GTX INC /DE/
Form 10-Q
November 12, 2013
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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 September 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 (Do not check if a smaller reporting company)	Smaller reporting company o

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

As of November 7, 2013, 63,185,389 shares of the registrant s Common Stock were outstanding.

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GTx, INC.

FORM 10-Q FOR THE QUARTER ENDED SEPTEMBER 30, 2013

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PART I: FINANCIAL INFORMATION

ITEM 1. FINANCIAL STATEMENTS

GTx, Inc.
CONDENSED BALANCE SHEETS

(in thousands, except share data)

	September 30, 2013 (unaudited)	December 31, 2012
ASSETS		
Current assets:		
Cash and cash equivalents	$ 19,283	$ 48,044
Short-term investments	1,675	8,045
Prepaid expenses and other current assets	830	726
Total current assets	21,788	56,815
Property and equipment, net	217	507
Intangible and other assets, net	487	452
Total assets	$ 22,492	$ 57,774
LIABILITIES AND STOCKHOLDERS EQUITY		
Current liabilities:		
Accounts payable	$ 1,253	$ 1,707
Accrued expenses and other current liabilities	3,912	7,788
Total current liabilities	5,165	9,495
Other long-term liabilities	389	578
Commitments and contingencies		
Stockholders equity:		
Common stock, $0.001 par value: 120,000,000 shares authorized at both September 30, 2013 and December 31, 2012; 63,185,389 and 62,818,424 shares issued and outstanding at September 30, 2013 and December 31, 2012, respectively	63	63
Additional paid-in capital	464,445	460,887
Accumulated deficit	(447,570)	(413,249)
Total stockholders equity	16,938	47,701
Total liabilities and stockholders equity	$ 22,492	$ 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 September 30,		Nine Months Ended September 30,	
	2013	**2012**	**2013**	**2012**
Expenses:				
Research and development expenses	$ 6,477	$ 9,764	$ 26,230	$ 28,836
General and administrative expenses	2,483	2,999	8,190	7,987
Total expenses	8,960	12,763	34,420	36,823
Loss from operations	(8,960)	(12,763)	(34,420)	(36,823)
Other income, net	23	(47)	99	14
Loss from operations before income taxes	(8,937)	(12,810)	(34,321)	(36,809)
Income tax benefit		5,812		6,548
Net loss from continuing operations	(8,937)	(6,998)	(34,321)	(30,261)
Income from discontinued operations before income taxes		20,214		22,752
Income tax expense		(8,115)		(8,851)
Net income from discontinued operations		12,099		13,901
Net income (loss)	$ (8,937)	$ 5,101	$ (34,321)	$ (16,360)
Net income (loss) per share - basic and diluted:				
Net loss from continuing operations	$ (0.14)	$ (0.11)	$ (0.54)	$ (0.48)
Net income from discontinued operations		0.19		0.22
Net income (loss) per share	$ (0.14)	$ 0.08	$ (0.54)	$ (0.26)
Weighted average shares outstanding:				
Basic and diluted	63,179,394	62,815,549	63,013,923	62,806,440

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)

	Nine Months Ended September 30,	
	2013	**2012**
Cash flows from operating activities:		
Net loss	$ (34,321)	$ (16,360)
Adjustments to reconcile net loss to net cash used in operating activities:		
Gain on sale of FARESTON®		(18,831)
Depreciation and amortization	333	596
Share-based compensation	2,227	1,878
Directors deferred compensation	105	128
Changes in assets and liabilities:		
Prepaid expenses and other assets	(150)	106
Accounts payable	(454)	(12)
Accrued expenses and other liabilities	(4,060)	5,451
Net cash used in operating activities	(36,320)	(27,044)
Cash flows from investing activities:		
Purchase of property and equipment	(32)	(125)
Purchase of short-term investments, held to maturity	(1,225)	(7,815)
Proceeds from maturities of short-term investments, held to maturity	7,595	9,955
Net cash provided by investing activities	6,338	2,015
Cash flows from financing activities:		
Payments on capital lease and financed equipment obligations	(5)	(67)
Proceeds from exercise of employee stock options	1,226	82
Net cash provided by financing activities	1,221	15
Net decrease in cash and cash equivalents	(28,761)	(25,014)
Cash and cash equivalents, beginning of period	48,044	63,745
Cash and cash equivalents, end of period	$ 19,283	$ 38,731

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

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GTx, Inc.
NOTES TO THE CONDENSED FINANCIAL STATEMENTS

(in thousands, except share and per share data)

(unaudited)

1. Business and Basis of Presentation

Business

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

The Company is developing selective androgen receptor modulators (SARMs), including its lead product candidate, enobosarm (GTx-024). SARMs are a new class of drugs with the potential to prevent and treat muscle wasting in patients with cancer and other musculoskeletal wasting or muscle loss conditions, including chronic sarcopenia (age related muscle loss), as well as the potential to be used as a hormonal therapy for the treatment of advanced breast cancer. The Company announced in August 2013 that its two Phase 3 clinical trials evaluating enobosarm 3 mg for the prevention and treatment of muscle wasting in patients with advanced non-small cell lung cancer (NSCLC) failed to meet the co-primary endpoints of lean body mass and physical function that were assessed statistically using responder analyses. The Company plans to meet with the United States Food and Drug Administration and representatives of certain member countries of the European Medicines Agency to review and discuss the results of the clinical trials and a feasible regulatory pathway forward to seek marketing approval for enobosarm 3 mg for the prevention and treatment of muscle wasting in patients with advanced NSCLC.

The Company is also conducting a Phase 2 open label study evaluating enobosarm 9 mg for the treatment of androgen receptor positive and estrogen receptor positive metastatic breast cancer in women who have previously responded to hormonal therapy for the treatment of their 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 secondary hormonal treatment for advanced prostate cancer used in combination with androgen deprivation therapy. The Company is presently conducting a Phase 2 clinical trial evaluating GTx-758 as secondary hormonal therapy in men with metastatic castration resistant prostate cancer.

The Company has experienced significant recurring operating losses since its inception and has limited funds. The failure of both enobosarm 3 mg Phase 3 clinical trials to meet each of the co-primary endpoints has significantly depressed the Company s stock price and has severely harmed the Company s ability to raise additional capital, and, consequently, its prospects as a going concern have been diminished. The Company has implemented a plan to reduce its operating expenses, including significantly reducing its workforce as announced in October 2013, in order to preserve capital while it evaluates feasible regulatory pathways for enobosarm 3 mg, conducts its two Phase 2 clinical trials of enobosarm 9 mg and GTx-758, and pursues discussions with potential partners. However, the expense reduction plan alone will not be sufficient to allow the Company to continue its operations for the next twelve months without raising additional funds.

Subsequent Events

The Company has evaluated all events or transactions that occurred after September 30, 2013 up through the date the condensed financial statements were issued.

In October 2013, the Company announced and implemented a reduction in its workforce following the announced results from its two Phase 3 clinical trials evaluating enobosarm 3 mg for the prevention and treatment of muscle wasting in patients with advanced NSCLC. The reduction in force was effective immediately and represented approximately 60% of the Company s total workforce. As a result of the workforce reduction, the Company estimates that it will record in the fourth quarter of 2013 compensation expense of approximately $1,300 related to cash severance expenses. Additionally, the Company expects to recognize a benefit of

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

(unaudited)

approximately $370 resulting from the reversal of share-based compensation expense related to the amendment of certain stock option provisions for the severed employees.

There were no other material recognizable or nonrecognizable subsequent events during the period evaluated.

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 nine months ended September 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.

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 September 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.

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

(unaudited)

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 September 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 nine months ended September 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 nine months ended September 30, 2012. See Note 4, *Discontinued Operations*, for further discussion.

FARESTON® Revenue Recognition

Revenue from product sales of FARESTON® for the three and nine months ended September 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 September 30, 2013 and December 31, 2012, the Company s accrual for product returns, was $995 and $1,189, respectively. Of these amounts, $332 and $370 have been included in Other long-term liabilities in the condensed balance sheet at September 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.

