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UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

FORM 10-Q

(Mark One)

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

For the quarterly period ended June 30, 2013

OR

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

For the transition period from to

Commission file number: 000-50549

GTx, Inc.
(Exact name of registrant as specified in its charter)

Delaware	**62-1715807**
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification No.)
175 Toyota Plaza **7th Floor** **Memphis, Tennessee**	**38103**
(Address of principal executive offices)	(Zip Code)

(901) 523-9700
(Registrant's telephone number, including area code)

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

Indicate by check mark whether the registrant has submitted electronically and posted on its corporate web site, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). Yes x No o

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

Large accelerated filer o	Accelerated filer x
Non-accelerated filer o	Smaller reporting company o
(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 o No x

As of July 17, 2013, 63,185,389 shares of the registrant's Common Stock were outstanding.

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PART I: FINANCIAL INFORMATION

ITEM 1. FINANCIAL STATEMENTS

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

	June 30, 2013 (unaudited)	December 31, 2012
ASSETS		
Current assets:		
Cash and cash equivalents	$ 28,252	$ 48,044
Short-term investments	3,390	8,045
Prepaid expenses and other current assets	1,002	726
Total current assets	32,644	56,815
Property and equipment, net	285	507
Intangible and other assets, net	562	452
Total assets	$ 33,491	$ 57,774
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	$ 1,796	$ 1,707
Accrued expenses and other current liabilities	6,503	7,788
Total current liabilities	8,299	9,495
Other long-term liabilities	436	578
Commitments and contingencies		
Stockholders' equity:		
Common stock, $0.001 par value: 120,000,000 shares authorized at both June 30, 2013 and December 31, 2012; 63,110,430 and 62,818,424 shares issued and outstanding at June 30, 2013 and December 31, 2012, respectively	63	63
Additional paid-in capital	463,326	460,887
Accumulated deficit	(438,633)	(413,249)
Total stockholders' equity	24,756	47,701
Total liabilities and stockholders' equity	$ 33,491	$ 57,774

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 June 30,		Six Months Ended June 30,	
	2013	2012	2013	2012
Expenses:				
Research and development expenses	$ 10,139	$ 9,237	$ 19,753	$ 19,072
General and administrative expenses	2,684	2,400	5,707	4,988
Total expenses	12,823	11,637	25,460	24,060
Loss from operations	(12,823)	(11,637)	(25,460)	(24,060)
Other income, net	21	53	76	61
Loss from operations before income taxes	(12,802)	(11,584)	(25,384)	(23,999)
Income tax benefit	—	355	—	736
Net loss from continuing operations	(12,802)	(11,229)	(25,384)	(23,263)
Income from discontinued operations before income taxes	—	1,203	—	2,538
Income tax expense	—	(355)	—	(736)
Net income from discontinued operations	—	848	—	1,802
Net loss	$ (12,802)	$ (10,381)	$ (25,384)	$ (21,461)
Net loss per share - basic and diluted:				
Net loss from continuing operations	$ (0.20)	$ (0.18)	$ (0.40)	$ (0.37)
Net income from discontinued operations	—	0.01	—	0.03
Net loss per share	$ (0.20)	$ (0.17)	$ (0.40)	$ (0.34)
Weighted average shares outstanding:				
Basic and diluted	62,994,771	62,805,662	62,929,816	62,801,835

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)

	Six Months Ended June 30,	
	2013	2012
Cash flows from operating activities:		
Net loss	$ (25,384)	$ (21,461)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	262	413
Share-based compensation	1,496	1,125
Directors' deferred compensation	74	88
Changes in assets and liabilities:		
Prepaid expenses and other assets	(394)	231
Accounts payable	89	243
Accrued expenses and other liabilities	(1,423)	917
Net cash used in operating activities	(25,280)	(18,444)
Cash flows from investing activities:		
Purchase of property and equipment	(32)	(86)
Purchase of short-term investments, held to maturity	(1,225)	(4,730)
Proceeds from maturities of short-term investments, held to maturity	5,880	6,815
Net cash provided by investing activities	4,623	1,999
Cash flows from financing activities:		
Payments on capital lease and financed equipment obligations	(4)	(44)
Proceeds from exercise of employee stock options	869	63
Net cash provided by financing activities	865	19
Net decrease in cash and cash equivalents	(19,792)	(16,426)
Cash and cash equivalents, beginning of period	48,044	63,745
Cash and cash equivalents, end of period	$ 28,252	$ 47,319

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

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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, cancer supportive care, 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 hormonal therapy for the treatment of advanced breast cancer. The Company is conducting two fully enrolled pivotal 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. The last patients completed these pivotal Phase 3 clinical trials in May 2013. The vital status (survival) of patients participating in the trials will continue to be periodically monitored in accordance with the clinical trial protocols. The Company plans to announce topline data from these pivotal Phase 3 clinical trials during the third quarter of 2013.

In the second quarter of 2013, the Company initiated 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 advanced 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 primary treatment for advanced prostate cancer used in combination with androgen deprivation therapy. The Company initiated in the third quarter of 2012 a Phase 2 clinical trial to evaluate GTx-758 as secondary hormonal therapy in men with metastatic castration resistant prostate cancer.

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, 2012. Operating results for the three and six months ended June 30, 2013 are not necessarily indicative of the results that may be expected for the entire fiscal year ending December 31, 2013.

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.

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

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.

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 June 30, 2013 and December 31, 2012, 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 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 June 30, 2013 and December 31, 2012, net of the valuation allowance, the net deferred tax assets were reduced to zero. Income taxes are described more fully in Note 10 to the Company's financial statements included in the Company's Annual Report on Form 10-K for the year ended December 31, 2012.

The Company has recognized the tax effect of discontinued operations of FARESTON® (see Note 4, *Discontinued Operations)* in the condensed statement of operations for the three and six months ended June 30, 2012 in accordance with the intra-period accounting rules. An offsetting tax benefit was recorded in continuing operations as tax expense was recognized for discontinued operations.

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.

Discontinued Operations

Effective September 30, 2012, the Company entered into an asset purchase agreement (the "FARESTON® Purchase Agreement") with Strakan International S.á r.l., an affiliate of ProStrakan Group plc ("ProStrakan") pursuant to which the Company agreed to transfer, sell and assign to ProStrakan all of the Company's rights and

certain assets related to FARESTON®. The Company has accounted for FARESTON® as a discontinued operation. As a result, revenue, cost of goods sold, and operating expenses related to FARESTON® were excluded from the respective captions in the condensed statement of operations and were included in discontinued operations for the three and six months ended June 30, 2012. See Note 4, *Discontinued Operations*, for further discussion.

FARESTON® Revenue Recognition

Revenue from product sales of FARESTON® for the three and six months ended June 30, 2012, which was included in income from discontinued operations before income taxes, was recognized less deductions for estimated sales discounts and sales returns. Revenue from product sales was recognized when persuasive evidence of an arrangement existed, title passed, the price was fixed or determinable, and collectability was reasonably assured. The Company accounted for rebates to certain governmental agencies as a reduction of product sales. The Company allows customers to return product within a specified time period prior to and subsequent to the product's labeled expiration date. Although the Company sold its rights and certain assets related to FARESTON® effective September 30, 2012, the Company 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 June 30, 2013 and December 31, 2012, the Company's accrual for product returns, was $1,045 and $1,189, respectively. Of these amounts, $332 and $370 have been included in "Other long-term liabilities" in the condensed balance sheet at June 30, 2013 and December 31, 2012, respectively, and represents the portion of the Company's product returns accrual estimated to be payable after one year. See Note 4, *Discontinued Operations*, for further discussion.

Reclassification

Certain prior period results have been reclassified to conform to the current period presentation.

Subsequent Events

The Company has evaluated all events or transactions that occurred after June 30, 2013 up through the date the condensed financial statements were issued. There were no material recognizable or nonrecognizable subsequent events during the period evaluated.

2. Share-Based Compensation

Share-based payments include stock option grants 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.

On May 2, 2013, the Company's stockholders approved the GTx, Inc. 2013 Equity Incentive Plan (the "2013 EIP") and the GTx, Inc. 2013 Non-Employee Director Equity Incentive Plan (the "2013 NEDEIP"), which became effective on that date. The 2013 EIP is the successor to the Company's 2004 Equity Incentive Plan (the "2004 EIP"), and the 2013 NEDEIP is the successor to the Company's Amended and Restated 2004 Non-Employee Directors' Stock Option Plan (the "2004 NEDSOP"). The total number of shares of the Company's common stock available for issuance under the 2013 EIP was initially 4,208,157 shares plus up to an additional 6,093,559 shares subject to outstanding awards granted under the 2004 EIP and each of the Genotherapeutics, Inc. Stock Option Plan, the GTx, Inc. 2000 Stock Option Plan, the GTx, Inc. 2001 Stock Option Plan and the GTx, Inc. 2002 Stock Option Plan (collectively, the "Prior Plans") that, from and after the effective date of the 2013 EIP, expire or terminate for any reason prior to exercise or settlement, are forfeited because of the failure to vest in those shares or are otherwise returned to the 2013 EIP share reserve pursuant to the terms of the plan. In addition, the shares of the Company's common stock available for issuance under the 2013 EIP will automatically increase on January 1st of each year, for ten years, commencing on January 1, 2014, in an amount equal to 4% of the total number of shares of the Company's common stock outstanding on

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GTx, Inc.
NOTES TO THE CONDENSED FINANCIAL STATEMENTS
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December 31 of the preceding calendar year, or such lesser (or no) amount as may be approved by the Company's Board of Directors. The total number of shares of the Company's common stock available for issuance under the 2013 NEDEIP was initially 404,000 shares plus up to an additional 449,667 shares subject to outstanding awards granted under the 2004 NEDSOP that, from and after the effective date of the 2013 NEDEIP, expire or terminate for any reason prior to exercise or settlement, are forfeited because of the failure to vest in those shares or are otherwise returned to the 2013 NEDEIP share reserve pursuant to the terms of the plan. In addition, the shares of the Company's common stock available for issuance under the 2013 NEDEIP will automatically increase on January 1st of each year, for ten years, commencing on January 1, 2014, in an amount equal to the lesser of 1% of the total number of shares of the Company's common stock outstanding on December 31 of the preceding calendar year and 500,000 shares, or such lesser (or no) amount as may be approved by the Company's Board of Directors. From and after the effective date of 2013 EIP and the 2013 NEDEIP, no further awards will be made under the Prior Plans and the 2004 NEDSOP. Stock options previously granted under the Prior Plans and the 2004 NEDSOP continue to be governed by the terms of the applicable plan. For more information on the terms of stock options granted to employees and directors, see 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, 2012.

The following table summarizes share-based compensation expense included within the condensed statements of operations for the three and six months ended June 30, 2013 and 2012:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2013	2012	2013	2012
Research and development expenses	$ 353	$ (158)	$ 695	$ 361
General and administrative expenses	361	444	875	852
Total share-based compensation	$ 714	$ 286	$ 1,570	$ 1,213

Share-based compensation expense recorded as general and administrative expense for the three months ended June 30, 2013 and 2012 included share-based compensation expense related to deferred compensation arrangements for the Company's non-employee directors of $32 and $43, respectively. Share-based compensation expense recorded as general and administrative expense for the six months ended June 30, 2013 and 2012 included share-based compensation expense related to deferred compensation arrangements for the Company's non-employee directors of $74 and $88, respectively. Share-based compensation expense recorded as research and development expense for the three and six months ended June 30, 2012 was offset by the reversal of previously recognized share-based compensation expense for non-vested stock options that were canceled in conjunction with the resignation of an executive officer during the three months ended June 30, 2012.

The Company uses the Black-Scholes-Merton option pricing valuation 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.

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GTx, Inc.
NOTES TO THE CONDENSED FINANCIAL STATEMENTS
(in thousands, except share and per share data)
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The fair value of options granted was estimated using the following assumptions for the periods presented:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2013	2012	2013	2012
Expected price volatility	75.7%	75.0%	74.5%	69.5%
Risk-free interest rate	1.0%	1.3%	1.1%	1.2%
Weighted average expected life in years	6.0 years	6.0 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, 2012	5,382,859	$ 7.96
Options granted	1,456,700	4.24
Options forfeited or expired	(258,818)	9.00
Options exercised	(246,339)	3.53
Options outstanding at June 30, 2013	6,334,402	7.24

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.

