Table of Contents

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, 2014

OR

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

For the transition period from to

Commission file number: 000-50549

GTx, Inc.

(Exact name of registrant as specified in its charter)

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

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

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

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

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

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

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

As of November 6, 2014, 76,014,531 shares of the registrant's Common Stock were outstanding.

Table of Contents

GTx, INC.
FORM 10-Q FOR THE QUARTER ENDED SEPTEMBER 30, 2014
INDEX

PAGE

Table of Contents

PART I: FINANCIAL INFORMATION

ITEM 1. FINANCIAL STATEMENTS

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

	September 30, 2014 (unaudited)	December 31, 2013
ASSETS		
Current assets:		
Cash and cash equivalents	$ 9,627	$ 14,529
Short-term investments	1,915	200
Prepaid expenses and other current assets	919	442
Total current assets	12,461	15,171
Property and equipment, net	42	112
Intangible and other assets, net	513	322
Total assets	$ 13,016	$ 15,605
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	$ 179	$ 808
Accrued expenses and other current liabilities	2,139	3,759
Total current liabilities	2,318	4,567
Other long-term liabilities	82	354
Commitments and contingencies		
Stockholders' equity:		
Common stock, $0.001 par value: 200,000,000 and 120,000,000 shares authorized at September 30, 2014 and December 31, 2013, respectively; 76,014,531 and 63,185,389 shares issued and outstanding at September 30, 2014 and December 31, 2013, respectively	76	63
Additional paid-in capital	490,766	465,981
Accumulated deficit	(480,226)	(455,360)
Total stockholders' equity	10,616	10,684
Total liabilities and stockholders' equity	$ 13,016	$ 15,605

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

Table of Contents

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,	
	2014	2013	2014	2013
Expenses:				
Research and development expenses	$ 3,362	$ 6,477	$ 17,616	$ 26,230
General and administrative expenses	1,594	2,483	7,275	8,190
Total expenses	4,956	8,960	24,891	34,420
Loss from operations	(4,956)	(8,960)	(24,891)	(34,420)
Other income, net	21	23	25	99
Net loss	$ (4,935)	$ (8,937)	$ (24,866)	$ (34,321)
Net loss per share:				
Basic and diluted	$ (0.06)	$ (0.14)	$ (0.34)	$ (0.54)
Weighted average shares outstanding:				
Basic and diluted	76,014,531	63,179,394	72,688,108	63,013,923

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

Table of Contents

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

	Nine Months Ended September 30,	
	2014	2013
Cash flows from operating activities:		
Net loss	$ (24,866)	$ (34,321)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	86	333
Share-based compensation	4,186	2,227
Directors' deferred compensation	94	105
Changes in assets and liabilities:		
Prepaid expenses and other assets	(679)	(150)
Accounts payable	(629)	(454)
Accrued expenses and other liabilities	(1,890)	(4,060)
Net cash used in operating activities	(23,698)	(36,320)
Cash flows from investing activities:		
Purchase of property and equipment	(5)	(32)
Purchase of short-term investments, held to maturity	(10,490)	(1,225)
Proceeds from maturities of short-term investments, held to maturity	8,775	7,595
Net cash (used in) provided by investing activities	(1,720)	6,338
Cash flows from financing activities:		
Net proceeds from the issuance of common stock and warrants	21,135	—
Tax payments related to shares withheld for vested restricted stock units	(617)	—
Payments on capital lease and financed equipment obligations	(2)	(5)
Proceeds from exercise of employee stock options	—	1,226
Net cash provided by financing activities	20,516	1,221
Net decrease in cash and cash equivalents	(4,902)	(28,761)
Cash and cash equivalents, beginning of period	14,529	48,044
Cash and cash equivalents, end of period	$ 9,627	$ 19,283

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

Table of Contents

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

1. Business and Basis of Presentation

Business

GTx, Inc. ("GTx" or the "Company"), a Delaware corporation incorporated on September 24, 1997 and headquartered in Memphis, Tennessee, is a biopharmaceutical company dedicated to the discovery, development and commercialization of small molecules for the treatment of cancer, including treatments for breast and prostate 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 that the Company believes have the potential to be used as a novel hormonal therapy for the treatment of advanced breast cancer, as well as the potential to prevent and treat muscle wasting in patients with cancer and other musculoskeletal wasting or muscle loss conditions. The Company announced during the second quarter of 2014 positive preliminary results from an ongoing Phase 2 proof-of-concept, open-label clinical trial evaluating a 9 mg oral daily dose of enobosarm for the treatment of patients with estrogen receptor ("ER") positive and androgen receptor ("AR") positive metastatic breast cancer who have previously responded to hormonal therapy. The Company's current strategy is focused on further development of enobosarm in two breast cancer indications targeting the androgen receptor. Subject to the completion of the private placement of its common stock and warrants that the Company announced on November 10, 2014 and the receipt of necessary regulatory approvals, it plans to initiate two Phase 2 clinical trials for these indications in the first half of 2015. One of these Phase 2 clinical trials is designed to enroll patients with ER positive and AR positive metastatic breast cancer, while the other Phase 2 clinical trial is designed to enroll patients with advanced AR positive triple-negative breast cancer.

The Company announced in August 2013 that its POWER 1 (platinum plus taxane chemotherapy) and POWER 2 (platinum plus non-taxane chemotherapy) Phase 3 clinical trials evaluating enobosarm 3 mg daily for the prevention and treatment of muscle wasting in patients with advanced non-small cell lung cancer ("NSCLC") failed to meet the statistical criterion for the co-primary endpoints of lean body mass and physical function that were assessed using responder analyses as pre-specified for the United States Food and Drug Administration ("FDA"). Enobosarm did, however, demonstrate a statistically significant effect versus placebo on the primary endpoint of physical function in the POWER 1 clinical trial as assessed by continuous variable analysis, as pre-specified in the Company's statistical analysis plan for the European Medicines Agency ("EMA"). The Company has been evaluating whether data from its two Phase 3 POWER clinical trials is sufficient to support the filing of a marketing authorization application ("MAA") in the European Union ("EU") for enobosarm 3 mg for the prevention and treatment of muscle wasting in patients with advanced NSCLC, and has met with representatives from member countries to the EMA to review and discuss the results of the POWER trials. Based upon recent input from these representatives, the Company believes that the data from the POWER trials is not sufficient to support the filing and approval of a MAA in the EU for enobosarm 3 mg for the prevention and treatment of muscle wasting in patients with advanced NSCLC without confirmatory data from another Phase 3 clinical trial of enobosarm in this indication. As a result of the input received from these representatives, the Company currently does not intend to file a MAA and will evaluate whether there is commercial rationale and partner interest to support additional clinical development required for approval. In the Company's meeting with the FDA in February 2014 to review and discuss the results of the POWER clinical trials, the Company learned that since data from the two POWER trials failed to meet the statistical criterion pre-specified for the co-primary endpoints of lean body mass and physical function, the FDA was not willing to accept a new drug application for enobosarm 3 mg for the prevention and treatment of muscle wasting in patients with advanced NSCLC. The Company's strategy does not currently include further development of enobosarm for this indication in the U.S. or in Europe unless such development is part of a collaborative arrangement or strategic partnership. The Company continues to evaluate other potential indications in the U.S. for enobosarm.