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Reclassification

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

Going Concern

The accompanying unaudited condensed financial statements have been prepared assuming the Company will continue as a going concern which contemplates the realization of assets and liabilities in the ordinary course of business. The Company has experienced significant recurring operating losses since its inception resulting in an accumulated deficit of $447,570 at September 30, 2013. At September 30, 2013, the Company had cash, cash equivalents and short-term investments of $20,958 compared to $56,089 at December 31, 2012. Currently, the Company has no ongoing collaborations for the development and commercialization of its product candidates and no source of revenue, nor does the Company expect to generate revenue for the foreseeable future. A substantial portion of the Company s efforts and expenditures have been devoted to enobosarm 3 mg, which was the subject of two Phase 3 clinical trials for the prevention and treatment of muscle wasting in patients with advanced NSCLC, and the Company has been substantially dependent on the successful development, regulatory approval and commercialization of enobosarm 3 mg. The failure of both enobosarm 3 mg Phase 3 clinical trials to meet each of the co-primary endpoints has significantly depressed the Company s stock price and has severely harmed its ability to raise additional capital, and consequently, the Company s prospects as a going concern have been diminished. As a result, the Company announced and implemented a plan on October 1, 2013 to reduce its operating expenses, including significantly reducing its workforce, in order to preserve capital. The Company estimates that its current cash and investments will not be sufficient to fund its ongoing operations for the next twelve months. If the Company does not have sufficient funds to continue its operations, it would be required to, among other things, make further reductions in its workforce, eliminate one or both of its ongoing clinical trials, discontinue the development of enobosarm and/or GTx-758, liquidate all or a portion of its assets, and/or seek protection under the provisions of the U.S. Bankruptcy Code. These factors raise substantial doubt about the Company s ability to continue as a going concern. The Company s financial statements do not include any adjustments that may result from the outcome of this uncertainty.

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 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

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(unaudited)

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 nine months ended September 30, 2013 and 2012:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2013	2012	2013	2012
Research and development expenses	$ 356	$ 335	$ 1,051	$ 696
General and administrative expenses	406	458	1,281	1,310
Total share-based compensation	$ 762	$ 793	$ 2,332	$ 2,006

Share-based compensation expense recorded as general and administrative expense for the three months ended September 30, 2013 and 2012 included share-based compensation expense related to deferred compensation arrangements for the Company s non-employee directors of $31 and $40, respectively. Share-based compensation expense recorded as general and administrative expense for the nine months ended September 30, 2013 and 2012 included share-based compensation expense related to deferred compensation arrangements for the Company s non-employee directors of $105 and $128, respectively. Share-based compensation expense recorded as research and development expense for the three and nine months ended September 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 nine months ended September 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.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2013	2012	2013	2012
Expected price volatility	74.8%	73.8%	74.5%	69.6%
Risk-free interest rate	1.8%	1.1%	1.1%	1.2%
Weighted average expected life in years	6.5 years	6.5 years	6.5 years	6.5 years

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GTx, Inc.
NOTES TO THE CONDENSED FINANCIAL STATEMENTS

(in thousands, except share and per share data)

(unaudited)

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	1,459,200	4.24
Options forfeited or expired	(531,076)	8.69
Options exercised	(321,298)	3.81
Options outstanding at September 30, 2013	5,989,685	7.21

3. Basic and Diluted Net Income (Loss) Per Share

Basic and diluted net income (loss) per share attributable to common stockholders is calculated based on the weighted average number of common shares outstanding during the period. Diluted net income (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 6,136,483 and 5,408,065 for the three months ended September 30, 2013 and 2012, respectively, and 6,432,994 and 5,639,672 for the nine months ended September 30, 2013 and 2012, respectively, were excluded from the calculations of diluted income (loss) per share as inclusion of the options would have had an anti-dilutive effect on the net income (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 $20,214 and $22,752 for the three and nine months ended September 30, 2012, respectively, was reported as income from discontinued operations in the condensed statement of operations and both periods included the gain of $18,831 recognized on the sale of FARESTON®. For the three months ended September 30, 2012, income from discontinued operations consisted of net product sales of $1,826 reduced by cost of product sales of $263 and FARESTON® operating expenses of $180. For the nine months ended

September 30, 2012, income from discontinued operations consisted of net product sales of $5,294 reduced by cost of product sales of $782 and FARESTON® operating expenses of $591. The Company remains liable for product returns related to sales of FARESTON® made by the Company prior to September 30, 2012. At September 30, 2013 and December 31, 2012, the Company s accrual for product returns, was $995 and $1,189, respectively.

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:

- our ability to preserve or realize any value from our enobosarm (GTx-024) and GTx-758 (Capesaris®) programs;

- the timing of regulatory discussions and submissions, and the timing, scope and anticipated outcome of related regulatory actions or guidance, including with respect to planned discussions with the United States Food and Drug Administration, or FDA, and representatives of certain member countries of the European Medicines Agency regarding feasible regulatory pathways forward for the development and commercialization of enobosarm 3 mg;

- our ability to establish and maintain potential new collaborative, partnering or other strategic arrangements for the development and commercialization of our product candidates;

- the anticipated progress of our clinical programs, including whether our ongoing clinical trials will achieve clinically relevant results;

- the timing, scope and anticipated initiation, enrollment and completion of our ongoing clinical trials and any future clinical trials that we may conduct;

- our ability to obtain and maintain regulatory approvals of our product candidates and any related restrictions, limitations, and/or warnings in the label of an approved product candidate;

- our ability to market, commercialize and achieve market acceptance for our product candidates;

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

- our estimates regarding the sufficiency of our cash resources and our ability to continue as a going concern and our expenses, capital requirements and need 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, ma potential, predicts, projects, should, will, would and similar expressions intended to identify forward-looking statements. Forward-lookin 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

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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.

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, including prevention and treatment of cancer-related muscle wasting, and other serious medical conditions.

Business Highlights

We are developing selective androgen receptor modulators, or SARMs, a new class of drugs with the potential to prevent and treat muscle wasting in patients with cancer and other musculoskeletal wasting or muscle loss conditions, including chronic sarcopenia (age related muscle loss), as well as the potential to be used as a hormonal therapy for the treatment of advanced breast cancer. Our lead SARM product candidate, enobosarm (GTx-024), has to date been evaluated in ten completed clinical trials enrolling approximately 1,250 subjects, including in a Phase 1b, two Phase 2 efficacy studies and two Phase 3 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 announced in August 2013 that the POWER 1 and POWER 2 (Prevention and treatment Of muscle Wasting in patients with cancER) Phase 3 clinical trials evaluating enobosarm 3 mg for the prevention and treatment of muscle wasting in patients with advanced non-small cell lung cancer, or NSCLC, failed to meet the co-primary endpoints of lean body mass and physical function that were assessed statistically using responder analyses. However, data from the studies have shown enobosarm s consistent effect on maintaining or improving lean body mass compared to placebo and that maintenance or improvement in lean body mass is potentially associated with longer survival in patients, regardless of treatment. Also, enobosarm was generally well tolerated, with the occurrence of serious adverse events similar across the placebo and treated groups. We plan to meet with the United States Food and Drug Administration, or FDA, to review and discuss the results of the clinical trials and a feasible pathway forward to seek marketing approval for enobosarm 3 mg for the prevention and treatment of muscle wasting in patients with advanced NSCLC. We also plan to meet with representatives from certain member countries of the European Medicines Agency to review and discuss the results of the clinical trials and a feasible regulatory pathway forward in Europe to seek marketing approval. We conducted our two Phase 3 clinical trials of enobosarm in clinical sites in the United States, Europe, Russia and South America. Each of the placebo-controlled, double-blind clinical trials was fully enrolled during the fourth quarter of 2012, with approximately 325 patients in each clinical trial. In each of these clinical trials, patients with Stage III or IV NSCLC were randomized to placebo or enobosarm 3 mg at the time they began first line chemotherapy. The last patients completed the Phase 3 clinical trials in May 2013. The vital status (survival) of patients participating in the trials will continue to be periodically monitored in accordance with the clinical trial protocols. The trials evaluated as co-primary endpoints the effect of enobosarm versus placebo on total lean body mass (muscle) assessed by dual x-ray absorptiometry, or DXA, and on physical function assessed by the Stair Climb Test at three months. Durability of effect was assessed as a secondary endpoint at five months in those patients who responded at Day 84.