Weighted average options outstanding to purchase shares of common stock of 6,488,055 and 5,580,340 for the three months ended June 30, 2013 and 2012, respectively, and 6,583,708 and 5,756,748 for the six months ended June 30, 2013 and 2012, respectively, were excluded from the calculations of diluted loss per share as inclusion of the options would have had an anti-dilutive effect on the net loss per share for these periods.

4. Discontinued Operations

On September 28, 2012, the Company entered into the FARESTON® Purchase Agreement with ProStrakan pursuant to which the Company agreed to transfer, sell and assign to ProStrakan all of the Company's rights to FARESTON® and certain assets related thereto. Effective September 30, 2012, the Company completed the sale of FARESTON® pursuant to the FARESTON® Purchase Agreement for a total cash purchase price of $21,671, including payment for purchased inventory. The Company accounted for FARESTON® as a discontinued operation. The FARESTON® operating income of $1,203 and $2,538 for the three and six months ended June 30, 2012, respectively, was reported as income from discontinued operations in the condensed statement of operations. For the three months ended June 30, 2012, income from discontinued operations consisted of net product sales of $1,639 reduced by cost of product sales of $245 and FARESTON® operating expenses of $191. For the six months ended June 30, 2012, income from discontinued operations consisted of net product sales of $3,468 reduced by cost of product sales of $519 and FARESTON® operating expenses of $411. The Company remains liable for product returns related to sales of FARESTON® made by the Company prior to September 30, 2012. At June 30, 2013 and December 31, 2012, the Company's accrual for product returns, was $1,045 and $1,189, respectively.

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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 anticipated progress of our research, development and 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 clinical trials and any future clinical trials that we may conduct;
- the timing of regulatory submissions and the timing, scope and anticipated outcome of related regulatory actions;
- our ability to establish and maintain potential new collaborative arrangements for the development and commercialization of our product candidates;
- 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 generate additional product candidates for clinical testing;

- our ability to protect our intellectual property and operate our business without infringing upon the intellectual property rights of others; and

- our estimates regarding the sufficiency of our cash resources, 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 forward-looking statements publicly, or to update the reasons actual results could differ materially from those anticipated in any forward-looking statements, even if new information becomes available in the future.

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Overview

Business Overview

We are a biopharmaceutical company dedicated to the discovery, development and commercialization of small molecules for the treatment of cancer, cancer supportive care, and other serious medical conditions.

Business Highlights

We are developing selective androgen receptor modulators, or SARMs, a new class of drugs with the potential to prevent and treat muscle wasting in patients with cancer and other musculoskeletal wasting or muscle loss conditions, including chronic sarcopenia (age related muscle loss), as well as the potential to be used as a hormonal therapy for the treatment of advanced breast cancer. Our lead SARM product candidate, enobosarm (GTx-024), has to date been evaluated in eight completed clinical trials enrolling approximately 600 subjects, including in a Phase 1b and two Phase 2 efficacy studies. 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 are conducting the POWER 1 and POWER 2 (Prevention and treatment Of muscle Wasting in patients with cancER) pivotal 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. We are conducting these trials in clinical sites in the United States, Europe, Russia and South America. Each of the pivotal Phase 3 placebo-controlled, double-blind clinical trials was fully enrolled during the fourth quarter of 2012, with approximately 325 patients in each clinical trial. In each of these clinical trials, patients with Stage III or IV non-small cell lung cancer were randomized to placebo or enobosarm 3 mg at the time they began first line chemotherapy. The last patients completed these pivotal Phase 3 clinical trials in May 2013. The vital status (survival) of patients participating in the trials will continue to be periodically monitored in accordance with the clinical trial protocols. The trials are evaluating as coprimary endpoints the effect of enobosarm versus placebo on total lean body mass (muscle) assessed by dual x-ray absorptiometry, or DXA, and on physical function assessed by the Stair Climb Test at three months. Durability of effect is being assessed as a secondary endpoint at five months in those patients who responded at Day 84.

In January 2013, the United States Food and Drug Administration, or FDA, designated enobosarm for the prevention and treatment of muscle wasting in patients with advanced non-small cell lung cancer as a fast track development program. Fast track status is a process designed by the FDA to facilitate the development and expedite the review of a new drug candidate that is intended to treat a serious or life-threatening condition and has the potential to fill an unmet medical need for that condition. In April 2013, after a pre-specified safety review in subjects enrolled in the two Phase 3 clinical trials, the independent Data Safety Monitoring Board, or DSMB, determined that the trials could continue as planned. This was the last review to be conducted by the DSMB until after the data is locked and unblinded for a final assessment of safety data from the two clinical trials. We plan to announce topline data from these pivotal Phase 3 clinical trials during the third quarter of 2013. Subject to obtaining positive data from these pivotal Phase 3 clinical trials and receiving marketing authorization from the appropriate regulatory authorities, we will consider either building a specialized sales and marketing organization to commercialize enobosarm in the United States and/or entering into strategic partnerships or collaborations for the development and commercialization of this product candidate.

SARMs also have the potential to be used as a hormonal therapy for the treatment of advanced breast cancer. Nonselective steroidal androgens have been used to treat breast cancer; however, the unwanted virilizing side effects have limited their widespread clinical use. We believe that enobosarm, by targeting the androgen receptor in estrogen receptor positive breast cancer, has the potential to provide clinical benefit to women with advanced breast cancer by treating their disease while minimizing the unwanted masculinizing side effects associated with steroidal androgens. In the second quarter of 2013, we initiated 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 advanced breast cancer. This proof of concept study will enroll 20 women at approximately six clinical sites in the United States. The women will receive 9 mg of enobosarm once a day until they show evidence of clinical progression or have completed 336 days of treatment. The primary endpoint is clinical benefit, which will be assessed at six months, and is defined as either

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those women receiving treatment who have demonstrated a complete response (disappearance of all targeted lesions), a partial response (at least a 30% decrease in the sum of the diameters of the targeted lesions) or stable disease (no disease progression from baseline).

Additionally, we are developing GTx-758 (Capesaris®), an oral nonsteroidal selective estrogen receptor alpha agonist, for secondary hormonal therapy in men with metastatic castration resistant prostate cancer, or CRPC, and, potentially, as a primary 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, hot flashes and insulin resistance, 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 primary treatment of advanced prostate cancer by reducing free testosterone to levels lower than those attainable with ADT alone and potentially reducing the estrogen deficiency side effects caused by the use of ADT.

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

GTx-758 has previously demonstrated the ability to increase the production of a protein called sex hormone binding globulin, or SHBG, in men with advanced prostate cancer and reduce 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 open-label clinical trial is the proportion of subjects with a $\geq$ 50% decline from baseline in serum PSA by day 90. Other key endpoints include serum SHBG, total and free testosterone levels and progression free survival in the study subjects. In addition, the clinical study will evaluate the ability of GTx-758 to treat certain estrogen deficiency side effects associated with luteinizing hormone releasing hormone agonists such as hot flashes, bone loss, and insulin resistance. The clinical trial requires subjects to continue receiving their ADT treatment, which will allow us to assess the safety and tolerability of GTx-758 in these subjects, including the incidence of VTEs. The trial design provides for 75 total subjects, with three sequential dosing arms. The first 25 subjects in the Phase 2 clinical trial are being enrolled in the GTx-758 125 mg dosing arm. Assuming that an acceptable incidence of VTEs has been observed when the last subject enrolled in the GTx-758 125 mg dose arm has completed one 30 day cycle of therapy and management decides to continue testing at the next higher dose, enrollment of the next 25 subjects will commence in the GTx-758 250 mg dosing arm. Similarly, the GTx-758 500 mg dosing arm will commence enrollment of the final 25 subjects when the last subject enrolled in the 250 mg dose arm has 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 decides to continue testing at the next higher dose.

Financial Highlights

Our net loss for the six months ended June 30, 2013 was $25.4 million. We expect to incur significant net losses in 2013 and for the foreseeable future as we continue our clinical development and research and development activities and potentially seek regulatory approval of our product candidates. 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® in the third quarter of 2012, we also currently have no source of revenue. Our current product candidates, enobosarm and GTx-758, will require significant additional research and development and financial resources in order to obtain necessary regulatory approvals for these product candidates and to develop them into commercially viable products. We expect that a substantial portion of our efforts and expenditures in the near term will be devoted to enobosarm, which is the subject of two ongoing pivotal Phase 3 clinical trials. Accordingly, our future prospects are substantially dependent on the successful development, regulatory approval and commercialization of enobosarm for the prevention and treatment of muscle wasting in patients with advanced non-small cell lung cancer. However, we do not expect to obtain FDA approval or any other

regulatory approvals to market any of our product candidates, including enobosarm, in the next twelve months, and it is possible that none of our product candidates will ever receive any regulatory approvals.

At June 30, 2013, we had cash, cash equivalents and short-term investments of $31.6 million compared to $56.1 million at December 31, 2012.

As of the date of this Quarterly Report on Form 10-Q, we estimate that our current cash, cash equivalents, and short-term investments, together with interest thereon, will be sufficient to meet our projected operating requirements for at least the next twelve months. We have based this estimate on our current business plan and assumptions that may prove to be wrong. We could utilize our available capital resources sooner than we currently expect, and we could need additional funding sooner than currently anticipated. In addition, while we believe that, based on our current estimates of clinical trial expenditures and enrollment status, our existing capital resources are adequate to complete all of our ongoing clinical trials of enobosarm and GTx-758, our clinical trials may encounter technical, enrollment or other difficulties that could increase our development costs beyond our current estimates. In any event, we need to raise substantial additional funding in the near term in order to fund our operations, to conduct any additional clinical trials of enobosarm and GTx-758, if the data from our pivotal Phase 3 clinical trials evaluating enobosarm are positive, to seek regulatory approval of enobosarm for the prevention and treatment of muscle wasting in patients with advanced non-small cell lung cancer, and to commercialize enobosarm if approved. If adequate funds are not available to us in the near term, we may be required to delay, reduce the scope of or eliminate one or more of our research or development programs, including our SARM and selective estrogen receptor alpha agonist programs, or conduct additional workforce or other expense reductions, any of which could have a material adverse effect on our business. We do not currently have any commitments for future external funding.

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.

We expect that our research and development expenses for fiscal year 2013 will increase as compared to fiscal year 2012 and to be primarily focused on the following:

- the continued clinical development of enobosarm;
- the continued clinical development of GTx-758; and

· the continued preclinical development of other potential product candidates.

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.

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Product Candidates

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

Product Candidate/ Proposed Indication	Program	Clinical Development Phase	Status
Enobosarm 3 mg Prevention and treatment of muscle wasting in patients with advanced non-small cell lung cancer	SARM	Phase 3	Topline data from the POWER 1 and POWER 2 pivotal Phase 3 clinical trials for the prevention and treatment of muscle wasting in patients with advanced non-small cell lung cancer expected to be announced in the third quarter of 2013.
Enobosarm 9 mg Treatment of women with androgen receptor positive and estrogen receptor positive advanced breast cancer	SARM	Phase 2	Initiated a proof of concept, Phase 2, open-label clinical trial of enobosarm for the treatment of androgen receptor positive and estrogen receptor positive metastatic breast cancer in the second quarter of 2013.
GTx-758 Secondary hormonal therapy in men with metastatic CRPC	Selective estrogen receptor alpha agonist	Phase 2	Phase 2 clinical trial for secondary hormonal therapy in men with metastatic CRPC is ongoing.

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, share-based compensation, long-term service contracts and other contingencies. We base our estimates on historical experience and on various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions.

While our significant accounting policies are more fully described in Note 2 to our financial statements appearing in our Annual Report on Form 10-K for the year ended December 31, 2012 filed with the SEC, we believe

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that the following accounting policies are most critical to aid you in fully understanding and evaluating our reported financial results.

Research and Development Expenses

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

Share-Based Compensation

We have stock option and equity incentive plans that provide for the purchase of our common stock by certain of our employees and non-employee directors. We 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.

