1/2012	2004	NQ	105,000	\$ 3.3600	0	42,000	0	63,000	105,000	42,000
0000594	1/1/2009	2004	NQ	75,000	\$ 16.8400	0	75,000	0	0	75,000	75,000
0000630	1/1/2010	2004	NQ	105,000	\$ 4.2000	0	84,000	0	21,000	105,000	84,000
0000770	1/1/2011	2004	NQ	105,000	\$ 2.6500	0	63,000	0	42,000	105,000	63,000
0000937	1/1/2013	2004	NQ	135,000	\$ 4.2000	0	27,000	0	108,000	135,000	27,000
				525,000		0	291,000	0	234,000	525,000	291,000

Information Currently on File

Tax	Rate %	Option Broker	Registration	Alternate Address
Federal	25.000			
Medicare	1.450			
Social Security	6.200			
TN-State	0.000			

2014 AMENDMENT TO THE
AMENDED AND RESTATED EMPLOYMENT AGREEMENT
BETWEEN GTx, INC. AND MARC S. HANOVER

This amendment (the “**2014 Amendment**”) to the Amended and Restated Employment Agreement, dated as of February 14, 2013 (the “**Agreement**”), between GTx, Inc. (“**Employer**”) and Marc. S. Hanover (“**Employee**”), is made effective as of April 3, 2014 (the “**Effective Date**”). Other than as to the terms and conditions contained in this 2014 Amendment, this 2014 Amendment shall not otherwise modify the Agreement, which Agreement shall remain in full force and effect. Capitalized terms not otherwise defined in this 2014 Amendment shall have the same meaning as in the Agreement.

WHEREAS, the Employee has been providing services to the Employer as President and Chief Operating Officer under the terms of the Agreement; and

WHEREAS, in connection with Employee’s appointment as Interim Chief Executive Officer (“**CEO**”) of the Employer, the Employer and Employee wish to amend the Agreement as set forth herein.

NOW, THEREFORE, in consideration of the mutual covenants and agreements hereinafter set forth, the adequacy and sufficiency of which is hereby acknowledged, the Employer and Employee agree as follows:

1. **Duties.** The Employee will continue to have such duties as are assigned or delegated to the Employee by the Board of Directors or the CEO (if the Employee is not then serving as Interim CEO) and will initially serve as President and Chief Operating Officer, and as Interim CEO, for the Employer.
2. **Benefits in the Event of Certain Terminations of Employment.** In the event of Employee’s Termination Without Cause (other than a termination by the Employer due to the Employee’s death or disability), or the Employee’s voluntary termination of employment due to a “Material Demotion” (as defined below), in each case occurring at any time during the “Transition Period” (as defined below) (each, a “**Covered Termination**”), and provided that the Employee signs and allows to become effective a Release within the time period provided therein (but not later than the 60th day following the Termination Date, such latest permitted effective date is the “Release Deadline” for purposes of this 2014 Amendment and the Agreement), then the Employee will be eligible to receive the following benefits (the “**Severance Benefits**”), subject to Section 9.2 of the Agreement:
 - a. The Employee shall receive as severance one (1) year of his Salary, payable in accordance with the Employer’s then current payroll schedule over the one (1) year

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period following the Termination Date, less deductions required by law. Notwithstanding the foregoing payment schedule, no severance will be paid prior to the effective date of the Release. Subject to Section 9.2 of the Agreement, on the first regular payroll pay day following the effective date of the Release, the Employer will pay the Employee the severance that the Employee would otherwise have received on or prior to such date but for the delay in payment related to the effectiveness of the Release, with the balance of the severance being paid as originally scheduled.