In January 2013, the 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.

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

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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 approximately 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 response, which will be assessed at six months, and is defined as either those women receiving treatment who have demonstrated a complete response (disappearance of all targeted lesions), a partial response (at least a 30% 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 secondary hormonal treatment for advanced prostate cancer used in combination with androgen deprivation therapy, or ADT. We believe GTx-758 has the potential to reduce free testosterone without also causing certain estrogen deficiency side effects, such as bone loss, 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 secondary hormonal treatment of advanced prostate cancer by reducing free testosterone to levels lower than those attainable with ADT alone and potentially reducing the estrogen deficiency side effects caused by the use of ADT.

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

GTx-758 has previously demonstrated the ability to increase the production of a protein called sex hormone binding globulin, or SHBG, that binds testosterone and thereby reduces free testosterone. By reducing free testosterone, we believe serum prostate specific antigen, or PSA, will be reduced in men with CRPC. The primary endpoint of the current Phase 2 open-label clinical trial is the proportion of subjects with a $\geq$ 50% decline from baseline in serum PSA by Day 90. Other key endpoints include serum SHBG and total and free testosterone levels, as well as prostate cancer progression, in the study subjects. In addition, the clinical study will evaluate the ability of GTx-758 to treat certain estrogen deficiency side effects associated with 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 original trial design provided for 75 total subjects to be enrolled in three sequential dosing arms, with the first 25 subjects in the study being enrolled in the GTx-758 125 mg dosing arm. Assuming that an acceptable incidence of VTEs was observed when the last subject enrolled in the GTx-758 125 mg dosing arm completed one 30 day cycle of therapy, enrollment of the next 25 subjects would commence in the GTx-758 250 mg dosing arm. Similarly, the GTx-758 500 mg dosing arm was to have commenced enrollment of the final 25 subjects when the last subject enrolled in the 250 mg dose arm completed one 30 day cycle of therapy, assuming an acceptable incidence of VTEs has been observed in both of the lower dosage arms and management had decided to continue testing at the next higher dose. After reviewing data collected to date from the GTx-758 125 mg dosing arm indicating the ability of the drug to substantially increase SHBG and lower free testosterone without any unexpected side effects occurring, the clinical trial protocol was amended to eliminate the 500 mg dosing arm and to increase the number of subjects to be enrolled in the 125 mg and the 250 mg dosing arms to 38 patients per arm. Enrollment in the 125 mg cohort is continuing and, assuming there continues to be no unacceptable incidences of VTEs observed in those patients being dosed at 125 mg of GTx-758, management will then, subject to our ability to obtain additional funding, determine whether it will then proceed with the dosing of the 250 mg cohort.

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Financial Highlights

We have experienced significant recurring operating losses since our inception resulting in an accumulated deficit of $447.6 million at September 30, 2013. Our net loss for the nine months ended September 30, 2013 was $34.3 million. We expect to incur significant net losses in 2013 and for the foreseeable future. We have funded our operations primarily through the sale of equity securities, collaboration and license agreements, and prior to September 2012, product revenue from sales of FARESTON. We currently have no ongoing collaborations for the development and commercialization of our product candidates and no source of revenue, nor do we expect to generate revenue for the foreseeable future. Enobosarm and GTx-758 will require significant additional clinical development and financial resources in order to obtain necessary regulatory approvals. A substantial portion of our efforts and expenditures have been devoted to enobosarm 3 mg and we have been substantially dependent on the successful development, regulatory approval and commercialization of enobosarm 3 mg. The failure of the two Phase 3 clinical trials evaluating enobosarm 3 mg for the prevention and treatment of muscle wasting in patients with advanced NSCLC to meet each of the co-primary endpoints has significantly depressed our stock price and has severely harmed our ability to raise additional capital, and, consequently, our prospects as a going concern have been diminished. As a result, in October 2013, we announced and implemented a plan to reduce our operating expenses, including a significant reduction in our workforce, in order to preserve capital while we evaluate feasible regulatory pathways for enobosarm 3 mg, conduct our two ongoing Phase 2 clinical trials of enobosarm 9 mg and GTx-758, and pursue discussions with potential partners.

At September 30, 2013, the Company had cash, cash equivalents and short-term investments of $21.0 million compared to $56.1 million at December 31, 2012. Although we implemented a plan to reduce our operating expenses, including a significant reduction in our workforce in October 2013, we do not believe that our current cash resources will be sufficient to fund our operations for the next twelve months. Accordingly, we need to raise substantial additional capital in the near term in order to fund our operations and to continue as a going concern. 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 the enobosarm 9 mg Phase 2 clinical trial and the 125 mg dose arm of the GTx-758 Phase 2 clinical trial, our clinical trials may encounter technical, enrollment or other difficulties that could increase our development costs beyond our current estimates, and we could otherwise exhaust our available financial resources sooner than we expect, which could result in the early termination of these clinical trials. In any event, we need to raise substantial additional funding in the near term in order to fund our operations and to continue as a going concern, including in order to conduct any additional clinical development of enobosarm and GTx-758. We do not currently have any commitments for future external funding. If we are unable to raise additional funds in the near term to fund our operations and to continue as a going concern, we would be required to, among other things, make further reductions in our workforce, eliminate one or both of our ongoing clinical trials, discontinue the development of enobosarm and/or GTx-758, liquidate all or a portion of our assets, and/or seek protection under the provisions of the U.S. Bankruptcy Code, all of which would have a material adverse effect on our business and stock price. In addition, the accompanying financial statements do not include any adjustments or charges that might be necessary should we be unable to continue as a going concern, such as charges related to impairment of our assets, the recoverability and classification of assets or the amounts and classification of liabilities or other similar adjustments.

We are actively seeking to raise additional funding, primarily through potential collaboration, partnering or other strategic arrangements, and we may seek to raise funds through public or private equity offerings or debt financings, or a combination of the above. To the extent that we raise additional funds through potential collaboration, partnering or other strategic arrangements, it may be necessary to relinquish rights to some of our technologies or product candidates, or grant licenses on terms that are not favorable to us, any of which could result in the stockholders of GTx having little or no continuing interest in our enobosarm and/or GTx-758 programs as stockholders or otherwise. To the extent we raise additional funds by issuing equity securities, our stockholders may experience significant dilution, particularly given our currently depressed stock price, and debt financing, if available, may involve restrictive covenants. Any debt financing or additional equity that we raise may contain terms that are not favorable to us or our stockholders. Our ability to raise additional funds and the terms upon which we are able to raise such funds have been severely harmed by the failure of the two enobosarm 3 mg Phase 3 clinical trials to meet each of the co-primary endpoints, uncertainty regarding our financial condition and uncertainty regarding the sufficiency of our capital resources. As a result of these and other factors, we cannot be certain that additional funding will be available on acceptable terms, or at all.

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Research and Development

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

We expect that our research and development expenses for fiscal year 2013 will decrease as compared to fiscal year 2012 as the last patients completed the Phase 3 POWER 1 and POWER 2 clinical trials for enobosarm 3 mg in May 2013.