The following table summarizes share-based compensation expense included within the condensed statements of operations for the three and six months ended June 30, 2013 and 2012:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2013	2012	2013	2012
	(in thousands)		(in thousands)	
Research and development expenses	$ 353	$ (158)	$ 695	$ 361
General and administrative expenses	361	444	875	852
Total share-based compensation	$ 714	$ 286	$ 1,570	$ 1,213

Share-based compensation expense recorded in the condensed statement of operations as general and administrative expense for the three months ended June 30, 2013 and 2012 included share-based compensation expense related to deferred compensation arrangements for our non-employee directors of $32,000 and $43,000, respectively. Share-based compensation expense recorded in the condensed statement of operations as general and administrative expense for the six months ended June 30, 2013 and 2012 included share-based compensation expense related to deferred compensation arrangements for our non-employee directors of $74,000 and $88,000, respectively. Share-based compensation expense recorded as research and development expense for the three and six months ended June 30, 2012 was offset by the reversal of previously recognized share-based compensation expense for non-vested stock options that were canceled in conjunction with the resignation of an executive officer during the three months ended June 30, 2012. At June 30, 2013, the total compensation cost related to non-vested awards not yet recognized was approximately $6.4 million with a weighted average expense recognition period of 3.37 years.

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Discontinued Operations

Effective September 30, 2012, we completed the sale of FARESTON® and have accounted for FARESTON® as a discontinued operation. As a result, revenue, cost of goods sold, and operating expenses relating to FARESTON® have been excluded from their respective captions in the condensed statements of operations and have been included in discontinued operations for the three and six months ended June 30, 2012.

FARESTON® Revenue Recognition

Revenue from product sales of FARESTON®, which is included in net income from discontinued operations for the three and six months ended June 30, 2012, was recognized less deductions for estimated sales discounts and sales returns. Revenue from product sales was recognized when persuasive evidence of an arrangement existed, title passed, the price was fixed or determinable, and collectability was reasonably assured. We accounted for rebates to certain governmental agencies as a reduction of product sales. We allow customers to return product within a specified time period prior to and subsequent to the product's labeled expiration date. Although we sold our rights and certain assets related to FARESTON® effective September 30, 2012, we retain the liability for future product returns relating to sales of FARESTON® by us prior to September 30, 2012. Therefore, we estimate an accrual for product returns based on factors which include historical product returns and estimated product in the distribution channel which is expected to exceed its expiration date. At June 30, 2013 and December 31, 2012, our accrual for product returns, was $1.0 million and $1.2 million, respectively.

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Results of Operations

Three and Six Months Ended June 30, 2013 and 2012

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 June 30,		Six Months Ended June 30,	
		2013	2012	2013	2012
		(in thousands)			

Enobosarm 3 mg	**SARM**				
Prevention and treatment of muscle wasting in patients with advanced non-small cell lung cancer		$ 6,445	$ 6,179	$ 12,423	$ 11,109
Enobosarm 9 mg	**SARM**				
Treatment of women with androgen receptor positive and estrogen receptor positive advanced breast cancer		628	—	951	—
GTx-758	**Selective ER alpha agonist**				
Secondary hormonal therapy in men with metastatic CRPC		1,368	1,129	3,082	4,240
Other research and development		1,698	1,929	3,297	3,723
Total research and development expenses		$ 10,139	$ 9,237	$ 19,753	$ 19,072

Research and development expenses increased to $10.1 million for the three months ended June 30, 2013 from $9.2 million for the three months ended June 30, 2012. Research and development expenses increased to $19.8 million for the six months ended June 30, 2013 from $19.1 million for the six months ended June 30, 2012.

For both the three months and six months ended June 30, 2013 compared to the respective prior comparable periods, research and development expenses related to enobosarm increased as we continued to conduct the two pivotal Phase 3 POWER 1 and POWER 2 clinical trials for enobosarm 3 mg that were fully enrolled in the fourth quarter of 2012, and initiated in the second quarter of 2013, a Phase 2 clinical trial evaluating enobosarm 9 mg for the treatment of androgen receptor positive and estrogen receptor positive advanced breast cancer in women who have previously responded to hormonal therapy for the treatment of their advanced breast cancer.

Additionally, research and development expenses for the three months ended June 30, 2013 related to GTx-758 increased as compared to the three months ended June 30, 2012. During the three months ended June 30, 2013, we were conducting our ongoing Phase 2 clinical trial evaluating GTx-758 as secondary hormonal therapy in men with metastatic CRPC, which was initiated in the third quarter of 2012. In the first quarter of 2012, we discontinued our three Phase 2 clinical trials of GTx-758 to treat men with advanced prostate cancer. Research and development

expenses for the six months ended June 30, 2013 related to GTx-758 decreased from the six months ended June 30, 2012 due to expenses incurred in 2012 related to the three Phase 2 clinical trials of GTx-758 that were discontinued.

"Other research and development" expenses include the cost of personnel, supplies and facilities associated with preclinical and discovery research and development activities.

General and Administrative Expenses

General and administrative expenses increased 12% to $2.7 million for the three months ended June 30, 2013 from $2.4 million for the three months ended June 30, 2012 due to increased legal costs related primarily to intellectual property activities.

General and administrative expenses increased 14% to $5.7 million for the six months ended June 30, 2013 from $5.0 million for the six months ended June 30, 2012 primarily due to increased legal costs related to intellectual property activities and the preparation of new equity incentive plans.

Discontinued Operations

Income from discontinued operations before income taxes was $1.2 million for the three months ended June 30, 2012 and consisted of net product sales of FARESTON® of $1.6 million reduced by cost of FARESTON® product sales of $245,000 and FARESTON® operating expenses of $191,000.

Income from discontinued operations before income taxes was $2.5 million for the six months ended June 30, 2012 and consisted of net product sales of FARESTON® of $3.5 million reduced by cost of FARESTON® product sales of $519,000 and FARESTON® operating expenses of $411,000.

Liquidity and Capital Resources

At June 30, 2013, we had cash, cash equivalents and short-term investments of $31.6 million, compared to $56.1 million at December 31, 2012. Net cash used in operating activities was $25.3 million and $18.4 million for the six months ended June 30, 2013 and 2012, respectively.

Net cash provided by investing activities was $4.6 million for the six months ended June 30, 2013 and resulted primarily from the maturities of short-term investments of $5.9 million offset by the purchase of short-term investments of $1.2 million. Net cash provided by investing activities was $2.0 million for the six months ended June 30, 2012 and resulted primarily from the maturities of short-term investments of $6.8 million offset by the purchase of short-term investments of $4.7 million.

Net cash provided by financing activities was $865,000 for the six months ended June 30, 2013 and reflects proceeds from the exercise of employee stock options of $869,000 partially offset by payments on capital lease obligations of $4,000. Net cash provided by financing activities was $19,000 for the six months ended June 30, 2012 and was provided primarily from proceeds from the exercise of employee stock options of $63,000 partially offset by payments on capital lease and financed equipment obligations of $44,000.

As of the date of this Quarterly Report on Form 10-Q, we estimate that our current cash, cash equivalents, and short-term investments, together with interest thereon, will be sufficient to meet our projected operating requirements for at least the next twelve months. We have based this estimate on our

current business plan and assumptions that may prove to be wrong. We could utilize our available capital resources sooner than we currently expect, and we could need additional funding sooner than currently anticipated. In addition, while we believe that, based on our current estimates of clinical trial expenditures and enrollment status, our existing capital resources are adequate to complete all of our ongoing clinical trials of enobosarm and GTx-758, our clinical trials may encounter technical, enrollment or other difficulties that could increase our development costs beyond our current estimates. In any event, we need to raise substantial additional funding in the near term in order to fund our operations, to conduct any additional clinical trials of enobosarm and GTx-758, if the data from our pivotal Phase 3 clinical trials evaluating

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enobosarm are positive, to seek regulatory approval of enobosarm for the prevention and treatment of muscle wasting in patients with advanced non-small cell lung cancer, and to commercialize enobosarm if approved.

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 potential 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 trials and other research and development activities, including our ongoing and any future clinical trials of enobosarm and GTx-758;
- the terms and timing of any potential collaborative, licensing and other arrangements that we may establish;
- the decision to initiate development of new potential medicines from our research and discovery activities;
- the amount and timing of any license 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 cost and timing of establishing medical education, sales, marketing and distribution capabilities;
- the cost of establishing clinical and commercial supplies of our product candidates and any products that we and/or any potential collaborators may develop;
- the effect of competing technological and market developments;
- the cost of filing, prosecuting, defending and enforcing any patent claims and other intellectual property rights, and the cost of defending any other litigation claims; and
- the extent to which we acquire or invest in businesses, products and technologies, although we currently have no commitments or agreements relating to any of these types of transactions.

We do not currently have any commitments for future external funding nor do we currently have any sources of revenue. Until we can generate a sufficient amount of product revenue, which we may never do, we expect to finance future cash needs through public or private equity offerings, debt financings or collaboration and licensing arrangements, or a combination of the above, as well as through interest income earned on the investment of our cash balances and short-term investments. In June 2011, we announced a workforce reduction of approximately 15% in order to reduce our operating expenses relating to our discontinued toremifene development programs. If we are unable to raise additional funds in the near term, we may need to further reduce our expenditures, perhaps significantly, to preserve our cash. Cost-cutting measures that we may take in the future may not be sufficient to enable us to meet our cash requirements, and they may negatively affect our business and growth prospects. To the extent we raise additional funds by issuing equity securities, our stockholders may experience dilution, and debt

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financing, if available, may involve restrictive covenants. Any debt financing or additional equity that we raise may contain terms that are not favorable to us or our stockholders. To the extent we raise additional funds through potential collaboration and licensing 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. Our ability to raise additional funds and the terms upon which we are able to raise such funds may be adversely impacted, perhaps significantly, by the outcomes of ongoing clinical trials of enobosarm and GTx-758, uncertainty regarding our financial condition and the sufficiency of our capital resources and/or current economic conditions, including the effects of, disruptions to and volatility in the credit and financial markets in the United States, the European Union and other regions of the world. As a result of these and other factors, we cannot be certain that additional funding will be available on acceptable terms, or at all. If adequate funds are not available to us in the near term, we may be required to delay, reduce the scope of or eliminate one or more of our research or development programs, including our SARM and selective ER alpha agonist programs, or conduct additional workforce or other expense reductions, any of which could have a material adverse effect on our business.

ITEM 3. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK

During the six months ended June 30, 2013, 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, 2012.

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 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 Chief Executive Officer and Chief 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 Chief Executive Officer and Chief 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 Chief Executive Officer and Chief 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 second quarter of 2013 that have 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 5, 2013.

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 June 30, 2013, we had an accumulated deficit of $438.6 million. Our net loss for the six months ended June 30, 2013 was $25.4 million. We expect to incur significant net losses in 2013 and for the foreseeable future as we continue our clinical development and research and 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 research and development and financial resources in order to obtain necessary regulatory approvals for these product candidates and to develop them into commercially viable products. We expect that a substantial portion of our efforts and expenditures in the near term will be devoted to enobosarm, which is the subject of two ongoing pivotal Phase 3 clinical trials. Accordingly, our future prospects are substantially dependent on the successful development, regulatory approval and commercialization of enobosarm for the prevention and treatment of muscle wasting in patients with advanced non-small cell lung cancer. However, we do not expect to obtain FDA approval or any other regulatory approvals to market any of our product candidates, including enobosarm, in the next twelve months, 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 financed our operations and internal growth 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. While we believe that, based on our current estimates of clinical trial expenditures and enrollment status, our existing capital resources are adequate to complete all of our ongoing clinical trials of enobosarm and GTx-758, we need to raise substantial additional funding in the near term in order to fund our operations, to conduct any additional clinical trials of enobosarm and GTx-758, and, if the data from our pivotal Phase 3 clinical trials evaluating enobosarm are positive, to seek regulatory approval of enobosarm for the prevention and treatment of muscle wasting in patients with advanced non-small cell lung cancer.

If we and/or any potential collaborators are unable to develop and commercialize any of our product candidates, if development is further delayed or is eliminated, or if sales revenue from any product candidate that receives marketing approval is insufficient, we may never become profitable and we will not be successful.

We 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 or commercialization efforts.*

We need to raise substantial additional capital in the near term to:

- fund our operations and conduct future clinical trials;

- 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

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commercial sale.