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 a secondary hormonal therapy in men with metastatic and high risk non-metastatic castration resistant prostate cancer.

In April 2014, the Company announced that Mitchell S. Steiner, its former Chief Executive Officer ("CEO"), Vice Chairman of the Board of Directors and a co-founder of the Company, was leaving the Company to pursue other business interests. Dr. Steiner resigned from his roles as CEO and Vice Chairman of the Board of Directors of the Company effective April 3, 2014. In connection with Dr. Steiner's resignation, Marc S. Hanover was appointed as the Company's interim Chief Executive Officer and was elected to the Board to serve the remainder of Dr. Steiner's term on the Board until the 2015 annual meeting of the Company's stockholders. Also in connection with Dr. Steiner's resignation, the Company entered into a severance agreement with Dr. Steiner, pursuant to which Dr. Steiner received severance benefits of twelve months of base salary continuation payments and continued healthcare coverage through the earliest of December 31, 2014 or the date he ceases to be eligible for COBRA continuation coverage. As a result of these severance benefits, the Company recognized cash severance related expenses of $483 during the nine months ended September 30, 2014. Additionally, all of Dr. Steiner's outstanding unvested stock options were vested and became immediately exercisable on April 13, 2014. The Company extended the post-termination exercise period of all of his stock options until the earlier to occur of (i) April 13, 2019 or (ii) the expiration of the term of a particular stock option grant. The Company recorded a one-time, noncash net compensation expense of $215 relating to these stock option modifications. See Note 2, *Share-Based Compensation*, for further information.

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 the Company's financial position, results of operations and cash flows for each period presented in accordance with accounting principles generally accepted in the United States for interim financial information and with the instructions to Form 10-Q and Article 10 of Regulation S-X. Accordingly, information and footnote disclosures normally included in financial statements prepared in accordance with accounting principles generally accepted in the United States have been condensed or omitted from the accompanying condensed financial statements. These interim condensed financial statements should be read in conjunction with the audited financial statements and related notes thereto, which are included in the Company's Annual Report on Form 10-K for the year ended December 31, 2013. Operating results for the three and nine months ended September 30, 2014 are not necessarily indicative of the results that may be expected for the entire fiscal year ending December 31, 2014.

Use of Estimates

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

Research and Development Expenses

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

Table of Contents

estimate of services received and degree of completion of the services in accordance with the specific third party contract. As a result of the October 2013 reduction in its workforce, the Company is no longer conducting drug discovery activities and is focusing its research and development activities on the ongoing clinical development of the Company's current product candidates.

Cash, Cash Equivalents and Short-term Investments

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

At September 30, 2014 and December 31, 2013, short-term investments consisted of Federal Deposit Insurance Corporation insured certificates of deposit with original maturities of greater than three months and less than one year. As the Company has the positive intent and ability to hold the certificates of deposit until maturity, these investments have been classified as held to maturity investments and are stated at cost, which approximates fair value. The Company considers these to be Level 2 investments as the fair values of these investments are determined using third-party pricing sources, which generally utilize observable inputs, such as interest rates and maturities of similar assets.

Income Taxes

The Company accounts for deferred taxes by recognition of deferred tax assets and liabilities for the expected future tax consequences of events that have been included in the financial statements or tax returns. Under this method, deferred tax assets and liabilities are determined based on the difference between the financial statement and tax basis of assets and liabilities using enacted tax rates in effect for the year in which the differences are expected to reverse. A valuation allowance is provided when it is more likely than not that some portion or all of the deferred tax assets will not be realized. Accordingly, at September 30, 2014 and December 31, 2013, net of the valuation allowance, the net deferred tax assets were reduced to zero. Income taxes are described more fully in Note 9 to the Company's financial statements included in the Company's Annual Report on Form 10-K for the year ended December 31, 2013.

Other Income, net

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

FARESTON® Revenue Recognition

Although the Company sold its rights and certain assets related to FARESTON® effective September 30, 2012, the Company retained the liability for future product returns relating to sales of FARESTON® made by the Company prior to September 30, 2012. Therefore, the Company estimates an accrual for product returns based on factors which include historical product returns and estimated product in the distribution channel which is expected to exceed its expiration date. At September 30, 2014 and December 31, 2013, the Company's accrual for product returns was $141 and $918, respectively. Of these amounts, $82 and $332 have been included in "Other long-term liabilities" in the condensed balance sheet at September 30, 2014 and December 31, 2013, respectively, and represents the portion of the Company's product returns accrual estimated to be payable after one year.

Table of Contents

Subsequent Events

(in thousands, except share and per share data)

The Company has evaluated all events or transactions that occurred after September 30, 2014 up through the date the condensed financial statements were issued. Other than as set forth below, there were no material recognizable or nonrecognizable subsequent events during the period evaluated.