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

Product Candidates

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

Enobosarm 3 mg Prevention and treatment of muscle wasting in patients with advanced non-small cell lung cancer	SARM	Phase 3	Announced in August 2013 that 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 failed to meet the co-primary endpoints of lean body mass and physical function that were assessed statistically using responder analyses.
Enobosarm 9 mg Treatment of women with androgen receptor positive and estrogen receptor positive advanced breast cancer	SARM	Phase 2	Phase 2, open-label clinical trial of enobosarm for the treatment of androgen receptor positive and estrogen receptor positive metastatic breast cancer is ongoing.

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. Additional funding will be necessary to proceed with the dosing of the 250 mg cohort of the trial.

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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 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.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2013	2012	2013	2012
	(in thousands)		(in thousands)	
Research and development expenses	$ 356	$ 335	$ 1,051	$ 696
General and administrative expenses	406	458	1,281	1,310
Total share-based compensation	$ 762	$ 793	$ 2,332	$ 2,006

Share-based compensation expense recorded in the condensed statement of operations as general and administrative expense for the three months ended September 30, 2013 and 2012 included share-based compensation expense related to deferred compensation arrangements for our non-employee directors of $31,000 and $40,000, respectively. Share-based compensation expense recorded in the condensed statement of operations as general and administrative expense for the nine months ended September 30, 2013 and 2012 included share-based compensation expense related to deferred compensation arrangements for our non-employee directors of $105,000 and $128,000, respectively. Share-based compensation expense recorded as research and development expense for the nine months ended September 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 nine months ended September 30, 2012. At September 30, 2013, the total compensation cost related to non-vested awards not yet recognized was approximately $5.7 million with a weighted average expense recognition period of 3.11 years.

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 nine months ended September 30, 2012.

FARESTON® Revenue Recognition

Revenue from product sales of FARESTON®, which is included in net income from discontinued operations for the three and nine months ended September 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 September 30, 2013 and December 31, 2012, our accrual for product returns, was $995,000 and $1.2 million, respectively.

Results of Operations

Three and Nine Months Ended September 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.

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Proposed Candidate / Proposed Indication	Program	Three Months Ended September 30,		Nine Months Ended September 30,	
		2013	2012	2013	2012
		(in thousands)			
Enobosarm 3 mg Prevention and treatment of muscle wasting in patients with advanced non-small cell lung cancer	**SARM**	$ 3,417	$ 6,312	$ 15,840	$ 17,420
Enobosarm 9 mg Treatment of women with androgen receptor positive and estrogen receptor positive advanced breast cancer	**SARM**	513		1,464	
GTx-758 Secondary hormonal therapy in men with metastatic CRPC	**Selective ER alpha agonist**	1,171	1,729	4,253	5,969
Other research and development		1,376	1,723	4,673	5,447
Total research and development expenses		$ 6,477	$ 9,764	$ 26,230	$ 28,836

Research and development expenses decreased for the three and nine months ended September 30, 2013 from the comparable periods of 2012. For both the three and nine months ended September 30, 2013, as compared to the respective prior year comparable periods, research and development expenses related to enobosarm 3 mg decreased as the last patients completed the Phase 3 POWER 1 and POWER 2 clinical trials for enobosarm 3 mg in May 2013. Research and development expenses for enobosarm 9 mg for both periods increased as we 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 metastatic 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 both the three and nine months ended September 30, 2013 related to GTx-758 decreased as compared to the three and nine months ended September 30, 2012. During the three and nine months ended September 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.

Other research and development expenses include the cost of personnel, supplies and facilities associated with preclinical and discovery research and development activities. As a result of the October 2013 reduction in our workforce, we are no longer conducting drug discovery activities and will be focusing our research and development activities on the ongoing clinical development of our current product candidates.

General and Administrative Expenses

General and administrative expenses decreased 17% to $2.5 million for the three months ended September 30, 2013 from $3.0 million for the three months ended September 30, 2012. This was due primarily to our incurring expense for employee performance bonuses in 2012. However, this performance bonus program has been suspended for 2013.

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General and administrative expenses increased 3% to $8.2 million for the nine months ended September 30, 2013 from $8.0 million for the nine months ended September 30, 2012, primarily due to increased legal costs related to intellectual property activities and the preparation of new equity incentive plans. This was partially offset by a decrease in expense for employee performance bonuses due to the suspension of the bonus program for 2013.

Discontinued Operations

Income from discontinued operations before income taxes was $20.2 million for the three months ended September 30, 2012 and consisted of the gain on the sale of FARESTON® of $18.8 million and net product sales of FARESTON® of $1.8 million reduced by cost of FARESTON® product sales of $263,000 and FARESTON® operating expenses of $180,000.

Income from discontinued operations before income taxes was $22.8 million for the nine months ended September 30, 2012 and consisted of the gain on the sale of FARESTON® of $18.8 million and net product sales of FARESTON® of $5.3 million reduced by cost of FARESTON® product sales of $782,000 and FARESTON® operating expenses of $591,000.

Liquidity and Capital Resources

At September 30, 2013, we had cash, cash equivalents and short-term investments of $21.0 million, compared to $56.1 million at December 31, 2012. Net cash used in operating activities was $36.3 million and $27.0 million for the nine months ended September 30, 2013 and 2012, respectively.

Net cash provided by investing activities was $6.3 million for the nine months ended September 30, 2013 and resulted primarily from the maturities of short-term investments of $7.6 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 nine months ended September 30, 2012 and resulted primarily from the maturities of short-term investments of $10.0 million offset by the purchase of short-term investments of $7.8 million.

Net cash provided by financing activities was $1.2 million for the nine months ended September 30, 2013 and reflects proceeds from the exercise of employee stock options of $1.2 million partially offset by payments on capital lease obligations of $5,000. Net cash provided by financing activities was $15,000 for the nine months ended September 30, 2012 and was provided primarily from proceeds from the exercise of employee stock options of $82,000 partially offset by payments on capital lease and financed equipment obligations of $67,000.

Our significant recurring operating losses and our need for additional capital to fund our ongoing operations raise substantial doubt about our ability to continue as a going concern. We currently have no ongoing collaborations for the development and commercialization of our product candidates and no source of revenue, nor do we expect to generate revenue for the foreseeable future. Accordingly, our ability to continue as a going concern will require us to obtain substantial additional capital in the near term to fund our operations, and there can be no assurance that sufficient additional capital will be available to us or that such funding, if available, will be available on terms favorable to us.

Enobosarm and GTx-758 will require significant additional clinical development and financial resources in order to obtain necessary regulatory approvals. A substantial portion of our efforts and expenditures have been devoted to enobosarm 3 mg, and we have been substantially dependent on the successful development, regulatory approval and commercialization of enobosarm 3 mg. The failure of the two Phase 3 clinical trials evaluating enobosarm 3 mg for the prevention and treatment of muscle wasting in patients with NSCLC clinical trials to meet each of the co-primary endpoints has significantly depressed our stock price and has severely harmed our ability to raise additional capital, and consequently, our prospects as a going concern have been diminished. As a result, we implemented a plan to reduce our operating expenses, in order to preserve capital while we evaluate feasible regulatory pathways for enobosarm 3 mg, conduct two Phase 2 clinical trials of enobosarm 9 mg and GTx-758, and pursue strategic discussions with potential partners. Although we implemented a plan to reduce our operating expenses, including a significant reduction in our workforce in October 2013, we do not believe that our current

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cash resources will be sufficient to fund our operations for the next twelve months. Accordingly, we need to raise substantial additional capital in the near term in order to fund our operations and to continue as a going concern.

While we believe that, based on our current estimates of clinical trial expenditures and enrollment status, our existing capital resources are adequate to complete the enobosarm 9 mg Phase 2 clinical trial and the 125 mg dose arm of the GTx-758 Phase 2 clinical trial, our clinical trials may encounter technical, enrollment or other difficulties that could increase our development costs beyond our current estimates, and we could otherwise exhaust our available financial resources sooner than we expect, which could result in the early termination of these clinical trials. In any event, we need to raise substantial additional funding in the near term in order to fund our operations and to continue as a going concern, including in order to conduct any additional clinical development of enobosarm and GTx-758. We do not currently have any commitments for future external funding. If we are unable to raise additional funds in the near term to fund our operations and to continue as a going concern, we would be required to, among other things, make further reductions in our workforce, eliminate one or both of our ongoing clinical trials, discontinue the development of enobosarm and/or GTx-758, liquidate all or a portion of our assets, and/or seek protection under the provisions of the U.S. Bankruptcy Code, all of which would have a material adverse effect on our business and stock price.