As of the date of this Quarterly Report on Form 10-Q, we estimate that our current cash, cash equivalents, and short-term investments, together with interest thereon, will be sufficient to meet our projected operating requirements for at least the next twelve months. We have based this estimate on our current business plan and assumptions that may prove to be wrong. We could utilize our available capital resources sooner than we currently expect, and we could need additional funding sooner than currently anticipated. In addition, while we believe that, based on our current estimates of clinical trial expenditures and enrollment status, our existing capital resources are adequate to complete all of our ongoing clinical trials of enobosarm and GTx-758, our clinical trials may encounter technical, enrollment or other difficulties that could increase our development costs beyond our current estimates. In any event, we need to raise substantial additional funding in the near term in order to fund our operations, to conduct any additional clinical trials of enobosarm and GTx-758, if the data from our pivotal Phase 3 clinical trials evaluating enobosarm are positive, to seek regulatory approval of enobosarm for the prevention and treatment of muscle wasting in patients with advanced non-small cell lung cancer, and to commercialize enobosarm if approved. Our future funding requirements will depend on many factors, including:

- the scope, rate of progress and cost of our clinical trials and other research and development activities, including our ongoing and any future clinical trials of enobosarm and GTx-758;
- the terms and timing of any potential collaborative, licensing and other arrangements that we may establish;
- the decision to initiate development of new potential medicines from our research and discovery activities;
- 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 cost and timing of establishing medical education, sales, marketing and distribution capabilities;
- the cost of establishing clinical and commercial supplies of our product candidates and any products that we and/or any potential collaborators may develop;
- the effect of competing technological and market developments;
- the cost of filing, prosecuting, defending and enforcing any patent claims and other intellectual property rights, and the cost of defending any other litigation claims; and
- the extent to which we acquire or invest in businesses, products and technologies, although we currently have no commitments or agreements relating to any of these types of transactions.

We do not currently have any commitments for future external funding nor do we currently have any sources of revenue. Until we can generate a sufficient amount of product revenue, which we may never do, we expect to finance future cash needs through public or private equity offerings, debt financings or collaboration and licensing arrangements, or a combination of the above, as well as through interest income earned on the investment of our cash balances and short-term investments. In June 2011, we announced a workforce reduction of approximately 15% in order to reduce our operating expenses relating to our discontinued toremifene development programs. If we are unable to raise additional funds in the near term, we may need to further reduce our expenditures, perhaps significantly, to preserve our cash. Cost-cutting measures that we may take in the future may not be sufficient to enable us to meet our cash requirements, and they may negatively affect our business and growth prospects.

To the extent we raise additional funds by issuing equity securities, our stockholders may experience dilution, and debt financing, if available, may involve restrictive covenants. Any debt financing or additional equity that we raise may contain terms that are not favorable to us or our stockholders. To the extent we raise additional funds through potential collaboration and licensing 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. Our ability to raise

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additional funds and the terms upon which we are able to raise such funds may be adversely impacted, perhaps significantly, by the outcomes of ongoing clinical trials of enobosarm and GTx-758, uncertainty regarding our financial condition and the sufficiency of our capital resources and/or current economic conditions, including the effects of, disruptions to and volatility in the credit and financial markets in the United States, the European Union and other regions of the world. As a result of these and other factors, we cannot be certain that additional funding will be available on acceptable terms, or at all. If adequate funds are not available to us in the near term, we may be required to delay, reduce the scope of or eliminate one or more of our research or development

programs, including our SARM and selective ER alpha agonist programs, or conduct additional workforce or other expense reductions, any of which could have a material adverse effect on our business.

Risks Related to Development of Product Candidates

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 research and 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 May 2010 that toremifene 20 mg failed to meet its primary efficacy endpoint in our Phase 3 clinical trial of toremifene 20 mg for the prevention of prostate cancer in high risk men with high grade prostatic intraepithelial neoplasia, after we had incurred significant development costs. Even if the results of a clinical trial are positive, the efficacy and/or safety results from the trial may be insufficient to support the submission of a new drug application, or 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 being studied in the 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 primary treatment for advanced prostate cancer used in combination with ADT, is dependent on our ability to find an appropriate dose that is both effective and safe for these patient populations. If an unacceptable incidence of venous thromboembolic events or other adverse events are observed in our current Phase 2 clinical trial of GTx-758, we may be required to abandon our development of GTx-758, in which case, we would not receive any return on our investment in that product candidate.

In our Phase 2 clinical trials for enobosarm for the treatment of muscle wasting in patients with cancer and healthy older males and postmenopausal females, we observed mild elevations of hepatic enzymes, which in certain circumstances may lead to liver failure, in a few patients in both the placebo and

enobosarm treated groups. Reductions in high-density lipoproteins have also been observed in subjects treated with enobosarm. Lower levels of high-density lipoproteins could lead to increased risk of adverse cardiovascular events.

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

- 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 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. If we elect to increase our expenditures to fund development or commercialization activities on our own, we will need to raise substantial additional capital, which may not be available to us on acceptable terms, or at all. If we do not have sufficient funds, we will not be able to bring our product candidates to market and generate product revenues.

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

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

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 our supply of enobosarm becomes unusable or 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 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, 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. On September 16, 2011, the Leahy-Smith America Invents Act, or the Leahy-Smith Act, was signed into law. The Leahy-Smith Act includes a number of significant changes to United States patent law. These include provisions that affect the way patent applications will be prosecuted and may also affect patent defense and enforcement. For example, the Leahy-Smith Act has introduced procedures making it easier for third-parties to challenge issued patents, as well as to intervene in the prosecution of patent applications. In addition, the Leahy-Smith Act will change the United States from a "first-to-invent" jurisdiction to a "first-inventor-to-file" jurisdiction and will change the definition of what constitutes prior art for an application. Finally, the Leahy-Smith Act contains new statutory provisions that still require the U.S. Patent and Trademark Office to issue new regulations for their implementation and it may take the courts years to interpret the provisions of the new statute. The Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have a material adverse effect on our business and financial condition.

We 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 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 drug discovery, development, and manufacture and process synthesis 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. 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 or any other

regulatory approvals to market any of our product candidates in the next twelve months, 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. For example, on July 9, 2012, Congress enacted the Food and Drug Administration Safety and Innovation Act (FDASIA) that, among other things, reauthorizes the Prescription Drug User Fee Act, or PDUFA, for an additional five years. FDASIA incorporates new FDA performance goals that effectively extend by two months the time period in which the FDA is expected to review and approve certain NDAs. Although the FDA has stated that it expects to meet PDUFA's updated timing goals, it has in the past provided its managers discretion to miss them due to heightened agency workload or understaffing in the review divisions; accordingly, it remains unclear whether and to what extent the FDA will adhere to PDUFA timing goals in the future. If the FDA were to miss a PDUFA timing goal for one of our product candidates, the development and commercialization of the product candidate could be delayed. In addition, the Food and Drug Administration Amendments Act of 2007, or FDAAA, which was enacted in September 2007, expands the FDA's authority to regulate drugs throughout the product life cycle, including enhanced authority to require post-approval studies and clinical trials. Other proposals have been made to impose additional requirements on drug approvals, further expand post-approval requirements and restrict sales and promotional activities. This legislation, and the additional proposals if enacted, may make it more difficult or burdensome for us or our potential collaborators to obtain approval of our product candidates. Even if the FDA approves a product candidate, the approval may impose significant restrictions on the indicated uses, conditions for use, labeling, advertising, promotion, marketing and/or production of such product, and may impose ongoing requirements for post-approval studies, including additional research and development and clinical trials. The 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 also may impose various civil or criminal sanctions for failure to comply with regulatory requirements, including withdrawal of product approval.

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

The FDA has substantial discretion in the approval process and may refuse to accept any application or may decide that our data 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 have met with the FDA to discuss the development program and required endpoints to obtain approval of enobosarm, there can be no assurance that the FDA will ultimately determine that data from our current pivotal Phase 3 clinical trials of enobosarm will be sufficient for approval of this product candidate. 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 for marketing approval of a product candidate, it may not result in marketing approval from the FDA.

We do not expect to receive regulatory approval for the commercial sale of any of our product candidates that are in development in the next twelve months, if at all. The inability to obtain FDA approval or approval from comparable authorities in other countries for our product candidates would prevent us or any potential collaborators from commercializing these product candidates in the United States 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 5, 2013, for additional information regarding risks associated with marketing approval, as well as risks related to potential post-approval requirements.

The "fast track" designation for development of any of our product candidates may not lead to a faster development or regulatory review or approval process and it does not increase the likelihood the product candidate will receive regulatory approval.

If a product candidate is intended for the treatment of a serious or life-threatening condition and the product candidate demonstrates the potential to address unmet medical needs for this condition, the drug sponsor may apply for FDA "fast track" designation for a particular indication. Marketing applications filed by sponsors of product candidates in the fast track process may qualify for priority review under the policies and procedures offered by the FDA, but the fast track designation does not assure any such review. Although we have obtained a fast track designation from the FDA for enobosarm for the prevention and treatment of muscle wasting in patients with advanced non-small cell lung cancer, receipt of fast track designation may not result in a faster development process, review or approval compared to drugs considered for approval under conventional FDA procedures. In addition, the FDA may withdraw our fast track designation at any time. If we lose our fast track designation for enobosarm, the approval process may be delayed. In addition, our fast track designation does not guarantee that we will be able to take advantage of the expedited review procedures and does not increase the likelihood that enobosarm will receive any regulatory approvals.

Risks Related to Commercialization

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

Any products that we and/or any potential collaborators may develop may not gain market acceptance among physicians, patients, health care payors and the medical community. If these products do not achieve an adequate level of acceptance, we may not generate material product revenues 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

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sales, marketing and distribution services, our product revenues are likely to be lower than if we market and sell any products that we develop ourselves.

If we and/or any potential collaborators are unable to obtain reimbursement or experience a reduction in reimbursement from third-party payors for products we sell, our revenues and prospects for profitability will suffer.

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

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

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

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

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

Similar cost containment initiatives exist in countries outside of the United States, particularly in the countries of the European Union, where the pricing of prescription pharmaceuticals is subject to governmental control. In these countries, pricing negotiations with governmental authorities can extend well beyond the receipt of regulatory marketing approval for a product and may require us or any potential collaborators to conduct a clinical trial that compares the cost effectiveness of our product candidates or products to other available therapies. The conduct of such a clinical trial could be expensive and result in delays in our or a potential collaborators' commercialization efforts. Third-party payors are challenging the prices charged for medical products and services, and many third-party payors limit reimbursement for newly-approved health care products. Recently budgetary pressures in many European Union countries are also causing governments to consider or implement various cost-containment measures, such as price freezes, increased price cuts and rebates. If budget pressures continue, governments may implement additional cost containment measures. Cost-control initiatives could decrease the price we might establish for products that we or any potential collaborators may develop or sell, which would result in lower product revenues or royalties payable to us.

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

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

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

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

If product liability lawsuits are brought against us, we may incur substantial liabilities and may be required to limit commercialization of any products that we may develop.

We face an inherent risk of product liability exposure related to our prior commercial sales of FARESTON® and the testing of our product candidates in human clinical trials, and we will face an even greater risk if we commercially sell any product that we may develop. If we cannot successfully defend ourselves against claims that our product candidates or products caused injuries, we will incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for any product candidates or products;
- injury to our reputation;
- withdrawal of clinical trial participants;
- 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 $30 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.

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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 non-small cell lung cancer. 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 CRPC, and, potentially, as a primary treatment for advanced prostate cancer used in combination with androgen deprivation therapy. There are various products approved or under clinical development to treat men with advanced prostate cancer who have metastatic CRPC which may compete with GTx-758. Dendreon Corporation markets and sells Provenge®, an autologous cellular immunotherapy, for the treatment of asymptomatic or minimally symptomatic metastatic castrate resistant prostate cancer. Medivation, Inc. has received approval for Xtandi®, an oral androgen receptor antagonist, for the treatment of metastatic castration-resistant prostate cancer in men previously treated with docetaxel. Medivation continues to develop Xtandi® for men with metastatic CRPC prior to receiving chemotherapy. Zytiga®, sold by Johnson & Johnson, has been approved for the treatment of metastatic CRPC prior in patients who have received prior chemotherapy and recently received approval for the treatment of metastatic castrate resistant prostate cancer prior to chemotherapy. Johnson & Johnson has agreed to acquire Aragon Pharmaceuticals, Inc., which has 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 and post docetaxel. GTx-758 is being developed as a treatment for this same patient population prior to the initiation of chemotherapy.