On November 9, 2014, the Company entered into a definitive securities purchase agreement (the "Purchase Agreement") with certain purchasers, including members of the Company's management and Board of Directors (the "Purchasers"), pursuant to which the Company agreed to issue and sell to the Purchasers, subject to customary closing conditions, an aggregate of 64,311,112 immediately separable units, comprised of an aggregate of 64,311,112 shares of common stock and warrants to purchase up to 64,311,112 additional shares of common stock, for an aggregate purchase price of $43,410 (the "Private Placement"). The closing of the Private Placement is subject to the satisfaction of customary closing conditions. The warrants the Company agreed to issue at the closing of the Private Placement, which would have a per share exercise price of $0.85, would be subject to net cash settlement if at the time of any exercise, there are then an insufficient number of authorized and reserved shares of common stock to effect a share settlement of the warrants. The Company does not currently have a sufficient number of authorized and unreserved shares of common stock necessary to settle exercises of the warrants in full in shares of common stock. Under the Purchase Agreement, the Company has agreed to seek stockholder approval, at a special or annual meeting to be held no later than May 27, 2015, of an amendment to the Company's Certificate of Incorporation to increase the Company's authorized common stock to an amount necessary to effect the share settlement of all of the warrants the Company agreed to issue at the closing of the Private Placement. Assuming such approval is obtained, warrant exercises would no longer be subject to net cash settlement. The warrants would generally become exercisable on the date such stockholder approval is obtained (but in no event later than June 1, 2015) and would continue to be exercisable for four years thereafter. The Company also agreed to certain registration obligations with respect to the resale by the Purchasers of the common stock issued or underlying the warrants issued in the Private Placement. The Purchase Agreement may be terminated by either the Company or the Purchasers if the closing has not occurred by November 21, 2014 (subject to extension to November 28, 2014 under limited circumstances).

2. Share-Based Compensation

Share-based payments include stock option grants and restricted stock units ("RSUs") 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 is required to provide service in exchange for the award. The Company's share-based compensation plans are described more fully in Note 3 to the financial statements included in the Company's Annual Report on Form 10-K for the year ended December 31, 2013.

The following table summarizes share-based compensation expense included within the condensed statements of operations for the three and nine months ended September 30, 2014 and 2013:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2014	2013	2014	2013
Research and development expenses	$ 89	$ 356	$ 2,419	$ 1,051
General and administrative expenses	177	406	1,861	1,281
Total share-based compensation	$ 266	$ 762	$ 4,280	$ 2,332

Share-based compensation expense recorded as general and administrative expense for both the three months ended September 30, 2014 and 2013 included share-based compensation expense related to deferred compensation arrangements for the Company's non-employee directors of $31. Share-based compensation expense recorded as general and administrative expense for the nine months ended September 30, 2014 and 2013 included share-based compensation expense related to deferred compensation arrangements for the Company's non-employee directors of $94 and $105, respectively. As a result of the modification of Dr. Steiner's options upon his resignation in April 2014, the Company recognized a one-time, noncash net compensation expense of $215, which was included in general and administrative expenses for the nine months ended September 30, 2014. This amount reflects the net of the aggregate incremental fair value associated with the modifications of $359, partially offset by the reversal of $144 of previously recognized share-based compensation expense for Dr. Steiner's unvested options.

The Company uses the Black-Scholes-Merton option pricing valuation model ("Black-Scholes Model") to value stock options. The expected life of options is determined by calculating the average of the vesting term and the contractual term of the options. The expected price volatility is based on the Company's historical stock price volatility. The risk-free interest rate is determined using U.S. Treasury rates where the term is consistent with the expected life of the stock options. Expected dividend yield is not considered as the Company has not made any dividend payments and has no plans of doing so in the foreseeable future. The amount of share-based compensation expense recognized is reduced ratably over the vesting period by an estimate of the percentage of options granted that are expected to be forfeited or canceled before becoming fully vested.

Table of Contents

The Company estimated the fair value of RSUs using the closing price of its stock on the grant date. The fair value of RSUs was amortized on a straight-line basis over the requisite service period of the awards. At December 31, 2013, the Company had 1,225,000 unvested RSUs outstanding with a weighted average grant date fair value per share of $1.87. All of the Company's outstanding RSUs vested during the second quarter of 2014 and no RSUs were outstanding at September 30, 2014. The number of RSUs vested includes 371,906 shares that were withheld on behalf of the Company's employees to satisfy the statutory tax withholding requirements.

The fair value of options granted to employees and non-employee directors was estimated using the following assumptions for the periods presented:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2014	2013	2014	2013
Expected price volatility	86.6%	74.8%	86.5%	74.5%
Risk-free interest rate	2.1%	1.8%	2.3%	1.1%

(in thousands, except share and per share data)

Weighted average expected life in years	7.0 years	6.5 years	6.9 years	6.5 years

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

	Number of Shares	Weighted Average Exercise Price Per Share
Options outstanding at December 31, 2013	6,445,342	$ 6.58
Options granted	3,042,500	1.36
Options forfeited or expired	(1,242,108)	9.03
Options exercised	—	—
Options outstanding at September 30, 2014	8,245,734	4.28

3. Basic and Diluted Net Loss Per Share

Basic and diluted net loss per share attributable to common stockholders is calculated based on the weighted average number of common shares outstanding during the period. Diluted net loss per share gives effect to the dilutive potential of common stock consisting of stock options, unvested restricted stock units and common stock warrants.

Weighted average potential shares of common stock of 18,395,096 and 6,136,483 for the three months ended September 30, 2014 and 2013, respectively, and 15,658,766 and 6,432,994 for the nine months ended September 30, 2014 and 2013, respectively, were excluded from the calculations of diluted loss per share as inclusion of the potential shares would have had an anti-dilutive effect on the net loss per share for these periods.

4. Common Stock

On March 6, 2014, the Company completed a private placement of units consisting of an aggregate of 11,976,048 shares of common stock and warrants to purchase an aggregate of 10,179,642 shares of its common stock for gross proceeds of $21,272. No underwriting discounts or commissions or similar fees were payable in connection with the issuance.

The warrants, which have a one year term expiring on March 6, 2015, have a per share exercise price of $1.67 that is payable only in cash. The Company assessed whether the warrants require accounting as derivatives. The Company determined that the warrants were indexed to the Company's own stock. As such, the Company has

Table of Contents

concluded the warrants meet the scope exception for determining whether the instruments require accounting as derivatives and are classified in stockholders' equity. The fair value of the warrants was estimated at $4,478 using the Black-Scholes Model with the following assumptions: expected volatility of 67%, risk free interest rate of 0.12%, expected life of one year and no dividends. The net proceeds from the private placement were allocated to the common stock and warrants based upon their relative fair values.