Our estimate of the period of time or events through which our financial resources will be adequate to support our projected operating requirements is a forward-looking statement and involves risks and uncertainties, and actual results could vary as a result of a number of factors, including the factors discussed under Part 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 activities, we are unable to estimate with certainty the amounts of increased capital outlays and operating expenditures associated with the future development of our product candidates, if any. Our future funding requirements will depend on many factors, including:

- the scope, rate of progress and cost of our clinical development programs, including ongoing and any future clinical trials of enobosarm and GTx-758;
- the terms and timing of any potential collaborative, licensing and other strategic arrangements that we may establish;
- the amount and timing of any 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 effect of competing technological and market developments; and

- the cost of filing, prosecuting, defending and enforcing any patent claims and other intellectual property rights, and the cost of defending any other litigation claims.

We currently have no ongoing collaborations for the development and commercialization of our product candidates and no source of revenue, nor do we expect to generate revenue for the foreseeable future. We are actively seeking to raise additional funding, primarily through potential collaboration, partnering or other strategic arrangements, and we may seek to raise funds through public or private equity offerings or debt financings, or a combination of the above. To the extent that we raise additional funds through potential collaboration, partnering or other strategic arrangements, it may be necessary to relinquish rights to some of our technologies or product candidates, or grant licenses on terms that are not favorable to us, any of which could result in the stockholders of GTx having little or no continuing interest in our enobosarm and/or GTx-758 programs as stockholders or otherwise. To the extent we raise additional funds by issuing equity securities, our stockholders may experience significant

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dilution, particularly given our currently depressed stock price, 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. Our ability to raise additional funds and the terms upon which we are able to raise such funds have been severely harmed by the failure of the two enobosarm 3 mg Phase 3 clinical trials to meet each of the co-primary endpoints, uncertainty regarding our financial condition and uncertainty regarding the sufficiency of our capital resources. 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 we are unable to raise additional funds in the near term to fund our operations and to continue as a going concern, we would be required to, among other things, make further reductions in our workforce, eliminate one or both of our ongoing clinical trials, discontinue the development of enobosarm and/or GTx-758, liquidate all or a portion of our assets, and/or seek protection under the provisions of the U.S. Bankruptcy Code, all of which would have a material adverse effect on our business and stock price.

ITEM 3. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK

During the nine months ended September 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 third 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.

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.

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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.*

We have experienced significant recurring losses since our inception. As of September 30, 2013, we had an accumulated deficit of $447.6 million. Our net loss for the nine months ended September 30, 2013 was $34.3 million. We expect to incur significant net losses in 2013 and for the foreseeable future as we continue our clinical development activities and potentially seek regulatory approval of our product candidates. These losses, among other things, have had and will continue to have an adverse effect on our stockholders equity and working capital.

Our current product candidates, enobosarm (GTx-024) and GTx-758 (Capesaris®), will require significant additional clinical development and financial resources in order to obtain necessary regulatory approvals for these product candidates and to develop them into commercially viable products. A substantial portion of our efforts and expenditures have been devoted to enobosarm 3 mg, which was the subject of two Phase 3 clinical trials for the prevention and treatment of muscle wasting in patients with advanced non-small cell lung cancer, or NSCLC, and we have been substantially dependent on the successful development, regulatory approval and commercialization of enobosarm 3 mg. The failure of these Phase 3 clinical trials to meet each of the co-primary endpoints has significantly depressed our stock price and has severely harmed our ability to raise additional capital, and consequently, our prospects as a going concern have been diminished. As a result, we implemented a plan to reduce our operating expenses, including a significant reduction in our workforce in October 2013, in order to preserve capital while we evaluate feasible regulatory pathways for enobosarm 3 mg, conduct two Phase 2 clinical trials of enobosarm 9 mg and GTx-758, and pursue discussions with potential partners. However, the expense reduction plan alone will not be sufficient to allow us to continue our operations for the next twelve months without raising additional funds.

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 the enobosarm 9 mg Phase 2 clinical trial and the 125 mg dose arm of the GTx-758 Phase 2 clinical trial, we need to raise substantial additional funding in the near term in order to fund our operations and to continue as a going concern, including in order to conduct any additional clinical development of enobosarm and GTx-758.

If we are unable to secure additional capital necessary to fund our operations and to continue as a going concern, if we and/or any potential collaborators are unable to develop and commercialize enobosarm and GTx-758, if development is further delayed or is eliminated, or if sales revenue from enobosarm or GTx-758 upon receiving marketing approval, if ever, is insufficient, we may never become profitable and we will not be successful.

We need to raise substantial additional capital in the near term and may be unable to raise capital when needed, which would force us to delay, reduce or eliminate our product development programs and could cause us to discontinue our operations. We cannot be certain that additional capital will be available to us and, if substantial additional capital is not available, we may not be able to continue as a going concern which may result in actions that could adversely impact our stockholders. *

As of September 30, 2013, we had cash, cash equivalents and short-term investments of $21.0 million. Although we implemented a plan to reduce our operating expenses, including a significant reduction in our workforce in October 2013, we do not believe that our current cash resources will be sufficient to fund our operations for the next twelve months. Accordingly, we need to raise substantial additional capital in the near term in order to fund our operations and to continue as a going concern.

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While we believe that, based on our current estimates of clinical trial expenditures and enrollment status, our existing capital resources are adequate to complete the enobosarm 9 mg Phase 2 clinical trial and the 125 mg dose arm of the GTx-758 Phase 2 clinical trial, our clinical trials may encounter technical, enrollment or other difficulties that could increase our development costs beyond our current estimates, and we could otherwise exhaust our available financial resources sooner than we expect, which could result in the early termination of these clinical trials. In any event, we need to raise substantial additional funding in the near term in order to fund our operations and to continue as a going concern, including in order to conduct any additional clinical development of enobosarm and GTx-758. Our future funding requirements will depend on many factors, including:

- the scope, rate of progress and cost of our clinical development programs, including our ongoing and any future clinical trials of enobosarm and GTx-758;

- the terms and timing of any potential collaborative, licensing and other strategic arrangements that we may establish;

- the amount and timing of any licensing fees, milestone payments and royalty payments from potential collaborators, if any;

- future clinical trial results;

- the cost and timing of regulatory filings and/or approvals to commercialize our product candidates and any related restrictions, limitations, and/or warnings in the label of an approved product candidate;

- the effect of competing technological and market developments; and

- the cost of filing, prosecuting, defending and enforcing any patent claims and other intellectual property rights, and the cost of defending any other litigation claims.

We currently have no ongoing collaborations for the development and commercialization of our product candidates and no source of revenue, nor do we expect to generate revenue for the foreseeable future. Accordingly, we are actively seeking to raise additional funding, primarily through potential collaboration, partnering or other strategic arrangements, and we may seek to raise funds through public or private equity offerings or debt financings, or a combination of the above.

If we are unable to raise additional funds in the near term to fund our operations and to continue as a going concern, we would be required to, among other things, make further reductions in our workforce, eliminate one or both of our ongoing clinical trials, discontinue the development of enobosarm and/or GTx-758, liquidate all or a portion of our assets, and/or seek protection under the provisions of the U.S. Bankruptcy Code, all of which would have a material adverse effect on our business and stock price. In addition, the accompanying financial statements do not

include any adjustments or charges that might be necessary should we be unable to continue as a going concern, such as charges related to impairment of our assets, the recoverability and classification of assets or the amounts and classification of liabilities or other similar adjustments.