Many of our competitors have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals and marketing approved products than we do. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These third parties compete with us in recruiting and retaining qualified scientific and management personnel, establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies and technology licenses complementary to our programs or advantageous to our business.

Risks Related to Employees and Growth

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

Our success depends on our continued ability to attract, retain and motivate highly qualified management, clinical and scientific personnel and on our ability to develop and maintain important relationships with leading academic institutions, clinicians and scientists. If we are not able to attract and keep senior management and key scientific personnel, particularly Dr. Mitchell S. Steiner, we may not be able to successfully develop or commercialize our product candidates. All of our employees are at-will employees and can terminate their employment at any time. We do not carry “key person” insurance covering members of senior management, other than $22.5 million of insurance covering Dr. Steiner.

In December 2009, we announced a reduction of approximately 26% of our workforce in order to reduce our operating expenses in connection with the receipt of the Complete Response Letter regarding our NDA for

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toremifene 80 mg. We also announced a reduction of approximately 15% of our workforce in June 2011 in connection with our decision to discontinue the development of toremifene 80 mg and toremifene 20 mg. These and any future workforce reductions may negatively affect our ability to retain or attract talented employees.

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

In order to commercialize our product candidates in the future, we may need to hire experienced sales and marketing personnel to sell and market those product candidates we decide to commercialize, and we will need to expand the number of our managerial, operational, financial and other employees to support commercialization. Competition exists for qualified personnel in the biotechnology field.

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

Risks Related to Our Common Stock

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

The market prices for securities of 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:

- delays in the initiation, enrollment and/or completion of our ongoing 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;
- our ability to enter into new collaborative arrangements with respect to our product candidates;
- the terms and timing of any future collaborative, licensing or other arrangements that we may establish;
- our ability to raise additional capital to carry through with our clinical development plans and current and future operations, and the terms and timing of any related financing arrangements;
- 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;
- regulatory developments in the United States and foreign countries;
- changes in the structure or reimbursement policies of health care payment systems;

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- any intellectual property infringement lawsuit involving us;
- actual or anticipated fluctuations in our results of operations;
- changes in financial estimates or recommendations by securities analysts;
- hedging or arbitrage trading activity that may develop regarding our common stock;
- sales of large blocks of our common stock;
- sales of our common stock by our executive officers, directors and significant stockholders;
- changes in accounting principles; and
- the loss of any of our key scientific or management personnel.

The stock markets in general, and the markets for biotechnology stocks in particular, have experienced significant volatility that has often been unrelated to the operating performance of particular companies. The financial markets continue to face significant uncertainty, resulting in a decline in investor confidence and concerns about the proper functioning of the securities markets, which decline in general investor confidence has resulted in depressed stock prices for many companies notwithstanding the lack of a fundamental change in their underlying business models or prospects. These broad market fluctuations may adversely affect the trading price of our common stock.

In the past, class action litigation has often been instituted against companies whose securities have experienced periods of volatility in market price. Any such litigation brought against us could result in substantial costs, which would hurt our financial condition and results of operations and divert management's attention and resources, which could result in delays of our clinical trials or commercialization efforts.

Our executive officers, directors and largest stockholders have the ability to control all matters submitted to stockholders for approval.*

As of June 30, 2013, our executive officers, directors and holders of 5% or more of our outstanding common stock beneficially owned approximately 63% of our outstanding common stock, and our executive officers and directors alone beneficially owned approximately 38% of our outstanding common stock. As a result, these stockholders, acting together, may or will 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.

Anti-takeover provisions in our charter documents and under Delaware law could make an acquisition of us, which may be beneficial to our stockholders, more difficult and may prevent attempts by our stockholders to replace or remove our current management.

Provisions in our certificate of incorporation and our bylaws may delay or prevent an acquisition of us or a change in our management. In addition, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our Board of Directors. Because our Board of Directors is responsible for appointing the members of our management team, these provisions could in turn affect any attempt by our stockholders to replace current members of our management team. These provisions include:

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

Moreover, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, which prohibits a person who owns in excess of 15% of our outstanding voting

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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 June 30, 2013, the average daily trading volume of our common stock on The NASDAQ Global Market was 202,720 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 June 30, 2013, we had 63,110,430 shares of common stock outstanding.

Moreover, J.R. Hyde, III, our largest stockholder, and certain of his affiliates, have rights, subject to some conditions, to require us to file registration statements covering the approximately 7.9 million shares of common stock held in the aggregate which are subject to registration rights 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 were to sell large blocks of shares in a short period of time, the market price of our common stock could drop substantially.

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: July 22, 2013

By: /s/ Mitchell S. Steiner
Mitchell S. Steiner, Chief Executive Officer
and Vice-Chairman of the Board of Directors
(Principal Executive Officer)

Date: July 22, 2013

By: /s/ Mark E. Mosteller
Mark E. Mosteller, Vice President
and Chief Financial Officer
(Principal Financial and Accounting Officer)

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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	8-K	000-50549	2.1	10/03/2012

	Registrant and Strakan International S.à r.l.				
3.1	Restated Certificate of Incorporation of GTx, Inc.	S-3	333-127175	4.1	08/04/2005
3.2	Certificate of Amendment of Restated Certificate of Incorporation of GTx, Inc.	8-K	000-50549	3.2	05/06/2011
3.3	Amended and Restated Bylaws of GTx, Inc.	8-K	000-50549	3.2	07/26/2007
4.1	Reference is made to Exhibits 3.1, 3.2 and 3.3				
4.2	Specimen of Common Stock Certificate	S-1	333-109700	4.2	12/22/2003
4.3	Amended and Restated Registration Rights Agreement between Registrant and J. R. Hyde, III dated August 7, 2003	S-1	333-109700	4.4	10/15/2003
4.4	Consent, Waiver and Amendment 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
10.1	GTx, Inc. 2013 Equity Incentive Plan	S-8	333-188377	99.1	05/06/2013
10.2+	Form of Stock Option Grant Notice and Option Agreement under the GTx, Inc. 2013 Equity Incentive Plan				
10.3	GTx, Inc. 2013 Non-Employee Director Equity Incentive Plan	S-8	333-188377	99.2	05/06/2013
10.4+	Form of Stock Option Grant Notice and Option Agreement under the GTx, Inc. 2013 Non-Employee Director Equity Incentive Plan				
10.5+	Second Memorandum of Understanding Concerning the Lease Agreement between Registrant and The University of Tennessee Research Foundation as Amended July 20, 2009				
31.1+	Certification of Chief Executive Officer, as required by Rule 13a-14(a) or Rule 15d-14(a)				
31.2+	Certification of Chief Financial Officer, as required by Rule 13a-14(a) or Rule 15d-14(a)				
32.1+	Certification of Chief Executive Officer, as required by Rule 13a-14(b) or Rule 15d-14(b) and Section 1350 of Chapter 63 of Title 18 of the United States Code (18 U.S.C. §1350)(1)				
32.2+	Certification of Chief 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(2)				
101.SCH+	XBRL Taxonomy Extension Schema Document(2)				
101.CAL+	XBRL Taxonomy Extension Calculation Linkbase Document(2)				
101.DEF+	XBRL Taxonomy Extension Definition Linkbase Document(2)				
101.LAB+	XBRL Taxonomy Extension Labels Linkbase Document(2)				
101.PRE+	XBRL Taxonomy Extension Presentation Linkbase Document(2)				

+ 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

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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.

(2) Pursuant to applicable securities laws and regulations, the Registrant is deemed to have complied with the reporting obligation relating to the submission of interactive data files in such exhibits and is not subject to liability under any anti-fraud provisions of the federal securities laws as long as the Registrant has made a good faith attempt to comply with the submission requirements and promptly amends the interactive data files after becoming

aware that the interactive data files fail to comply with the submission requirements. These interactive data files are deemed not filed or part of a registration statement or prospectus for purposes of sections 11 or 12 of the Securities Act of 1933, as amended, are deemed not filed for purposes of section 18 of the Securities Exchange Act of 1934, as amended, and otherwise are not subject to liability under these sections.

GTx, INC.
STOCK OPTION GRANT NOTICE
(2013 EQUITY INCENTIVE PLAN)

GTx, Inc. (the "***Company***"), pursuant to its 2013 Equity Incentive Plan (the "***Plan***"), hereby grants to Optionholder an option (the "***Option***") to purchase the number of shares of the Company's Common Stock set forth below. The Option is subject to all of the terms and conditions as set forth in this notice (the "***Grant Notice***"), in the Option Agreement and in the Plan, both of which are incorporated herein in their entirety. For your convenience, a copy of the Option Agreement is attached hereto. A copy of the Plan is available from the Company on request. Capitalized terms not explicitly defined herein but defined in the Plan or the Option Agreement will have the same definitions as in the Plan or the Option Agreement. If there is any conflict between the terms in the Option Agreement and the Plan, the terms of the Plan will control.

Optionholder:
Date of Grant:
Vesting Commencement Date:
Number of Shares Subject to Option:
Exercise Price (Per Share):
Total Exercise Price:
Expiration Date:

Type of Grant: o Incentive Stock Option o Nonstatutory Stock Option

Exercise Schedule: Same as Vesting Schedule

Vesting Schedule: Subject to any accelerated vesting pursuant to the terms of the Option Agreement or as otherwise specifically determined by the Board (or an authorized committee thereof), the Option vests with respect to twenty percent (20%) of the total number of shares of Common Stock subject to the Option (rounded down to the nearest whole share) on each of the first, second, third, fourth and fifth anniversaries of the Vesting Commencement Date, subject to Optionholder's Continuous Service on each applicable vesting date.

Payment: By one or a combination of the following items:

- o By cash (as described in the Plan)
- o Pursuant to a Regulation T Program, if the Common Stock is publicly traded
- o By delivery of already-owned shares, if the Common Stock is publicly traded
- o If and only to the extent the option is a Nonstatutory Stock Option, and subject to the Company's consent at the time of exercise, by a "net exercise" arrangement

Additional Terms/Acknowledgements: Optionholder acknowledges receipt of, and understands and agrees to, this Grant Notice, the Option Agreement, the Plan and the stock plan prospectus for the Plan. As of the Date of Grant, this Grant Notice, the Option Agreement, and the Plan set forth the entire understanding between Optionholder and the Company regarding the Option and supersede all prior oral and written agreements with respect to the Option, with the exception, if applicable, of (i) any written employment agreement or offer letter agreement between the Company and Optionholder specifying the terms that should govern the Option and (ii) any compensation recovery policy that is adopted by the Company or is otherwise required by applicable law. By accepting the Option, Optionholder consents to receive documents governing the Option by electronic delivery and to participate in the Plan through an on-line or electronic system established and maintained by the Company or another third party designated by the Company.

* * *

GTx, INC.

By: _____
Signature

Title: _____

Date: _____

OPTIONHOLDER:

_____
Signature

Date: _____

ATTACHMENT: Option Agreement

ATTACHMENT I

GTx, INC.
2013 EQUITY INCENTIVE PLAN

OPTION AGREEMENT
(INCENTIVE STOCK OPTION OR NONSTATUTORY STOCK OPTION)

Pursuant to your Stock Option Grant Notice (the "***Grant Notice***") and this Option Agreement (this "***Option Agreement***"), GTx Inc. (the "***Company***") has granted you an option (the "***Option***") under its 2013 Equity Incentive Plan (the "***Plan***") to purchase the number of shares of the Company's Common

Stock indicated in your Grant Notice at the exercise price indicated in your Grant Notice. The Option is granted to you effective as of the date of grant set forth in the Grant Notice (the "***Date of Grant***"). If there is any conflict between the terms in this Option Agreement and the Plan, the terms of the Plan will control. Defined terms not explicitly defined in this Option Agreement or in the Grant Notice, but defined in the Plan, will have the same definitions as in the Plan.

The details of the Option, in addition to those set forth in the Grant Notice and the Plan, are as follows:

1. VESTING. Subject to Section 7 below, the Option will vest as provided in your Grant Notice. Unless specifically provided to the contrary by the Board (or an authorized committee thereof), vesting will cease, in all events, upon the termination of your Continuous Service.

2. NUMBER OF SHARES AND EXERCISE PRICE. The number of shares of Common Stock subject to the Option and the exercise price per share in your Grant Notice will be adjusted for Capitalization Adjustments as provided in the Plan.