5. University of Tennessee Research Foundation License Agreement

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

Table of Contents

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

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

Forward-Looking Information

This Quarterly Report on Form 10-Q contains forward-looking statements. The forward-looking statements are contained principally in the sections entitled "Management's Discussion and Analysis of Financial Condition and Results of Operations" and "Risk Factors." These statements involve known and unknown risks, uncertainties and other factors that may cause our actual results, performance or achievements to be materially different from any future results, performances or achievements expressed or implied by the forward-looking statements. Forward-looking statements include statements about:

- the implementation of our business strategies, including our ability to preserve or realize any significant value from our enobosarm (GTx-024) and GTx-758 (Capesaris®) programs;
- the therapeutic and commercial potential of our product candidates;
- our ability to establish and maintain potential new collaborative, partnering or other strategic arrangements for the development and commercialization of our product candidates;
- the anticipated progress of our clinical programs, including whether our ongoing and planned clinical trials of enobosarm and GTx-758 will achieve clinically relevant results;
- the timing, scope and anticipated initiation, enrollment and completion of our ongoing and planned clinical trials;
- the timing of potential regulatory discussions and submissions, and the anticipated timing, scope and outcome of related regulatory actions or guidance;
- 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;
- our ability to complete the private placement of our common stock and warrants to purchase common stock announced on November 10, 2014; and
- our estimates regarding the sufficiency of our cash resources 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,” “may,” “plans,” “potential,” “predicts,” “projects,” “should,” “will,” “would” and similar expressions intended to identify forward-looking statements. Forward-looking statements reflect our current views with respect to future events, are based on assumptions and are subject to risks, uncertainties and other important factors. We discuss many of these risks in this Quarterly Report on Form 10-Q in greater detail in the section entitled “Risk Factors” under Part II, Item 1A below. Given these risks, uncertainties and other important factors, you should not place undue reliance on these forward-looking statements. Also, forward-looking statements represent our estimates and assumptions only as of the date of this Quarterly Report on Form 10-Q. You should read this Quarterly Report on Form 10-Q and the documents that we incorporate by reference in and have filed as exhibits to this Quarterly Report on Form 10-Q, completely and with the understanding that our actual future results may be materially different from what we expect. Except as required by law, we assume no obligation to update any

Table of Contents

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

Overview

Business Overview

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

Our current strategy is focused on further development of enobosarm (GTx-024), our lead product candidate, in two breast cancer indications targeting the androgen receptor. Subject to the completion of the private placement of our common stock and warrants that we announced on November 10, 2014 and the receipt of necessary regulatory approvals, we plan to initiate two Phase 2 clinical trials for these indications in the first half of 2015. One of these Phase 2 clinical trials is designed to enroll patients with estrogen receptor, or ER, positive and androgen receptor, or AR, positive metastatic breast cancer, while the other Phase 2 clinical trial is designed to enroll patients with advanced AR positive triple-negative breast cancer, or TNBC. We are also continuing our Phase 2 clinical trial of GTx-758 as a secondary hormonal treatment for men with castration-resistant prostate cancer.

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

Business Highlights

We are developing selective androgen receptor modulators, or SARMs, a new class of drugs that we believe have the potential to be used as a hormonal therapy for the treatment of advanced breast cancer, as well as the potential to prevent and treat muscle wasting in patients with cancer and other musculoskeletal wasting or muscle loss conditions. Our lead product candidate, enobosarm, has to date been evaluated in 21 completed or ongoing clinical trials enrolling approximately 1,554 subjects, including in three Phase 2 and two Phase 3 clinical trials. Enobosarm is the generic name given to the compound by the USAN Council and the World Health Organization and is the first compound to receive the SARM stem in its name, recognizing enobosarm as the first in this new class of compounds.

We announced during the second quarter of 2014 positive preliminary results from an ongoing Phase 2 proof-of-concept, open-label clinical trial evaluating enobosarm 9 mg oral daily for the treatment of patients with ER positive and AR positive metastatic breast cancer who have previously responded to hormonal therapy. We believe that SARMs have the potential to be used as a novel 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. Moreover, steroidal androgens may also be converted to estrogens that could stimulate tumor growth. We believe that enobosarm has the potential to provide clinical benefit to women whose AR positive advanced breast cancer is progressing by treating their disease while minimizing the unwanted virilizing side effects associated with steroidal androgens, and unlike steroidal androgens, enobosarm cannot be converted into an estrogen. Additionally based on our prior clinical studies of enobosarm, we believe that women with AR positive advanced breast cancer receiving treatment with enobosarm may receive the benefit of increased lean body mass and reduction in fat mass.

In our ongoing Phase 2 proof-of-concept clinical trial in the U.S., we enrolled 22 postmenopausal women with ER positive metastatic breast cancer who have previously responded to hormonal therapy to assess clinical benefit response at six months of enobosarm 9 mg once daily treatment, which was defined as those patients receiving treatment who have demonstrated (i) a complete response (disappearance of all targeted lesions), (ii) a partial response (at least a 30 percent decrease in the sum of the diameters of the targeted lesions), or (iii) stable disease (no disease progression from baseline). The primary endpoint was assessed in 17 AR positive patients, including one patient who had AR status determined outside the protocol specified window. Six of these 17 patients demonstrated clinical benefit at six months, including the aforementioned patient, exceeding the pre-defined statistical threshold requiring that at least three of 14 patients with an AR positive metastatic lesion demonstrate clinical benefit. Seven patients in total (one patient with indeterminate AR status) achieved clinical benefit at six months as stable disease. The results also demonstrated that, after a median duration on study of 81 days, 41 percent of all patients (9/22)

achieved clinical benefit as best response and also had increased prostate specific antigen, or PSA, which appears to be an indicator of AR activity. No confirmed complete or partial responses have been observed in the study and one patient currently remains on study with stable disease. Enobosarm was well tolerated. The most common adverse events, or AEs, reported were pain, fatigue, nausea, hot flash/night sweats, and arthralgia. The majority of AEs were Grade 1. There were two serious adverse events, or SAEs, reported during the study. One of the SAEs, bone pain of the chest cage, was assessed as possibly related to enobosarm.

Based on the positive preliminary results from our Phase 2 proof-of-concept clinical trial in patients with ER positive and AR positive metastatic breast cancer, as well as positive data reported in medical literature regarding the use of androgens for the treatment of breast cancer and our preclinical data demonstrating tumor growth inhibition with enobosarm in animal models of disease, we believe enobosarm has the potential to be an effective treatment alternative with a favorable side effect profile for women with ER positive and AR positive metastatic breast cancer, as well as for women with advanced AR positive TNBC. Subject to the completion of the private placement of our common stock and warrants that we announced on November 10, 2014 and receipt of necessary regulatory approvals, we plan to initiate in the first half of 2015 two open-label Phase 2 clinical trials designed to evaluate the efficacy and safety of enobosarm in patients with AR positive advanced breast cancer. Our planned Phase 2 ER positive and AR positive metastatic breast cancer clinical trial is designed to enroll approximately 90 patients who have previously failed first line hormonal therapy. This planned open-label clinical trial is designed to randomize patients to either a 9 mg or 18 mg daily dose of enobosarm and will assess clinical benefit response after six months of daily treatment. We plan to conduct this clinical trial using a Simon's two-stage design, which in this case enrolls approximately half of the patients in the first stage, and, upon achievement of a pre-specified minimal response rate, proceeds with enrollment of the second stage.