To the extent that we raise additional funds through potential collaboration, partnering or other strategic arrangements, it may be necessary to relinquish rights to some of our technologies or product candidates, or grant licenses on terms that are not favorable to us, any of which could result in the stockholders of GTx having little or no continuing interest in our enobosarm and/or GTx-758 programs as stockholders or otherwise. To the extent we raise additional funds by issuing equity securities, our stockholders may experience significant dilution, particularly given our currently depressed stock price, and debt financing, if available, may involve restrictive covenants. Any debt financing or additional equity that we raise may contain terms that are not favorable to us or our stockholders. Our ability to raise additional funds and the terms upon which we are able to raise such funds have been severely harmed by the failure of the two enobosarm 3 mg Phase 3 clinical trials to meet both of the co-primary endpoints, uncertainty regarding our financial condition and uncertainty regarding the sufficiency of our capital resources. As a result of these and other factors, we cannot be certain that additional funding will be available on acceptable terms, or at all.

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Risks Related to Development of Product Candidates

We have been substantially dependent on the success of enobosarm, and the recent failure of our two Phase 3 clinical trials evaluating enobosarm 3 mg to meet both of the co-primary endpoints, has harmed our prospects to continue as a going concern.*

A substantial portion of our efforts and expenditures have been devoted to enobosarm 3 mg, which was the subject of two Phase 3 clinical trials for the prevention and treatment of muscle wasting in patients with advanced NSCLC, and we have been substantially dependent on the successful development, regulatory approval and commercialization of enobosarm 3 mg. We announced in August 2013 that these two Phase 3 clinical trials failed to meet the co-primary endpoints of lean body mass and physical function that were assessed statistically using responder analyses. The failure of both clinical trials to meet these co-primary endpoints has significantly depressed our stock price and has severely harmed our ability to raise additional capital, and consequently, our prospects as a going concern have been diminished. Although data from the Phase 3 clinical trials demonstrated enobosarm s ability to maintain or improve lean body mass compared to placebo and its potential to positively impact survival in those patients demonstrating a lean body mass response, and we plan to meet with the FDA and representatives from certain member countries of the European Medicines Agency to review and discuss the results of the clinical trials and a feasible pathway forward to seek marketing approval for enobosarm 3 mg, there is no guarantee that we will be able to determine a feasible pathway forward with the applicable regulatory authorities. In the event that we are unable to determine a feasible pathway forward, we will likely be unable to enter into one or more collaborations or licensing arrangements with third parties for enobosarm or otherwise obtain sufficient funding, in which case, we may be required to cease further development of our enobosarm program and forego any return on our investment from our enobosarm program. This would severely harm our future prospects and may result in our ceasing operations. Even if we determine a feasible pathway forward, our ability to conduct any further clinical development of enobosarm is subject to our ability to raise additional funds, which has been severely harmed by the results of the two enobosarm 3 mg Phase 3 clinical trials, and there can be no assurances that we will be successful in obtaining additional funding in any event.

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

Significant additional clinical development and financial resources will be required to obtain necessary regulatory approvals for our product candidates and to develop them into commercially viable products. Preclinical and clinical testing is expensive, can take many years to complete and has an uncertain outcome. Success in preclinical testing and early clinical trials does not ensure that later clinical trials will be successful, and interim results of a clinical trial do not necessarily predict final results. Typically, the failure rate for development candidates is high. If a product candidate fails at any stage of development, we will not have the anticipated revenues from that product candidate to fund our operations, and we will not receive any return on our investment in that product candidate. For example, we announced in August 2013 that our two Phase 3 clinical trials evaluating enobosarm 3 mg for the prevention and treatment of muscle wasting in patients with advanced NSCLC failed to meet the co-primary endpoints of lean body mass and physical function that were assessed statistically using responder analyses. However, data from the Phase 3 clinical trials has shown enobosarm s consistent effect on maintaining or improving lean body mass compared to placebo and that maintenance or improvement in lean body mass is potentially associated with longer survival in patients regardless of treatment. Although we plan to meet with the FDA and representatives of certain member countries of the European Medicines Agency to review and discuss the results of the clinical trials and a feasible pathway forward to seek marketing approval for enobosarm 3 mg, there is no guarantee that we will be able to determine a feasible pathway forward with the applicable regulatory agencies, which could result in our ceasing further development of enobosarm program. 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 United States Food and Drug Administration, or 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.

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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

- our product candidates may not have the desired effects or may include undesirable side effects.

If any of these events were to occur and, as a result, we or any potential collaborators have significant delays in or termination of clinical trials, our costs could increase and our ability to generate revenue could be impaired, which would materially and adversely impact our business, financial condition and growth prospects.

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

In three Phase 2 clinical trials of GTx-758, which we discontinued in February 2012, we observed venous thromboembolic events, or blood clots, in subjects treated with GTx-758 at the doses then being studied in these clinical trials (1000 mg and higher per day) and reported those events to the FDA. There were two deaths in subjects treated with GTx-758 and two deaths in subjects treated with Lupron Depot®. In February 2012, the FDA placed all of our then ongoing clinical studies of GTx-758 on full clinical hold, and we suspended further enrollment into these studies and notified clinical sites to discontinue treatment of subjects with GTx-758. In May 2012, the FDA notified us that it had removed the full clinical hold on GTx-758. In the third quarter of 2012, we initiated a Phase 2 clinical trial to evaluate GTx-758, at doses lower than those which were previously being tested in our discontinued Phase 2 clinical trials, as secondary hormonal therapy in men with metastatic castration resistant prostate cancer, or CRPC. Although our current Phase 2 clinical trial is evaluating GTx-758 at doses lower than those which were previously being tested in our discontinued Phase 2 clinical trials, we cannot be confident that we will not observe an unacceptable incidence of venous thromboembolic events or other adverse events in the current Phase 2 clinical trial. Our ability to develop GTx-758 as an effective secondary hormonal therapy for men with metastatic CRPC or, potentially, as a secondary hormonal treatment for advanced prostate cancer used in combination with ADT, is dependent on both our ability to obtain additional funding and our ability to find an appropriate dose that is both effective and safe for these patient populations. If an unacceptable incidence of venous thromboembolic events or other adverse events are observed in our current Phase 2 clinical trial of GTx-758, we may be required to abandon our development of GTx-758, in which case, we would not receive any return on our investment in that product candidate.

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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.

Risks Related to Our Dependence on Third Parties

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

Our strategy includes selectively partnering or collaborating with leading pharmaceutical and biotechnology companies to assist us in furthering development and potential commercialization of our product candidates. We face significant competition in seeking appropriate collaborators,

and collaborations are complex and time consuming to negotiate and document. We may not be successful in entering into new collaborations with third parties on acceptable terms, or at all. In addition, we are unable to predict when, if ever, we will enter into any additional collaborative arrangements because of the numerous risks and uncertainties associated with establishing such arrangements. If we are unable to negotiate new collaborations, we may have to curtail the development of a particular product candidate, reduce, delay, or terminate its development or one or more of our other development programs, delay its potential commercialization or reduce the scope of our sales or marketing activities or increase our expenditures and undertake development or commercialization activities at our own expense. For example, we may have to cease further development of our enobosarm program if we are unable to raise sufficient funding for any additional clinical development of enobosarm through a new partnership, collaboration or financing, even if we determine there is a feasible regulatory pathway forward for its development. In this regard, we do not have sufficient funds to conduct any additional clinical development of our product candidates beyond the Phase 2 clinical trials we are currently conducting, and our ability to conduct any further clinical development of our product candidates, including the 250 mg cohort of our GTx-758 Phase 2 clinical trial, is subject to our ability to raise additional funds, which has been severely harmed by the failure of the two enobosarm 3 mg Phase 3 clinical trials to meet both of the co-primary endpoints, and there can be no assurances that we will be successful in obtaining additional funding in any event. If we are not able to raise substantial additional capital in the near term, we will not be able to bring our product candidates to market and generate product revenues.