3. EXERCISE RESTRICTION FOR NON-EXEMPT EMPLOYEES. If you are an Employee eligible for overtime compensation under the Fair Labor Standards Act of 1938, as amended (that is, a "***Non-Exempt Employee***"), and except as otherwise provided in the Plan, you may not exercise your option until you have completed at least six (6) months of Continuous Service measured from the Date of Grant, even if you have already been an employee for more than six (6) months. Consistent with the provisions of the Worker Economic Opportunity Act, you may exercise the Option as to any vested portion prior to such six (6) month anniversary in the case of (i) your death or Disability, (ii) a Corporate Transaction in which the Option is not assumed, continued or substituted, (iii) a Change in Control, or (iv) your termination of Continuous Service on your Retirement.

4. EXERCISE PRIOR TO VESTING ("EARLY EXERCISE"). You may not exercise the Option prior to vesting.

5. METHOD OF PAYMENT. You must pay the full amount of the exercise price for the shares of Common Stock subject to the Option that you wish to exercise. You may pay the

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exercise price in cash (as described in the Plan) or in any other manner ***permitted by your Grant Notice,*** which may include one or more of the following:

(a) Provided that at the time of exercise the Common Stock is publicly traded, pursuant to a program developed under Regulation T as promulgated by the Federal Reserve Board that, prior to the issuance of Common Stock, results in either the receipt of cash (or check) by the Company or the receipt of irrevocable instructions to pay the aggregate exercise price to the Company from the sales proceeds. This manner of payment is also known as a "broker-assisted exercise," "same day sale," or "sell to cover."

(b) Provided that at the time of exercise the Common Stock is publicly traded, by delivery to the Company (either by actual delivery or attestation) of already-owned shares of Common Stock that are owned free and clear of any liens, claims, encumbrances or security interests, and that are valued at Fair Market Value on the date of exercise. "***Delivery***" for these purposes, in the sole discretion of the Company at the time you exercise the Option (or any vested portion thereof), will include delivery to the Company of your attestation of ownership of such shares of Common Stock in a form approved by the Company. You may not exercise the Option (or any exercisable portion thereof) by delivery to the Company of Common Stock if doing so would violate the provisions of any law, regulation or agreement restricting the redemption of the Company's stock.

(c) If the Option is a Nonstatutory Stock Option, subject to the consent of the Company at the time of exercise, by a "net exercise" arrangement pursuant to which the Company will reduce the number of shares of Common Stock issued upon exercise of the Option (or any vested portion thereof) by the largest whole number of shares with a Fair Market Value that does not exceed the aggregate exercise price. You must pay any remaining balance of the aggregate exercise price not satisfied by the "net exercise" in cash or other permitted form of payment. Shares of Common Stock will no longer be outstanding under the Option and will not be exercisable thereafter if those shares (i) are used to pay the exercise price pursuant to the "net exercise," (ii) are delivered to you as a result of such exercise, and (iii) are withheld to satisfy your tax withholding obligations.

6. WHOLE SHARES. You may exercise the Option (or any vested portion thereof) only for whole shares of Common Stock.

7. VESTING ACCELERATION AFTER A CHANGE IN CONTROL

(a) If a Change in Control occurs and either (i) your Continuous Service with the Company or its successor or the successor's parent (together, the "***Successor Company***") is terminated by the Successor Company without Cause (as defined in the Plan) or (ii) your Continuous Service with the Successor Company is terminated as a result of a Constructive Termination (as defined below), in either case, on or within twelve (12) months after the effective time of the Change in Control, then, immediately prior to such termination, the outstanding and unvested portion of the Option shall become fully vested and exercisable.

(b) For purposes of this Option Agreement, "***Constructive Termination***" means that you terminate your employment with the Company (or the Successor Company) on or within twelve (12) months following a Change in Control if any of the following occurs:

(i) the board of the Successor Company requires you to resign from the Company in a manner that terminates your Continuous Service, as a condition of the closing of the Change in Control;

(ii) the assignment to you of any duties or responsibilities which results in a significant diminution in your function as in effect immediately prior to the effective date of the Change in Control; *provided*, *however*, that a mere change in your title or reporting relationships shall not constitute a Constructive Termination; or

(iii) a material reduction of at least five percent (5%) by the Company (or the Successor Company) in your annual base salary, as in effect on the effective date of the Change in Control; or

(iv) any failure by the Company (or the Successor Company) to continue in effect any benefit plan or program, including fringe benefits and incentive plans, in which you are participating immediately prior to the effective date of the Change in Control (hereinafter referred to as "***Benefit Plans***"); or the taking of any action by the Company (or the Successor Company) that would adversely affect your participation in or reduce your benefits under the Benefit Plans; *provided, however,* that a "Constructive Termination" shall not exist under this paragraph following a Change in Control if the Company (or the Successor Company) offers a range of benefit plans and programs which, taken as a whole, are comparable to the Benefit Plans or are substantially similar to the benefit plans offered by the Successor Company to its similarly situated employees;

(v) the relocation of your business office to a location more than fifty (50) miles from the location at which you performed duties as of the effective date of the Change in Control, except for required travel by you on business of the Company (or the Successor Company) to an extent substantially consistent with your business travel obligations prior to the Change in Control; or

(vi) a material breach by the Company (or the Successor Company) of any provision of this Option Agreement;

provided, however, that in order to have a basis for Constructive Termination, you must provide written notice of such event to the board of the Successor Company within thirty (30) days following the event giving rise to Constructive Termination, provide the Successor Company with thirty (30) days to cure such event, and, if not cured, your resignation from all positions you then hold with the Company and the Successor Company is effective not later than six (6) months after the date of your written notice to the board (or such earlier date as reasonably requested in writing by the board of the Successor Company).

8. SECURITIES LAW COMPLIANCE. In no event may you exercise the Option (or any vested portion thereof) unless the shares of Common Stock issuable upon exercise are then registered under the Securities Act or, if not registered, the Company has determined that your

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exercise and the issuance of the shares would be exempt from the registration requirements of the Securities Act. The exercise of the Option (or any vested portion thereof) also must comply with all other applicable laws and regulations governing the Option, and you may not exercise the Option (or any vested portion thereof) if the Company determines that such exercise would not be in material compliance with such laws and regulations (including any restrictions on exercise required for compliance with Treas. Reg. 1.401(k)-1(d)(3), if applicable).

9. TERM. You may not exercise the Option before the Date of Grant or after the expiration of the term of the Option. The term of the Option expires, subject to the provisions of Section 5(h) of the Plan, upon the earliest of the following:

(a) immediately upon the date on which the event giving rise to the termination for Cause first occurred (or, if required by law, the date of the termination of your Continuous Service for Cause);

(b) three (3) months after the termination of your Continuous Service for any reason other than for Cause, your Retirement, your Disability or your death (except as otherwise provided in Section 9(d) below); *provided, however,* that if during any part of such three (3) month period the Option is not exercisable solely because doing so would violate the registration requirements under the Securities Act, the Option will not expire until the earlier of the Expiration Date or until it has been exercisable for an aggregate period of three (3) months after the termination of your Continuous Service; *provided further,* that if (i) you are a Non-Exempt Employee, (ii) your Continuous Service terminates within six (6) months after the Date of Grant, and (iii) you have vested in a portion of the Option at the time of your termination of Continuous Service, the Option will not expire until the earlier of (A) the later of (x) the date that is seven (7) months after the Date of Grant, and (y) the date that is three (3) months after the termination of your Continuous Service, and (B) the Expiration Date;

(c) twelve (12) months after the termination of your Continuous Service due to your Disability (except as otherwise provided in Section 9(d) below);

(d) eighteen (18) months after your death if you die either during your Continuous Service or within three (3) months after your Continuous Service terminates for any reason other than Cause;

(e) twenty-four (24) months after the termination of your Continuous Service due to your Retirement;

(f) unless this Option is not assumed, continued or replaced by the successor or acquiring entity, twelve (12) months after the termination of your Continuous Service, where such termination occurs either (i) as a condition of a Change in Control or (ii) upon the effectiveness of a Change in Control;

(g) the Expiration Date indicated in your Grant Notice; or

(h) the day before the tenth (10th) anniversary of the Date of Grant.

If the Option is an Incentive Stock Option, note that to obtain the federal income tax advantages associated with an Incentive Stock Option, the Code requires that at all times beginning on the Date of Grant and ending on the day three (3) months before the date of the Option's exercise, you must be an employee of the Company or an Affiliate, except in the event of your death or Disability. The Company has provided for extended exercisability of the Option under certain circumstances for your benefit but cannot guarantee that the Option will necessarily be treated as an Incentive Stock Option if you continue to provide services to the Company or an Affiliate as a Consultant or Director after your employment terminates or if you exercise the Option more than three (3) months after the date your employment with the Company or an Affiliate terminates.

10. EXERCISE.

(a) You may exercise the vested portion of the Option during its term by (i) delivering a Notice of Exercise (in a form designated by the Company), or making the required electronic election with the Company's designated broker, and (ii) paying the exercise price and any applicable

withholding taxes to the Company's stock plan administrator, or to such other person as the Company may designate, together with such additional documents as the Company may then require.

(b) By exercising the Option you agree that, as a condition to any exercise of the Option, the Company may require you to enter into an arrangement providing for the payment by you to the Company of any tax withholding obligation of the Company arising by reason of (i) the exercise of the Option, (ii) the lapse of any substantial risk of forfeiture to which the shares of Common Stock are subject at the time of exercise, or (iii) the disposition of shares of Common Stock acquired upon such exercise.

(c) If the Option is an Incentive Stock Option, by exercising the Option you agree that you will notify the Company in writing within fifteen (15) days after the date of any disposition of any of the shares of the Common Stock issued upon exercise of the Option that occurs within two (2) years after the Date of Grant or within one (1) year after such shares of Common Stock are transferred upon exercise of the Option.

11. TRANSFERABILITY. Except as otherwise provided in this Section 11, the Option is not transferable, except by will or by the laws of descent and distribution, and is exercisable during your life only by you.

(a) Certain Trusts. Upon receiving written permission from the Board or its duly authorized designee, you may transfer the Option to a trust if you are considered to be the sole beneficial owner (determined under Section 671 of the Code and applicable state law) while the Option is held in the trust. You and the trustee must enter into transfer and other agreements required by the Company.

(b) Domestic Relations Orders. Upon receiving written permission from the Board or its duly authorized designee, and provided that you and the designated transferee enter into transfer and other agreements required by the Company, you may transfer the Option pursuant to the terms of a domestic relations order, official marital settlement agreement or other

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divorce or separation instrument as permitted by Treasury Regulation 1.421-1(b)(2) that contains the information required by the Company to effectuate the transfer. You are encouraged to contact the Company's General Counsel regarding the proposed terms of any division of the Option prior to finalizing the domestic relations order or marital settlement agreement to help ensure the required information is contained within the domestic relations order or marital settlement agreement. If the Option is an Incentive Stock Option, the Option may be deemed to be a Nonstatutory Stock Option as a result of such transfer.

(c) Beneficiary Designation. Upon receiving written permission from the Board or its duly authorized designee, you may, by delivering written notice to the Company, in a form approved by the Company and any broker designated by the Company to handle option exercises, designate a third party who, on your death, will thereafter be entitled to exercise the Option and receive the Common Stock or other consideration resulting from such exercise. In the absence of such a designation, your executor or administrator of your estate will be entitled to exercise the Option and receive, on behalf of your estate, the Common Stock or other consideration resulting from such exercise.

12. OPTION NOT A SERVICE CONTRACT. The Option is not an employment or service contract, and nothing in the Option, the Grant Notice, this Option Agreement or the Plan will be deemed to create in any way whatsoever any obligation on your part to continue in the employ of the Company or an Affiliate, or of the Company or an Affiliate to continue your employment. In addition, nothing in the Option, the Grant Notice, this Option Agreement or the Plan will obligate the Company or an Affiliate, their respective stockholders, boards of directors, officers or employees to continue any relationship that you might have as a Director or Consultant for the Company or an Affiliate.

13. WITHHOLDING OBLIGATIONS.

(a) At the time you exercise the Option, in whole or in part, and at any time thereafter as the Company requests, you hereby authorize withholding from payroll and any other amounts payable to you, and otherwise agree to make adequate provision for (including by means of a "same day sale" pursuant to a program developed under Regulation T as promulgated by the Federal Reserve Board to the extent permitted by the Company), any sums required to satisfy the federal, state, local and foreign tax withholding obligations of the Company or any Affiliate that arise in connection with the exercise of the Option.