Although the majority of breast cancers are determined to be hormone receptor positive (expressing ER, progesterone receptor (PR), or human epidermal growth factor receptor 2 (HER2)), 15% to 20% of women diagnosed with breast cancer will have TNBC which is characterized by a lack of expression of ER, PR, or HER2. TNBC occurs more frequently in younger patients (less than 50 years of age) and generally exhibits a more aggressive pattern of progression and has lower survival rates. For those patients with advanced TNBC, standard treatment options are limited to cytotoxic chemotherapy. However, even after an initial response to chemotherapy, the duration of the response may be short and there may be a higher likelihood of visceral metastases, rapidly progressing disease, and inferior survival compared to hormone receptor positive breast cancer. Therefore, a multitude of clinical institutes and advocacy organizations have put an emphasis on research focused on identifying therapeutic targets in TNBC. One such target is the androgen receptor. The androgen receptor is the most highly expressed steroid receptor in breast cancer with up to 95% of ER positive breast cancers expressing AR, and up to 35% of TNBC. Historically, the androgen receptor has been considered to be anti-proliferative and beneficial in hormone receptor positive breast cancers. In TNBC, data from peer-reviewed literature indicates that the presence of the androgen receptor and androgen synthesizing enzymes is associated with lower proliferation, lower tumor grade, better overall survival, and more favorable clinical outcomes, as compared to those patients with TNBC not expressing AR. The general consensus in current literature also suggests that the AR biomarker, PSA, is a favorable prognostic marker in breast cancer. Based on these findings, research has been focused on the androgen receptor as a potential therapeutic target and, historically, androgens have been used to treat breast cancer. We have studied SARMs in preclinical TNBC cell and animal models. This preclinical data suggests that the growth of TNBC cells expressing AR was inhibited by AR agonists, but not by the antagonist bicalutamide or structurally similar inactive molecules. In addition, the growth of TNBC tumors in animal models was inhibited by SARMs. We believe that this data, coupled with the early clinical success of androgens in breast cancer, supports the clinical evaluation of enobosarm as a novel targeted therapy to treat AR positive TNBC. Therefore, subject to the completion of our recently-announced private placement of our common stock and warrants and receipt of necessary regulatory approvals, we plan to initiate in the first half of 2015, a Phase 2 clinical trial designed to evaluate the efficacy and safety of enobosarm in patients with advanced AR positive TNBC. This planned proof-of-concept open-label trial is designed to enroll approximately 80 patients, who will be randomized to either a 9 mg or 18 mg daily dose of enobosarm and will assess clinical benefit response after four months of daily treatment. This clinical trial is also designed to be conducted using a Simon's two-stage design.

We announced in August 2013 that the POWER 1 (platinum plus taxane chemotherapy) and POWER 2 (platinum plus non-taxane chemotherapy) (Prevention and treatment Of muscle Wasting in patients with cancER) Phase 3 clinical trials evaluating enobosarm 3 mg daily for the prevention and treatment of muscle wasting in

patients with advanced non-small cell lung cancer, or NSCLC, failed to meet the statistical criterion for the co-primary endpoints of lean body mass and physical function that were assessed using responder analyses as pre-specified for the United States Food and Drug Administration, or FDA. However, efficacy data from the studies demonstrated enobosarm's consistent effect on maintaining or improving lean body mass compared to placebo. As for safety, enobosarm was generally well tolerated, with the occurrence of SAEs similar across the placebo and treated groups. A final analysis for survival was performed following 450 deaths in patients across both studies, which occurred in June 2014. As expected, enobosarm 3 mg did not adversely affect patient survival, as there were no statistical differences in survival between subjects in the placebo and enobosarm arms.

Enobosarm 3 mg demonstrated a statistically significant effect versus placebo on the primary endpoint of physical function through Day 84 in the POWER 1 clinical trial as assessed by continuous variable analysis, as pre-specified in our statistical analysis plan for the European Medicines Agency, or EMA. Therefore, we met with representatives from two member countries to the EMA to review and discuss the results of the POWER trials to determine the feasibility of submitting a marketing authorization application, or MAA, in the European Union, or EU, for enobosarm for the prevention and treatment of muscle wasting in patients with advanced NSCLC. Recently, we met again with the Medicines and Healthcare Products Regulatory Agency, or MHRA, which is the organization responsible for regulating all medicines and medical devices in the United Kingdom and is a member of the EMA, to review with them more detailed analyses of data from the POWER trials. Based on this meeting, we believe that data from the POWER trials is not sufficient to support the filing and approval of a MAA in the EU for enobosarm 3 mg for the prevention and treatment of muscle wasting in patients with advanced NSCLC without confirmatory data from another Phase 3 clinical trial for this indication. Therefore, we currently do not intend to file a MAA and will evaluate whether there is commercial rationale and partner interest to support additional clinical development required for approval. In our meeting with the FDA in February 2014 to review and discuss the results of the POWER clinical trials, we learned that since data from the POWER trials failed to meet the primary statistical criterion pre-specified for the co-primary endpoints of lean body mass and physical function, the FDA was not willing to accept a new drug application for enobosarm 3 mg for the prevention and treatment of muscle wasting in patients with advanced NSCLC. Our strategy does not currently include further development of enobosarm 3 mg for this indication in the U.S. or in Europe unless such development is part of a collaborative arrangement or strategic partnership. We continue to evaluate other potential indications in the U.S. for enobosarm.

Additionally, we are developing GTx-758 (Capesaris®), an oral nonsteroidal selective estrogen receptor alpha agonist, for secondary hormonal therapy in men with 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 testosterone without also causing certain estrogen deficiency side effects, such as bone loss and hot flashes, which are common with current androgen deprivation therapies for prostate cancer. We also believe that GTx-758 may be effective, in combination with ADT, as a secondary hormonal treatment of advanced prostate cancer by reducing 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.