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

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

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

- we may not be able to control the amount and timing of resources that our potential collaborators may devote to our product candidates;

- potential collaborations may experience financial difficulties or changes in business focus;

- we may be required to relinquish important rights such as marketing and distribution rights;

- should a collaborator fail to develop or commercialize one of our compounds or product candidates, we may not receive any future milestone payments and will not receive any royalties for the compound or product candidate;

- business combinations or significant changes in a collaborator s business strategy may also adversely affect a collaborator s willingness or ability to complete its obligations under any arrangement;

- under certain circumstances, a collaborator could move forward with a competing product candidate developed either independently or in collaboration with others, including our competitors; and

- collaborative arrangements are often terminated or allowed to expire, which could delay the development and may increase the cost of developing our product candidates.

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

We do not currently own or operate manufacturing facilities, and we rely, and expect to continue to rely, on third parties for the production of clinical and commercial quantities of our product candidates. Our current and anticipated future dependence upon others for the manufacture of our product candidates may adversely affect our future profit margins, if any, and our ability to develop product candidates and commercialize any product candidates on a timely and competitive basis.

We rely on third-party vendors for the manufacture of enobosarm drug substance. If the contract manufacturers that we are currently utilizing to meet our supply needs for enobosarm or any future SARM product candidates prove incapable or unwilling to continue to meet our supply needs, we could experience a delay in conducting any additional clinical trials of enobosarm or any future SARM product candidates. In addition, we rely on third-party contractors for the manufacture of GTx-758 drug substance. We may not be able to maintain or renew our existing or any other third-party manufacturing arrangements on acceptable terms, if at all. If our suppliers fail to meet our requirements for GTx-758, enobosarm or any future product candidates for any reason, we would be required to obtain alternate suppliers. Any inability to obtain alternate suppliers, including an inability to obtain approval from the FDA of an alternate supplier, would delay or prevent the clinical development and commercialization of these product candidates.

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

Reliance on third-party manufacturers entails risks, to which we would not be subject if we manufactured product candidates or products ourselves, including:

- reliance on the third party for regulatory compliance and quality assurance;

- the possible breach of the manufacturing agreement by the third party because of factors beyond our

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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.

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

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

We may be subject to competition from third parties with products in the same class of products as our product candidates or products with the same active pharmaceutical ingredients as our product candidates in those jurisdictions in which we have no patent protection. Even if patents are issued to us or our licensors regarding our product candidates or methods of using them, those patents can be challenged by our competitors who can argue such patents are invalid or unenforceable, lack of utility, lack sufficient written description or enablement, or that the claims of the issued patents should be limited or narrowly construed. Patents also will not protect our product candidates if competitors devise ways of making or using these product candidates without legally infringing our patents. The Federal Food, Drug, and Cosmetic Act and FDA regulations and policies create a regulatory environment that encourages companies to challenge branded drug patents or to create non-infringing versions of a patented product in order to facilitate the approval of abbreviated new drug applications for generic substitutes. These same types of incentives encourage competitors to submit new drug applications that rely on literature and clinical data not prepared for or by the drug sponsor, providing another less burdensome pathway to approval.

We also rely on trade secrets to protect our technology, especially where we do not believe that patent protection is appropriate or obtainable. However, trade secrets are difficult to protect. Our employees, consultants, contractors, outside scientific collaborators and other advisors may unintentionally or willfully disclose our confidential information to competitors, and confidentiality agreements may not provide an adequate remedy in the event of unauthorized disclosure of confidential information. Enforcing a claim that a third party illegally obtained and is using our trade secrets is expensive and time-consuming, and the outcome is unpredictable. Moreover, our competitors may independently develop equivalent knowledge, methods and know-how. Failure to obtain or maintain trade secret protection could adversely affect our competitive business position.

If we infringe intellectual property rights of third parties, it may increase our costs or prevent us from being able to commercialize our product candidates.*

There is a risk that we are infringing the proprietary rights of third parties because numerous United States and foreign issued patents and pending patent applications, which are owned by third parties, exist in the fields that are the focus of our development and manufacturing efforts. Others might have been the first to make the inventions covered by each of our or our licensors pending patent applications and issued patents and/or might have been the first to file patent applications for these inventions. In addition, because patent applications can take many years to issue, there may be currently pending applications, unknown to us or our licensors, which may later result in issued patents that cover the production, manufacture, synthesis, commercialization, formulation or use of our product candidates. In addition, the production, manufacture, synthesis, commercialization, formulation or use of our product candidates may infringe existing patents of which we are not aware. Defending

ourselves against third-party claims, including litigation in particular, would be costly and time consuming and would divert management s attention from our business, which could lead to delays in our development or commercialization efforts. If third parties are successful in their claims, we might have to pay substantial damages or take other actions that are adverse to our business.

As a result of intellectual property infringement claims, or to avoid potential claims, we might:

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- 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 for the foreseeable future, if at all. The process of obtaining regulatory approvals is expensive, often takes many years, if approval is obtained at all, and can vary substantially based upon the type, complexity and novelty of the product candidates involved.

Changes in the regulatory approval policy during the development period, changes in or the enactment of additional regulations or statutes, or changes in regulatory review for each submitted product application may cause delays in the approval or rejection of an application. 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

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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. Since our two Phase 3 clinical trials of enobosarm 3 mg failed to meet each of the co-primary endpoints, there can be no assurance that the FDA will ultimately determine that data from our clinical trials of enobosarm will be found acceptable by the FDA for the purpose of either approving the product candidate or designing one or more additional clinical trials to be conducted by us to confirm those endpoints met in the Phase 3 trials. 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 for the foreseeable future, 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 NSCLC, 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;

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

If we are unable to establish sales and marketing capabilities or establish and maintain agreements with third parties to market and sell our product candidates, we may be unable to generate product revenue from such candidates.

We have limited experience as a company in the sales, marketing and distribution of pharmaceutical products. In the event one of our product candidates is approved, we will need to establish sales and marketing capabilities or establish and maintain agreements with third parties to market and sell our product candidates. We may be unable to build our own sales and marketing capabilities, and there are risks involved with entering into arrangements with third parties to perform these services, which could delay the commercialization of any of our product candidates if approved for commercial sale. In addition, to the extent that we enter into arrangements with third parties to perform sales, marketing and distribution services, our product revenues are likely to be lower than if we market and sell any products that we develop ourselves.

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

Sales of products developed by us and/or any potential collaborators are dependent on the availability and extent of reimbursement from third-party payors. Changes in the reimbursement policies of these third-party payors that reduce reimbursements for any products that we

and/or any potential collaborators may develop and sell could negatively impact our future operating and financial results.

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

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

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

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

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

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

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

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

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

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

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

- decreased demand for any product candidates or products;

- injury to our reputation;

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- 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.

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

With respect to our SARM program, there are other SARM product candidates in development that may compete with enobosarm and any future SARM product candidates, if approved for commercial sale, including SARMs in development from Ligand Pharmaceuticals Inc. and GlaxoSmithKline plc. Pfizer Inc., Eli Lilly and Company and Amgen Inc. have myostatin inhibitors in development that may compete with

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

We are developing GTx-758 for secondary hormonal therapy in men with metastatic CRPC, and, potentially, as a secondary hormonal treatment for advanced prostate cancer used in combination with androgen deprivation therapy. There are various products approved or under clinical development to treat men with advanced prostate cancer who have metastatic CRPC which may compete with GTx-758. Dendreon Corporation markets and sells Provenge®, an autologous cellular immunotherapy, for the treatment of asymptomatic or minimally symptomatic metastatic castrate resistant prostate cancer. Medivation, Inc. has received approval for Xtandi®, an oral androgen receptor antagonist, for the treatment of metastatic castration-resistant prostate cancer in men previously treated with docetaxel. Medivation continues to develop Xtandi® for men with metastatic CRPC prior to receiving chemotherapy. Zytiga®, sold by Johnson & Johnson, has been approved for the treatment of metastatic CRPC prior in patients who have received prior chemotherapy and recently received approval for the treatment of metastatic castrate resistant prostate cancer prior to chemotherapy. Johnson & Johnson 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.