(b) If the Option is a Nonstatutory Stock Option, then upon your request and subject to approval by the Company (or the Board, if necessary for compliance with applicable laws) and compliance with any applicable legal conditions or restrictions, the Company may withhold from fully vested shares of Common Stock otherwise issuable to you upon the exercise of the Option a number of whole shares of Common Stock having a Fair Market Value, determined by the Company as of the date of exercise, not in excess of the minimum amount of tax required to be withheld by law (or such lower amount as may be necessary to avoid classification of your option as a liability for financial accounting purposes).

(c) You may not exercise the Option unless the tax withholding obligations of the Company and any Affiliate are satisfied. Accordingly, you may not be able to exercise the

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Option when desired even though the Option is vested, and the Company will have no obligation to issue a certificate for shares of Common Stock unless such obligations are satisfied.

14. TAX CONSEQUENCES. You hereby agree that the Company does not have a duty to design or administer the Plan or its other compensation programs in a manner that minimizes your tax liabilities. You will not make any claim against the Company, or any of its Officers, Directors, Employees or Affiliates related to tax liabilities arising from the Option or your other compensation. In particular, you acknowledge that the Option is exempt from Section 409A of the Code only if the exercise price per share specified in the Grant Notice is at least equal to the "fair market value" per share of the Common Stock on the Date of Grant and there is no other impermissible deferral of compensation associated with the option.

15. NOTICES. Any notices provided for in the Option, this Option Agreement, the Grant Notice or the Plan will be given in writing (including electronically) and will be deemed effectively given upon receipt or, in the case of notices delivered by mail by the Company to you, five (5) days after deposit in the U.S. mail, postage prepaid, addressed to you at the last address you provided to the Company. The Company may, in its sole discretion, decide to deliver any documents related to participation in the Plan and the Option by electronic means or to request your consent to participate in the Plan by

electronic means. By accepting the Option, you consent to receive such documents by electronic delivery and to participate in the Plan through an on-line or electronic system established and maintained by the Company or another third party designated by the Company.

16. GOVERNING PLAN DOCUMENT. The Option is subject to all the provisions of the Plan, the provisions of which are hereby made a part of the Option, and is further subject to all interpretations, amendments, rules and regulations, which may from time to time be promulgated and adopted pursuant to the Plan. If there is any conflict between the provisions of the Option and those of the Plan, the provisions of the Plan will control. In addition, the Option (and any compensation paid or shares issued under the Option) is subject to recoupment in accordance with The Dodd–Frank Wall Street Reform and Consumer Protection Act and any implementing regulations thereunder, any clawback policy adopted by the Company and any compensation recovery policy otherwise required by applicable law. No recovery of compensation under such a clawback policy will be an event giving rise to a right to resign for "good reason" or for a "constructive termination" (or similar term) under any agreement with the Company.

17. OTHER DOCUMENTS. You hereby acknowledge receipt of and the right to receive a document providing the information required by Rule 428(b)(1) promulgated under the Securities Act, which includes the Plan prospectus. In addition, you acknowledge receipt of the Company's policy permitting certain individuals to sell shares only during certain "window" periods and the Company's insider trading policy, in effect from time to time.

18. EFFECT ON OTHER EMPLOYEE BENEFIT PLANS. The value of the Option will not be included as compensation, earnings, salaries, or other similar terms used when calculating your benefits under any employee benefit plan sponsored by the Company or any Affiliate, except as such plan otherwise expressly provides. The Company expressly reserves its rights to amend, modify, or terminate any of the Company's or any Affiliate's employee benefit plans.

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19. VOTING RIGHTS. You will not have voting or any other rights as a stockholder of the Company with respect to the shares to be issued pursuant to the Option until such shares are issued to you. Upon such issuance, you will obtain full voting and other rights as a stockholder of the Company. Nothing contained in the Option, and no action taken pursuant to its provisions, will create or be construed to create a trust of any kind or a fiduciary relationship between you and the Company or any other person.

20. SEVERABILITY. If all or any part of this Option Agreement or the Plan is declared by any court or governmental authority to be unlawful or invalid, such unlawfulness or invalidity will not invalidate any portion of this Option Agreement or the Plan not declared to be unlawful or invalid. Any Section of this Option Agreement (or part of such a Section) so declared to be unlawful or invalid shall, if possible, be construed in a manner which will give effect to the terms of such Section or part of a Section to the fullest extent possible while remaining lawful and valid.

21. MISCELLANEOUS.

(a) The rights and obligations of the Company under the Option will be transferable to any one or more persons or entities, and all covenants and agreements hereunder will inure to the benefit of, and be enforceable by the Company's successors and assigns.

(b) You agree upon request to execute any further documents or instruments necessary or desirable in the sole determination of the Company to carry out the purposes or intent of the Option.

(c) You acknowledge and agree that you have reviewed the Option and this Option Agreement in its entirety, have had an opportunity to obtain the advice of counsel prior to executing and accepting the Option, and fully understand all provisions of the Option.

(d) This Option Agreement will be subject to all applicable laws, rules, and regulations, and to such approvals by any governmental agencies or national securities exchanges as may be required.

(e) All obligations of the Company under the Plan and this Option Agreement will be binding on any successor to the Company, whether the existence of such successor is the result of a direct or indirect purchase, merger, consolidation, or otherwise, of all or substantially all of the business and/or assets of the Company.

* * *

This Option Agreement will be deemed to be signed by you upon the signing by you of the Grant Notice to which it is attached.

GTx, INC.
STOCK OPTION GRANT NOTICE
(2013 NON-EMPLOYEE DIRECTOR EQUITY INCENTIVE PLAN)

GTx, Inc. (the "***Company***"), pursuant to its 2013 Non-Employee Director Equity Incentive Plan (the "***Plan***"), hereby grants to Optionholder an option (the "***Option***") to purchase the number of shares of the Company's Common Stock set forth below. The Option is subject to all of the terms and conditions as set forth in this notice (the "***Grant Notice***"), in the Option Agreement and in the Plan, both of which are incorporated herein in their entirety. For your convenience, a copy of the Option Agreement is attached hereto. A copy of the Plan is available from the Company on request. Capitalized terms not explicitly defined herein but defined in the Plan or the Option Agreement will have the same definitions as in the Plan or the Option Agreement. If there is any conflict between the terms in the Option Agreement and the Plan, the terms of the Plan will control.

Optionholder:
Date of Grant:
Vesting Commencement Date:
Number of Shares Subject to Option:
Exercise Price (Per Share):
Total Exercise Price:
Expiration Date:

Type of Grant: Nonstatutory Stock Option

Exercise Schedule: Same as Vesting Schedule

Vesting Schedule: Subject to any accelerated vesting pursuant to the terms of the Option Agreement or as otherwise specifically determined by the Board, the Option vests with respect to one-third (1/3rd) of the total number of shares of Common Stock subject to the Option (rounded down to the nearest whole share) on each of the first, second and third anniversaries of the Vesting Commencement Date, subject to Optionholder's Continuous Service on each applicable vesting date.

Payment: By one or a combination of the following items:

- o By cash (as described in the Plan)
- o Pursuant to a Regulation T Program, if the Common Stock is publicly traded
- o By delivery of already-owned shares, if the Common Stock is publicly traded
- o Subject to the Company's consent at the time of exercise, by a "net exercise" arrangement

Additional Terms/Acknowledgements: Optionholder acknowledges receipt of, and understands and agrees to, this Grant Notice, the Option Agreement, the Plan and the stock plan prospectus for the Plan. As of the Date of Grant, this Grant Notice, the Option Agreement, and the Plan set forth the entire understanding between Optionholder and the Company regarding the Option and supersede all prior oral and written agreements with respect to the Option, with the exception, if applicable, of any compensation recovery policy that is adopted by the Company or is otherwise required by applicable law. By accepting the Option, Optionholder consents to receive documents governing the Option by electronic delivery and to participate in the Plan through an on-line or electronic system established and maintained by the Company or another third party designated by the Company.

* * *

GTx, INC.

By: _____
Signature

Title: _____

Date: _____

OPTIONHOLDER:

_____
Signature

Date: _____

ATTACHMENT: Option Agreement

ATTACHMENT I

GTx, INC.
2013 NON-EMPLOYEE DIRECTOR EQUITY INCENTIVE PLAN

OPTION AGREEMENT
(NONSTATUTORY STOCK OPTION)

Pursuant to your Stock Option Grant Notice (the "***Grant Notice***") and this Option Agreement (this "***Option Agreement***"), GTx Inc. (the "***Company***") has granted you an option (the "***Option***") under its 2013 Non-Employee Director Equity Incentive Plan (the "***Plan***") to purchase the number of shares of the Company's Common Stock indicated in the Grant Notice at the exercise price indicated in the Grant Notice. The Option is granted to you effective as of the

date of grant set forth in the Grant Notice (the "***Date of Grant***"). If there is any conflict between the terms in this Option Agreement and the Plan, the terms of the Plan will control. Defined terms not explicitly defined in this Option Agreement or in the Grant Notice, but defined in the Plan, will have the same definitions as in the Plan.

The details of the Option, in addition to those set forth in the Grant Notice and the Plan, are as follows:

1. VESTING. The Option will vest and become exercisable as provided in the Grant Notice and the Plan. Unless specifically provided to the contrary by the Board, vesting will cease, in all events, upon the termination of your Continuous Service.

2. NUMBER OF SHARES AND EXERCISE PRICE. The number of shares of Common Stock subject to the Option and the exercise price per share in the Grant Notice will be adjusted for Capitalization Adjustments as provided in the Plan.

3. METHOD OF PAYMENT. You must pay the full amount of the exercise price for the shares of Common Stock subject to the Option that you wish to exercise. You may pay the exercise price in cash (as described in the Plan) or in any other manner ***permitted by the Grant Notice,*** which may include one or more of the following:

(a) Provided that at the time of exercise the Common Stock is publicly traded, pursuant to a program developed under Regulation T as promulgated by the Federal Reserve Board that, prior to the issuance of Common Stock, results in either the receipt of cash (or check) by the Company or the receipt of irrevocable instructions to pay the aggregate exercise price to the Company from the sales proceeds. This manner of payment is also known as a "broker-assisted exercise," "same day sale," or "sell to cover."

(b) Provided that at the time of exercise the Common Stock is publicly traded, by delivery to the Company (either by actual delivery or attestation) of already-owned shares of Common Stock that are owned free and clear of any liens, claims, encumbrances or security interests, and that are valued at Fair Market Value on the date of exercise. "***Delivery***" for these purposes, in the sole discretion of the Company at the time you exercise the Option (or any

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vested portion thereof), will include delivery to the Company of your attestation of ownership of such shares of Common Stock in a form approved by the Company. You may not exercise the Option (or any exercisable portion thereof) by delivery to the Company of Common Stock if doing so would violate the provisions of any law, regulation or agreement restricting the redemption of the Company's stock.

(c) Subject to the consent of the Board prior to the time of exercise, by a "net exercise" arrangement pursuant to which the Company will reduce the number of shares of Common Stock issued upon exercise of the Option (or any vested portion thereof) by the largest whole number of shares with a Fair Market Value that does not exceed the aggregate exercise price. You must pay any remaining balance of the aggregate exercise price not satisfied by the "net exercise" in cash or other permitted form of payment. Shares of Common Stock will no longer be outstanding under the Option and will not be exercisable thereafter if those shares (i) are used to pay the exercise price pursuant to the "net exercise," or (ii) are delivered to you as a result of such exercise.

4. WHOLE SHARES. You may exercise the Option (or any vested portion thereof) only for whole shares of Common Stock.

5. SECURITIES LAW COMPLIANCE. In no event may you exercise the Option (or any vested portion thereof) unless the shares of Common Stock issuable upon exercise are then registered under the Securities Act or, if not registered, the Company has determined that your exercise and the issuance of the shares would be exempt from the registration requirements of the Securities Act. The exercise of the Option (or any vested portion thereof) also must comply with all other applicable laws and regulations governing the Option, and you may not exercise the Option (or any vested portion thereof) if the Company determines that such exercise would not be in material compliance with such laws and regulations.