We are currently conducting a Phase 2 open-label clinical trial in men who have developed metastatic or high risk non-metastatic CRPC while on ADT. GTx-758 has previously demonstrated the ability to increase the production of a protein called sex hormone binding globulin, or SHBG, that binds testosterone and thereby reduces free testosterone. By reducing free testosterone, we believe serum PSA will be reduced in men with CRPC. The primary endpoint of the current Phase 2 clinical trial is the proportion of subjects with a greater than or equal to 50% decline from baseline in serum PSA by day 90. Other key endpoints include serum SHBG and total and free testosterone levels, as well as prostate cancer progression, in the study subjects. In addition, the clinical trial is evaluating the ability of GTx-758 to treat certain estrogen deficiency side effects associated with luteinizing

Table of Contents

hormone releasing hormone agonists such as hot flashes and bone loss. The Phase 2 clinical trial is designed to 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 from the GTx-758 125 mg dosing arm indicating the ability of the drug to substantially increase SHBG and lower testosterone without any unexpected side effects occurring, the clinical trial protocol was amended in the third quarter of 2013 to eliminate the 500 mg dosing arm and to increase the number of subjects to be enrolled in the 125 mg and the 250 mg dosing arms to 38 patients per arm. Enrollment in the 125 mg cohort has been completed without any occurrence of VTEs, and after a pre-specified safety review by the Independent Data Safety Monitoring Board, we are now enrolling subjects in the 250 mg arm. Based on the safety and efficacy data observed in the 125 mg cohort and there being no unexpected side effects observed in the first ten metastatic patients enrolled in the 250 mg cohort, enrollment of the 250 mg cohort was opened to individuals with metastatic or high risk non-metastatic CRPC. To date, there has been one reported incidence of a VTE in a patient enrolled in the 250 mg cohort, resulting in the patient's discontinuation from active treatment. The study is ongoing and, subject to meeting our current enrollment projections, data from all patients in the study is expected early in the second quarter of 2015. After receipt of data from this clinical trial, we will evaluate potential next steps in the clinical development of GTx-758, including potentially seeking a partnering or collaborative arrangement in order to fund additional clinical development.

Financial Highlights

Our net loss for the three months ended September 30, 2014 was $4.9 million. We expect to incur significant operating losses for the foreseeable future as we continue our clinical development activities and potentially seek regulatory approval of our product candidates. We have funded our operations primarily through the sale of equity securities, collaboration and license agreements, and prior to September 2012, product revenue from sales of FARESTON®, the rights to which we sold to a third party in the third quarter of 2012. We currently have no ongoing collaborations for the development and commercialization of our product candidates and no source of revenue, nor do we expect to generate revenue for the foreseeable future. We do not expect to obtain FDA or EMA approval, or any other regulatory approvals, to market any of our product candidates, including enobosarm, for the foreseeable future, and it is possible that none of our product candidates will ever receive any regulatory approvals.

At September 30, 2014, we had cash, cash equivalents and short-term investments of $11.5 million compared to $14.7 million at December 31, 2013. On November 9, 2014, we entered into a securities purchase agreement providing for a private placement of units consisting of an aggregate of 64.3 million shares of our common stock and warrants to purchase an aggregate of 64.3 million shares of our common stock for an aggregate purchase price of $43.4 million. The closing of the private placement, which has not yet occurred, is subject to the satisfaction of customary closing conditions. See "Liquidity and Capital Resources" for more information on our recently-announced private placement. On March 6, 2014, we completed a prior private placement of units consisting of 11,976,048 shares of common stock and warrants to purchase 10,179,642 shares of our common stock for gross proceeds of approximately $21.3 million. The warrants, which have a one year term expiring on March 6, 2015, have a per share exercise price of $1.67 that is payable only in cash. If exercised in full, the warrants could result in additional gross proceeds of approximately $17.0 million.

We estimate that our current cash, cash equivalents and short-term investments, together with interest thereon, will be sufficient to meet our projected operating requirements only into the second quarter of 2015. While we estimate that the net proceeds from our recently-announced private placement, if consummated, would, together with our current cash, cash equivalents and short-term investments, be sufficient to fund our operations through 2016 (during which time, at a minimum, we expect to obtain stage one results from each of our planned open-label Phase 2 clinical trials of enobosarm in patients with AR positive advanced breast cancer), if we are unable to complete our recently-announced private placement on the anticipated terms or at all, we will be unable to initiate our planned Phase 2 clinical trials of enobosarm in patients with AR positive advanced breast cancer and our

Table of Contents

business would be substantially harmed. If we are unable to complete the private placement or raise additional funds in an alternative transaction in the near term in order to fund our operations through and beyond the second quarter of 2015 and to continue as a going concern, we would be required to, among other things, make further reductions in our workforce, abandon our plans to initiate our planned Phase 2 clinical trials of enobosarm in patients with AR positive advanced breast cancer, 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 severely harm our business and financial condition. Even if the private placement is consummated, we would need to obtain substantial additional funding to seek regulatory approval for enobosarm in patients with AR positive advanced breast cancer. In addition, if we decide to undertake any further development of GTx-758 and/or enobosarm for the prevention and treatment of muscle wasting in patients with NSCLC, we would need to obtain additional funding for such development, either through financing or by entering into collaborative arrangements or partnerships with third parties for such further development.

While we have been able to fund our operations to date, 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 also do not have any commitments for future external funding apart from those under our recently-announced private placement, the consummation of which has not yet occurred and is subject to the satisfaction of certain closing conditions. Until we can generate a sufficient amount of product revenue, which we may never do, we expect to finance future cash needs through potential collaboration, partnering or other strategic arrangements, as well as through public or private equity offerings or debt financings, or a combination of the foregoing. Our ability to raise additional funds and the terms upon which we are able to raise such funds have been severely harmed by the failure of the two enobosarm 3 mg Phase 3 clinical trials to meet both of the co-primary endpoints agreed upon with the FDA, and may in the future be adversely impacted by the uncertainty regarding the prospects of our planned development of enobosarm for the treatment of patients with AR positive advanced breast cancer and our ability to advance the development of enobosarm 3 mg and GTx-758, if at all. Our ability to raise additional funds and the terms upon which we are able to raise such funds may also be adversely affected by the uncertainties regarding our financial condition, the sufficiency of our capital resources, our ability to maintain the listing of our common stock on the NASDAQ Global Market and recent and potential future management turnover. As a result of these and other factors, we cannot be certain that additional funding will be available on acceptable terms, or at all. If adequate funds are not available when we need them, we may be required to delay, reduce the scope of or eliminate one or more of our research or development programs, including our enobosarm and GTx-758 programs, or conduct additional workforce or other expense reductions, any of which could have a material adverse effect on our business and our prospects.