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GTx-758 is being developed as a treatment for this same patient population prior to the initiation of chemotherapy.

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

Risks Related to Employees and Growth

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

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

In October 2013, we announced a reduction of approximately 60% of our workforce in order to reduce our expenses and preserve capital in connection with the failure of our two Phase 3 clinical trials evaluating enobosarm 3 mg for the prevention and treatment of muscle wasting in patients with advanced NSCLC to meet their respective co-primary endpoints. We also announced in October 2013 that our Chief Financial Officer notified us of his intent to resign from GTx, effective December 31, 2013. As a result of our October 2013 workforce reduction, only 37 employees remained as employees of GTx as of October 31, 2013. Accordingly, we will be operating with a shortage of resources and may not be able to effectively conduct our operations with this limited number of employees. In addition, we announced past workforce reductions in each of December 2009 and June 2011, and our history of implementing workforce reductions, along with the potential for future workforce reductions, may negatively affect our ability to retain or attract talented employees.

If we are able to raise sufficient additional funds necessary to continue as a going concern and to pursue the development of our product candidates, we will need to hire additional employees. Any inability to manage future growth could harm our ability to develop and commercialize our product candidates, increase our costs and adversely impact our ability to compete effectively.*

If we are able to raise sufficient additional funds necessary to continue as a going concern and to pursue the development of our product candidates, we will need to hire experienced personnel to develop and commercialize our product candidates, and we will need to expand the number of our managerial, operational, financial and other employees to support that growth. Competition exists for qualified personnel in the biotechnology field.

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

Risks Related to Our Common Stock

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

The market prices for securities of biotechnology companies in general have been highly volatile and may continue to be so in the future. The following factors, in addition to other risk factors described in this section, may have a significant impact on the market price of our common stock:

- our financial situation, including our ability to raise additional capital in the near term to fund our operations and to continue as a going concern, and the terms and timing of any related financing

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arrangements;

- announcements regarding our ability to determine, in consultation with the FDA and representatives of certain member countries of the European Medicines Agency, a feasible pathway forward to seek marketing approval for enobosarm 3 mg for the prevention and treatment of muscle wasting in patients with advanced NSCLC;

- 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;

- announcements regarding further cost-cutting initiatives or restructurings;

- our ability to enter into new collaborative, licensing or other strategic arrangements with respect to our product candidates;

- the terms and timing of any future collaborative, licensing or other arrangements that we may establish;

- the timing of achievement of, or failure to achieve, our and any potential collaborators clinical, regulatory and other milestones, such as the commencement of clinical development, the completion of a clinical trial or the receipt of regulatory approval;

- announcement of FDA approval or non-approval of our product candidates or delays in or adverse events during the FDA review process;

- actions taken by regulatory agencies with respect to our product candidates or products, our clinical trials or our sales and marketing activities, including regulatory actions requiring or leading to restrictions, limitations and/or warnings in the label of an approved product candidate;

- the commercial success of any product approved by the FDA or its foreign counterparts;

- introductions or announcements of technological innovations or new products by us, our potential collaborators, or our competitors, and the timing of these introductions or announcements;

- market conditions for equity investments in general, or the biotechnology or pharmaceutical industries in particular;

- announcements regarding our ability to comply with the minimum listing requirements of The NASDAQ Stock Market LLC;

- regulatory developments in the United States and foreign countries;

- changes in the structure or reimbursement policies of health care payment systems;

- any intellectual property infringement lawsuit involving us;

- actual or anticipated fluctuations in our results of operations;

- changes in financial estimates or recommendations by securities analysts;

- hedging or arbitrage trading activity that may develop regarding our common stock;

- sales of large blocks of our common stock;

- sales of our common stock by our executive officers, directors and significant stockholders;

- changes in accounting principles; and

- the loss of any of our key scientific or management personnel.

The stock markets in general, and the markets for biotechnology stocks in particular, have experienced significant volatility that has often been unrelated to the operating performance of particular companies. The financial markets continue to face significant uncertainty, resulting in a decline in investor confidence and concerns

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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 September 30, 2013, our executive officers, directors and holders of 5% or more of our outstanding common stock beneficially owned approximately 66% 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, have the ability to control all matters requiring approval by our stockholders, including the election of directors and the approval of mergers or other business combination transactions. The interests of this group of stockholders may not always coincide with our interests or the interests of other stockholders.

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

Our common stock is currently listed on The NASDAQ Global Market. The NASDAQ Stock Market LLC, or NASDAQ, has minimum requirements that a company must meet in order to remain listed on The NASDAQ Global Market. These requirements include maintaining a minimum closing bid price of $1.00 per share, and the closing bid price of our common stock on November 7, 2013 was $1.59 share. If the closing bid price of our common stock were to fall below $1.00 per share for 30 consecutive trading days or we do not meet other applicable listing requirements, we would fail to be in compliance with NASDAQ s listing standards. There can be no assurance that we will continue to meet the minimum bid price requirement, or any other NASDAQ continued listing requirement, in the future. If we fail to meet these requirements, including the minimum bid price requirement, NASDAQ may notify us that we have failed to meet the minimum listing requirements and initiate the delisting process. If our common stock is delisted, the liquidity of our common stock would be adversely affected and the market price of our common stock could decrease, and our ability to obtain sufficient additional capital to fund our operations and to continue as a going concern would be substantially impaired.

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.

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

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

For the 12-month period ended September 30, 2013, the average daily trading volume of our common stock on The NASDAQ Global Market was 563,919 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 September 30, 2013, we had 63,185,389 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: November 12, 2013 By: /s/ Mitchell S. Steiner

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

Date: November 12, 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 Registrant and Strakan International S.à r.l.	8-K	000-50549	2.1	10/03/2012
3.1	Restated Certificate of Incorporation of GTx, Inc.	S-3	333-127175	4.1	08/04/2005
3.2	Certificate of Amendment of Restated Certificate of Incorporation of GTx, Inc.	8-K	000-50549	3.2	05/06/2011
3.3	Amended and Restated Bylaws of GTx, Inc.	8-K	000-50549	3.2	07/26/2007
4.1	Reference is made to Exhibits 3.1, 3.2 and 3.3				
4.2	Specimen of Common Stock Certificate	S-1	333-109700	4.2	12/22/2003
4.3	Amended and Restated Registration Rights Agreement between Registrant and J. R. Hyde, III dated August 7, 2003	S-1	333-109700	4.4	10/15/2003
4.4	Consent, Waiver and Amendment 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+	Form of Retention Benefits Letter Agreement for Mitchell S. Steiner and Marc S. Hanover				
10.2+	Form of Retention Benefits Letter Agreement for James T. Dalton and Henry P. Doggrell				
10.3+	Form of Retention Stock Option Grant Notice and Option Agreement under the GTx, Inc. 2013 Equity Incentive Plan				
10.4+	Form of Retention Restricted Stock Unit Grant Notice and Restricted Stock Unit Award Agreement under the GTx, Inc. 2013 Equity Incentive Plan				
10.5+	Third Memorandum of Understanding, made effective as of October 1, 2013, Concerning the Lease Agreement between Registrant and The University of Tennessee Research Foundation as Amended July 20, 2009				
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
101.SCH+	XBRL Taxonomy Extension Schema Document
101.CAL+	XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF+	XBRL Taxonomy Extension Definition Linkbase Document
101.LAB+	XBRL Taxonomy Extension Labels Linkbase Document
101.PRE+	XBRL Taxonomy Extension Presentation Linkbase Document

+ Filed herewith

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