6. TERM. You may not exercise the Option before the Date of Grant or after the expiration of the term of the Option. The term of the Option expires, subject to the provisions of Section 5(h) of the Plan, upon the earliest of the following:

(a) immediately upon the date on which the event giving rise to the termination for Cause first occurred (or, if required by law, the date of the termination of your Continuous Service for Cause);

(b) three (3) months after the termination of your Continuous Service for any reason other than for Cause, your Disability or your death (except as otherwise provided in this Option Agreement); *provided, however,* that if during any part of such three (3) month period the Option is not exercisable solely because doing so would violate the registration requirements under the Securities Act, the Option will not expire until the earlier of the Expiration Date or until it has been exercisable for an aggregate period of three (3) months after the termination of your Continuous Service;

(c) twelve (12) months after the termination of your Continuous Service due to your Disability (except as otherwise provided in this Option Agreement);

(d) eighteen (18) months after your death if you die either during your Continuous Service or within three (3) months after your Continuous Service terminates for any reason other than Cause;

(e) unless this Option is not assumed, continued or replaced by the successor or acquiring entity, twelve (12) months after the termination of your Continuous Service, where such termination occurs either (i) as a condition of a Change in Control or (ii) upon the effectiveness of a Change in Control;

(f) the Expiration Date indicated in the Grant Notice; or

(g) the day before the tenth (10th) anniversary of the Date of Grant.

7. EXERCISE.

(a) You may exercise the vested portion of the Option during its term by (i) delivering a Notice of Exercise (in a form designated by the Company), or making the required electronic election with the Company's designated broker, (ii) paying the exercise price to the Company's stock plan administrator or to such other person as the Company may designate, and (iii) paying any applicable withholding taxes, and (iv) delivering such additional documents as the Company may then require.

8. TRANSFERABILITY. Except as otherwise provided in this Section 8, the Option is not transferable, except by will or by the laws of descent and distribution, and is exercisable during your life only by you.

(a) Certain Trusts. Upon receiving written permission from the Board or its duly authorized designee, you may transfer the Option to a trust if you are considered to be the sole beneficial owner (determined under Section 671 of the Code and applicable state law) while the Option is held in the trust. You and the trustee must enter into transfer and other agreements required by the Company.

(b) Domestic Relations Orders. Upon receiving written permission from the Board or its duly authorized designee, and provided that you and the designated transferee enter into transfer and other agreements required by the Company, you may transfer the Option pursuant to the terms of a domestic relations order, official marital settlement agreement or other divorce or separation instrument that contains the information required by the Company to effectuate the transfer. You are encouraged to contact the Company's General Counsel regarding the proposed terms of any division of the Option prior to finalizing the domestic relations order or marital settlement agreement to help ensure the required information is contained within the domestic relations order or marital settlement agreement.

(c) Beneficiary Designation. Upon receiving written permission from the Board or its duly authorized designee, you may, by delivering written notice to the Company, in a form approved by the Company and any broker designated by the Company to handle option exercises, designate a third party who, on your death, will thereafter be entitled to exercise the Option and receive the Common Stock or other consideration resulting from such exercise. In the absence of such a designation, your executor or administrator of your estate will be entitled

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to exercise the Option and receive, on behalf of your estate, the Common Stock or other consideration resulting from such exercise.

9. OPTION NOT A SERVICE CONTRACT. The Option is not an employment or service contract, and nothing in the Option, the Grant Notice, this Option Agreement or the Plan will be deemed to create in any way whatsoever any obligation on your part to continue providing services to the Company or an Affiliate, or of the Company or an Affiliate to continue your services. In addition, nothing in the Option, the Grant Notice, this Option Agreement or the Plan will obligate the Company or an Affiliate, their respective stockholders, boards of directors, officers or employees to continue any relationship that you might have as a Director or Consultant for the Company or an Affiliate.

10. WITHHOLDING OBLIGATIONS.

(a) Regardless of any action the Company takes with respect to any or all ordinary income tax, payroll tax or other tax-related withholding due in connection with the Option, you acknowledge that the ultimate liability for all such taxes and/or other tax-related withholding is and remains your responsibility. The Company makes no representations or undertakings regarding the treatment of any such taxes or tax-related withholding in connection with any aspect of the Option, including the grant, vesting or exercise of the Option, or the subsequent sale of shares of Common Stock acquired upon exercise of the Option.

(b) You may not exercise the Option unless the applicable tax withholding obligations of the Company and any Affiliate are satisfied. Accordingly, you may not be able to exercise the Option when desired even though the Option is vested, and the Company will have no obligation to issue a certificate for shares of Common Stock unless such obligations are satisfied.

11. TAX CONSEQUENCES. You hereby agree that the Company does not have a duty to design or administer the Plan or its other compensation programs in a manner that minimizes your tax liabilities. You will not make any claim against the Company, or any of its Officers, Directors, Employees or Affiliates related to tax liabilities arising from the Option or your other compensation. In particular, you acknowledge that the Option is exempt from Section 409A of the Code only if the exercise price per share specified in the Grant Notice is at least equal to the "fair market value" per share of the Common Stock on the Date of Grant and there is no other impermissible deferral of compensation associated with the option.

12. NOTICES. Any notices provided for in the Option, this Option Agreement, the Grant Notice or the Plan will be given in writing (including electronically) and will be deemed effectively given upon receipt or, in the case of notices delivered by mail by the Company to you, five (5) days after deposit in the U.S. mail, postage prepaid, addressed to you at the last address you provided to the Company. The Company may, in its sole discretion, decide to deliver any documents related to participation in the Plan and the Option by electronic means or to request your consent to participate in the Plan by electronic means. By accepting the Option, you consent to receive such documents by electronic delivery and to participate in the Plan through an on-line or electronic system established and maintained by the Company or another third party designated by the Company.

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13. GOVERNING PLAN DOCUMENT. The Option is subject to all the provisions of the Plan, the provisions of which are hereby made a part of the Option, and is further subject to all interpretations, amendments, rules and regulations, which may from time to time be promulgated and adopted pursuant to the Plan. If there is any conflict between the provisions of the Option and those of the Plan, the provisions of the Plan will control. In addition, the Option (and any compensation paid or shares issued under the Option) is subject to recoupment in accordance with any clawback policy adopted by the Company and any compensation recovery policy otherwise required by applicable law.

14. OTHER DOCUMENTS. You hereby acknowledge receipt of and the right to receive a document providing the information required by Rule 428(b)(1) promulgated under the Securities Act, which includes the Plan prospectus. In addition, you acknowledge receipt of the Company's policy permitting certain individuals to sell shares only during certain "window" periods and the Company's insider trading policy, in effect from time to time.

15. EFFECT ON OTHER EMPLOYEE BENEFIT PLANS. The value of the Option will not be included as compensation, earnings, salaries, or other similar terms used when calculating your benefits under any employee benefit plan sponsored by the Company or any Affiliate, except as such plan otherwise expressly provides. The Company expressly reserves its rights to amend, modify, or terminate any of the Company's or any Affiliate's employee benefit plans.

16. VOTING RIGHTS. You will not have voting or any other rights as a stockholder of the Company with respect to the shares to be issued pursuant to the Option until such shares are issued to you. Upon such issuance, you will obtain full voting and other rights as a stockholder of the Company. Nothing contained in the Option, and no action taken pursuant to its provisions, will create or be construed to create a trust of any kind or a fiduciary relationship between you and the Company or any other person.

17. SEVERABILITY. If all or any part of this Option Agreement or the Plan is declared by any court or governmental authority to be unlawful or invalid, such unlawfulness or invalidity will not invalidate any portion of this Option Agreement or the Plan not declared to be unlawful or invalid. Any Section of this Option Agreement (or part of such a Section) so declared to be unlawful or invalid shall, if possible, be construed in a manner which will give effect to the terms of such Section or part of a Section to the fullest extent possible while remaining lawful and valid.

18. MISCELLANEOUS.

(a) The rights and obligations of the Company under the Option will be transferable to any one or more persons or entities, and all covenants and agreements hereunder will inure to the benefit of, and be enforceable by the Company's successors and assigns.

(b) You agree upon request to execute any further documents or instruments necessary or desirable in the sole determination of the Company to carry out the purposes or intent of the Option.

(c) You acknowledge and agree that you have reviewed the Option and this Option Agreement in its entirety, have had an opportunity to obtain the advice of counsel prior to executing and accepting the Option, and fully understand all provisions of the Option.

(d) This Option Agreement will be subject to all applicable laws, rules, and regulations, and to such approvals by any governmental agencies or national securities exchanges as may be required.

(e) All obligations of the Company under the Plan and this Option Agreement will be binding on any successor to the Company, whether the existence of such successor is the result of a direct or indirect purchase, merger, consolidation, or otherwise, of all or substantially all of the business and/or assets of the Company.

* * *

This Option Agreement will be deemed to be signed by you upon the signing by you of the Grant Notice to which it is attached.

Second Memorandum of Understanding
Concerning the Lease Agreement between
The University of Tennessee Research Foundation and GTx, Inc.
As Amended July 20, 2009

RECITALS

WHEREAS, The University of Tennessee Research Foundation (the "SUBLESSOR") and The University of Tennessee (the "LESSOR") have entered into a Lease dated March 7, 2001, which was subsequently amended by agreements dated April 1, 2005 and July 20, 2009 (the "Lease"); and

WHEREAS, the SUBLESSOR and GTx, Inc., a Delaware corporation (the "SUBLESSEE"), have entered into a Sublease dated October 1, 2009 with the consent of LESSOR (the "Sublease");

WHEREAS, the SUBLESSOR and SUBLESSEE desired to reduce the portion of the premises leased to the SUBLESSEE and the Rent to be paid by SUBLESSEE to SUBLESSOR and subsequently signed a Memorandum of Understanding on April 19, 2011 effective May 1, 2011;

WHEREAS, the SUBLESSOR and SUBLESSEE desire to continue the arrangement for reduced premises and Rent for an additional five months;

NOW THEREFORE, in consideration of the foregoing and other good and valuable consideration set forth herein, the parties agree as follows:

1. This MOU is to become effective on May 1, 2013 (the "Effective Date") and will continue in effect through September 30, 2013.

2. This MOU can be canceled with 90 days advance written notice and acceptance by all parties, in which event the lease premises will automatically return to the entire leased premises described in the Sublease.

3. All parties agree to respect the privacy and confidentiality of intellectual property of each other as provided by Tennessee and federal law.

4. All other terms and conditions of the 2011 MOU are to remain in effect.

IN WITNESS WHEREOF, the LESSOR, SUBLESSOR and the SUBLESSEE have executed this Second Memorandum of Understanding in duplicate on the date written below.

THE UNIVERSITY OF TENNESSEE RESEARCH FOUNDATION (SUBLESSOR)

By: /s/ Richard Magid
Richard Magid
Vice President

Date: April 19, 2013

GTx, Inc. (SUBLESSEE)

By: /s/ Henry P. Doggrell
Henry P. Doggrell
Vice President, Chief Legal Officer

Date: April 19, 2013

THE UNIVERSITY OF TENNESSEE (LESSOR)

By: /s/ Charles M. Peccolo
Charles M. Peccolo
Treasurer

Date: April 25, 2013

EXHIBIT 31.1

CHIEF EXECUTIVE OFFICER CERTIFICATION

I, Mitchell S. Steiner, 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: July 22, 2013

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

EXHIBIT 31.2

CHIEF FINANCIAL OFFICER CERTIFICATION

I, Mark E. Mosteller, certify that:

1. I have reviewed this 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: July 22, 2013

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

EXHIBIT 32.1

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

In connection with the Quarterly Report of GTx, Inc. (the "Company") on Form 10-Q for the quarterly period ended June 30, 2013, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I, Mitchell S. Steiner, Chief Executive Officer of the Company certify, pursuant to Rule 13a-14(b) or Rule 15d-14(b) and 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that, 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: July 22, 2013

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

This certification accompanies the Form 10-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.

EXHIBIT 32.2

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

In connection with the Quarterly Report of GTx, Inc. (the "Company") on Form 10-Q for the quarterly period ended June 30, 2013, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I, Mark E. Mosteller, Chief Financial Officer of the Company certify, pursuant to 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: July 22, 2013

/s/ Mark E. Mosteller
Mark E. Mosteller, CPA
Vice President and Chief 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.