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, supplies, and facilities associated with our research activities, screening and identification of product candidates, formulation and synthesis activities, manufacturing, preclinical studies, toxicology studies, clinical trials, regulatory and medical affairs activities, quality assurance activities and license fees. As a result of the October 2013 reduction in our workforce, we are no longer conducting drug discovery activities and we are focusing our research and development activities on the ongoing clinical development of our current product candidates.

We expect that our research and development expenses for fiscal year 2014 will decrease as compared to fiscal year 2013 due to the completion of the POWER 1 and POWER 2 clinical trials in 2013 and, for the remainder of the year, will be primarily focused on our ongoing Phase 2 clinical trials of enobosarm and GTx-758, and preparatory activities related to our two planned Phase 2 clinical trials of enobosarm for the treatment of women with AR positive advanced breast cancer.

Table of Contents

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

Product Candidates

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

Product Candidate/ Proposed Indication	Program	Clinical Development Phase	Status

Enobosarm Treatment of women with AR positive advanced breast cancer (9 mg and 18 mg)	SARM	Phase 2	Announced positive preliminary results from the Phase 2 proof-of-concept clinical trial in the second quarter of 2014. Subject to the completion of our recently-announced private placement and the receipt of necessary regulatory approvals, plan to initiate two Phase 2 clinical trials in two breast cancer indications targeting the androgen receptor in the first half of 2015.
Enobosarm Prevention and treatment of muscle wasting in patients with advanced NSCLC (3 mg)	SARM	Phase 3	No further development planned at this time.
GTx-758 Secondary hormonal therapy in men with metastatic and non-metastatic CRPC	Selective ER alpha agonist	Phase 2	Completed enrollment of the 125 mg cohort of the Phase 2 clinical trial for secondary hormonal therapy in men with metastatic CRPC and currently enrolling the 250 mg cohort in both metastatic and high risk non-metastatic CRPC.

General and Administrative Expenses

Our general and administrative expenses consist primarily of salaries and other related costs for personnel serving executive, finance, legal, human resources, information technology, and investor relations functions. General and administrative expenses also include facility costs, insurance costs, and professional fees for legal, accounting, and public relation services.

Table of Contents

Critical Accounting Policies and Significant Judgments and Estimates

Our management's discussion and analysis of our financial condition and results of operations is based on our condensed financial statements, which have been prepared in accordance with accounting principles generally accepted in the United States of America for interim financial statements. The preparation of these financial statements requires us to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of the condensed financial statements as well as the reported revenues and expenses during the reporting periods. On an ongoing basis, we evaluate our estimates and judgments related to revenue recognition, income taxes, intangible assets, long-term service contracts and other contingencies. We base our estimates on historical experience and on various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions.

While our significant accounting policies are more fully described in Note 2 to our financial statements appearing in our Annual Report on Form 10-K for the year ended December 31, 2013 filed with the SEC, we believe that the following accounting policies are most critical to aid you in fully understanding and evaluating our reported financial results.

Research and Development Expenses

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

Share-Based Compensation

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

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

Share-based compensation also includes, beginning October 2013, restricted stock units, or RSUs, granted to employees under our 2013 equity incentive plan. We estimated the fair value of RSUs using the closing price of our stock on the grant date. The fair value of RSUs was amortized on a straight-line basis over the requisite service period of the awards. All outstanding RSUs vested during the second quarter of 2014 and no RSUs were outstanding at September 30, 2014.

Table of Contents

The following table summarizes share-based compensation expense included within the condensed statements of operations for the three and nine months ended September 30, 2014 and 2013:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2014	2013	2014	2013
	(in thousands)		(in thousands)	
Research and development expenses	$ 89	$ 356	$ 2,419	$ 1,051
General and administrative expenses	177	406	1,861	1,281
Total share-based compensation	$ 266	$ 762	$ 4,280	$ 2,332

Share-based compensation expense recorded in the condensed statement of operations as general and administrative expense for both the three months ended September 30, 2014 and 2013 included share-based compensation expense related to deferred compensation arrangements for our non-employee directors of $31,000. Share-based compensation expense recorded in the condensed statement of operations as general and administrative expense for the nine months ended September 30, 2014 and 2013 included share-based compensation expense related to deferred compensation arrangements for our non-employee directors of $94,000 and $105,000, respectively. At September 30, 2014, the total compensation cost related to non-vested awards not yet recognized was approximately $4.2 million with a weighted average expense recognition period of 4.20 years.

FARESTON® Revenue Recognition

Although we sold our rights and certain assets related to FARESTON® effective September 30, 2012, we retain the liability for future product returns relating to sales of FARESTON® by us prior to September 30, 2012. Therefore, we estimate an accrual for product returns based on factors which include historical product returns and estimated product in the distribution channel which is expected to exceed its expiration date. At September 30, 2014 and December 31, 2013, our accrual for product returns was $141,000 and $918,000, respectively. The accrual for product returns decreased during the current period due to the closure of the return period for a portion of the previously sold inventory.

Recent Accounting Pronouncements

In August 2014, the Financial Accounting Standards Board issued Accounting Standard Update 2014-15, *Disclosure of Uncertainties about an Entity's Ability to Continue as a Going Concern*. The new guidance is intended to define management's responsibility to evaluate whether there is substantial doubt about an organization's ability to continue as a going concern within one year of the date the financial statements are issued and to provide related footnote disclosure. This new guidance is effective for the first annual period ending after December 15, 2016 and interim periods thereafter. The adoption of the standard update will not have an impact on our financial position or results of operations.

Results of Operations

Three and Nine Months Ended September 30, 2014 and 2013

Research and Development Expenses

The following table identifies the research and development expenses for each of our clinical product candidates, as well as research and development expenses pertaining to our other research and development efforts, for each of the periods presented. Research and development spending for past periods is not indicative of spending in future periods.

Table of Contents

Proposed Candidate / Proposed Indication	Program	Three Months Ended September 30,		Nine Months Ended September 30,	
		2014	2013	2014	2013
		(in thousands)			
Enobosarm Prevention and treatment of muscle wasting in patients with advanced non-small cell lung cancer (3 mg)	**SARM**	$ 1,909	$ 3,417	$ 11,686	$ 15,840
Enobosarm Treatment of women with ER positive and AR positive metastatic breast cancer (9 mg)	**SARM**	635	513	2,262	1,464
GTx-758 Secondary hormonal therapy in men with metastatic and non-metastatic CRPC	**Selective ER alpha agonist**	782	1,171	3,471	4,253
Other research and development		36	1,376	197	4,673
Total research and development expenses		$ 3,362	$ 6,477	$ 17,616	$ 26,230

Research and development expenses decreased to $3.4 million for the three months ended September 30, 2014 from $6.5 million for the three months ended September 30, 2013. Research and development expenses decreased to $17.6 million for the nine months ended September 30, 2014 from $26.2 million for the nine months ended September 30, 2013.