- b. The vesting of all stock options then held by the Employee and granted to the Employee by the Employer prior to April 3, 2014 (the “**Prior Options**”) will be accelerated in full, such that all unvested Prior Options will become immediately vested and exercisable as of the Termination Date.
 - c. The stock option to purchase 500,000 shares of the Company’s common stock granted to Employee on April 3, 2014 (the “**April Option**” and together with the Prior Options, the “**Options**”) will be credited with that number of months of service required for purposes of vesting in such April Option such that on the Termination Date, the number of shares subject to the April Option that would have vested as if Employee had continued in service with the Employer for an additional 24 months will immediately vest and become exercisable.
 - d. Each of the Options will remain outstanding and exercisable until the earlier of (i) the original expiration of the term of the Options and (ii) the date which occurs five (5) years following the Termination Date.
3. **Definitions.** For purposes of this 2014 Amendment, the following terms have the meanings specified:
 - a. “**Material Demotion**” shall mean, without the Employee’s consent, a material reduction in the Employee’s authority, duties or responsibilities as in effect prior to the Effective Date with respect to (and only with respect to) the positions of President and Chief Operating Officer, or the assignment to the Employee of any duties and responsibilities which are materially inconsistent with and adverse to such authority, duties and responsibilities. For avoidance of doubt, and notwithstanding the last sentence of Section 2.3 of the Agreement (which provides that “Employee may be re-assigned or transferred to another management position, as designated by the Board or the CEO, which may or may not provide the same level of responsibility as the initial assignment”), if during the Transition Period the then-serving CEO or the Board transfers or reassigns Employee to a position other than President and Chief Operating Officer, Employee shall be eligible to resign and receive the Severance Benefits under this 2014 Amendment on account of a “Material Demotion.”

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- b. “**Transition Period**” shall mean the period commencing on the first date the next person appointed as Employer’s Chief Executive Officer shall assume the duties of Employer’s Chief Executive Officer (the “**New CEO Hire Date**”) and ending on the earlier to occur of (i) the 181st day following the New CEO Hire Date or (ii) a Change of Control.

4. Termination of this 2014 Amendment; Incorporation of Agreement.

- a. Unless otherwise amended or extended by written agreement of the parties, this 2014 Amendment shall automatically terminate without further action of the parties on the first to occur of (i) the lapse of 181 days after the New CEO Hire Date or (ii) a Change of Control. For avoidance of doubt, (x) if Employee's employment with Employer is terminated by the Employer for any reason, or if Employee voluntarily resigns his employment with Employer for any reason, in each case prior to the New Hire CEO Date, then Employee shall not be entitled to any Severance Benefits under this 2014 Amendment; (y) if a Change of Control occurs (1) prior to the New CEO Hire Date, then Employee shall not be entitled to any Severance Benefits under this 2014 Amendment or (2) within 180 days after the New CEO Hire Date, then Employee shall be entitled to the Severance Benefits under this 2014 Amendment if and only if a Covered Termination occurs prior to such Change of Control; and (z) any severance benefits to which the Employee would be entitled in the event of a Change of Control Termination shall be determined solely by reference to the Agreement.
- b. All terms of the Agreement that do not conflict with the terms of this 2014 Amendment shall be incorporated into this 2014 Amendment and shall remain in full force and effect.

IN WITNESS WHEREOF, the parties have executed and delivered this 2014 Amendment, all effective as of the Effective Date.

MARC S. HANOVER

/s/ Marc S. Hanover

GTx, Inc.

By: /s/ Henry P. Doggrell

Name: Henry P. Doggrell

Title: Vice President, Chief Legal Officer

PRINCIPAL EXECUTIVE OFFICER CERTIFICATION

I, Marc S. Hanover, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q 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: May 12, 2014

/s/ Marc S. Hanover

Marc S. Hanover

President, Chief Operating Officer and interim Chief Executive Officer

(Principal Executive Officer)

PRINCIPAL FINANCIAL OFFICER CERTIFICATION

I, Jason T. Shackelford, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q 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: May 12, 2014

/s/ Jason T. Shackelford

Jason T. Shackelford

Corporate Controller, Senior Director of Accounting
and Acting Principal Financial and Accounting Officer
(Principal Financial Officer)

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 Quarterly Report of GTx, Inc. (the "Company") on Form 10-Q for the three months ended March 31, 2014, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I, Marc S. Hanover, 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, to the best of my knowledge:

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: May 12, 2014

/s/ Marc S. Hanover

Marc S. Hanover

President, Chief Operating Officer and interim Chief Executive Officer
(Principal Executive Officer)

This certification accompanies the Form 10-Q 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-Q), irrespective of any general incorporation language contained in such filing.

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 Quarterly Report of GTx, Inc. (the "Company") on Form 10-Q for the three months ended March 31, 2014, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I, Jason T. Shackelford, 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, to the best of my knowledge:

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: May 12, 2014

/s/ Jason T. Shackelford

Jason T. Shackelford

Corporate Controller, Senior Director of Accounting
and Acting Principal Financial and Accounting Officer
(Principal Financial Officer)

This certification accompanies the Form 10-Q 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-Q), irrespective of any general incorporation language contained in such filing.