Research and development expenses for enobosarm 3 mg decreased for both periods as the last patients completed the POWER 1 and POWER 2 Phase 3 clinical trials for enobosarm 3 mg in May 2013. This was partially offset by increased activities related to satisfying the prerequisites necessary for our then-planned MAA submission for enobosarm 3 mg, including conducting seven Phase 1 clinical trials. Research and development expenses for enobosarm for the treatment of women with ER positive and AR positive metastatic breast cancer increased for both periods as we initiated in the second quarter of 2013 a Phase 2 proof-of-concept clinical trial evaluating a 9 mg daily dose of enobosarm for the treatment of ER positive and AR positive metastatic breast cancer in women who have previously responded to hormonal therapy. Research and development expenses related to GTx-758 decreased for both periods related to the ongoing Phase 2 clinical trial to evaluate GTx-758 as secondary hormonal therapy in men with metastatic CRPC due to the timing of patient activities and related management expenses as this trial was initiated in the third quarter of 2012.

"Other research and development" expenses include the cost of personnel, supplies and facilities associated with preclinical and discovery research and development activities and has decreased from the prior year comparable period as we are no longer conducting drug discovery activities and are focusing our research and development activities on the ongoing clinical development of our current product candidates.

General and Administrative Expenses

General and administrative expenses decreased 36% to $1.6 million for the three months ended September 30, 2014 from $2.5 million for the three months ended September 30, 2013. General and administrative expenses decreased 11% to $7.3 million for the nine months ended September 30, 2014 from $8.2 million for the nine months ended September 30, 2013. The decrease for the three and nine months ended September 30, 2014 from the prior period comparable quarter was due to a reduction in personnel costs as a result of the workforce reduction implemented in October 2013, a decrease in the accrual for product returns due to the closure of the return period for

Table of Contents

a portion of the previously sold inventory, and decreased legal fees. For the nine months ended September 2014, these decreases were partially offset by increases related to cash retention bonuses, stock options modifications, and stock option and RSU grants made to employees as part of our efforts to retain essential employees needed for us to continue our business operations following the October 2013 workforce reduction, as well as severance and stock option modifications related to the resignation of our CEO during the second quarter of 2014.

Liquidity and Capital Resources

At September 30, 2014, we had cash, cash equivalents and short-term investments of $11.5 million, compared to $14.7 million at December 31, 2013. Net cash used in operating activities was $23.7 million and $36.3 million for the nine months ended September 30, 2014 and 2013, respectively.

Net cash used in investing activities was $1.7 million for the nine months ended September 30, 2014 and resulted primarily from the purchase of short-term investments of $10.5 million offset by the maturities of short-term investments of $8.8 million. 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 financing activities was $20.5 million for the nine months ended September 30, 2014 and reflects proceeds from the issuance of common stock and warrants, partially offset by $617,000 of employee withholding tax payments related to vested RSUs and payments of $2,000 on capital lease obligations. Net cash provided by financing activities was $1.2 million for the nine months ended September 30, 2013 and was provided primarily from proceeds from the exercise of employee stock options of $1.2 million partially offset by payments on capital lease and financed equipment obligations of $5,000.

On November 9, 2014, we entered into a definitive securities purchase agreement with certain purchasers, including members of our management and Board of Directors, pursuant to which we agreed to issue and sell in a private placement to the purchasers, subject to customary closing conditions, an aggregate of 64.3 million immediately separable units, comprised of an aggregate of 64.3 million shares of common stock and warrants to purchase up to 64.3 million additional shares of common stock, for an aggregate purchase price of $43.4 million. The closing of the private placement is subject to the satisfaction of customary closing conditions. The warrants we agreed to issue at the closing of the private placement, which would have a per share exercise price of $0.85, would be subject to net cash settlement if at the time of any exercise, there are then an insufficient number of authorized and reserved shares of our common stock to effect a share settlement of the warrants. We do not currently have a sufficient number of authorized and unreserved shares of common stock necessary to settle exercises of the warrants in full in shares of common stock. Under the purchase agreement, we agreed to seek stockholder approval, at a special or annual meeting to be held no later than May 27, 2015, of an amendment to our Certificate of Incorporation to increase our authorized common stock to an amount necessary to effect the share settlement of all of the warrants we agreed to issue at the closing of the private placement. Assuming such approval is obtained, warrant exercises would no longer be subject to net cash settlement. If we are unable to obtain such stockholder approval, our obligation to net cash settle warrant exercises could be substantial and we are further obligated under the securities purchase agreement to maintain adequate funds in order to satisfy any such cash settlement obligations, which may require us to raise additional capital and in any event could adversely impact our liquidity in future periods. The warrants would generally become exercisable on the date such stockholder approval is obtained (but in no event later than June 1, 2015) and would continue to be exercisable for four years thereafter. The purchase agreement may be terminated by either us or the purchasers if the closing has not occurred by November 21, 2014 (subject to extension to November 28, 2014 under limited circumstances). If we are unable to consummate the private placement, our ability to execute on our stated business strategy could be precluded or, at a minimum, would be substantially impaired or delayed. This, in turn, would have a material adverse effect on our financial condition, business prospects and the price of our common stock.

We estimate that our current cash, cash equivalents and short-term investments, together with interest thereon, will be sufficient to meet our projected operating requirements only into the second quarter of 2015. While we estimate that the net proceeds from our recently-announced private placement, if consummated, would, together with our current cash, cash equivalents and short-term investments, be sufficient to fund our operations through 2016 (during which time, at a minimum, we expect to obtain stage one results from each of our planned open-label Phase 2 clinical trials of enobosarm in patients with AR positive advanced breast cancer), if we are unable to complete the private placement on the anticipated terms or at all, we will be unable to initiate our

planned Phase 2 clinical trials of enobosarm in patients with AR positive advanced breast cancer and our business would be substantially harmed. If we are unable to complete the private placement or raise additional funds in an alternative transaction in the near term in order to fund our operations through and beyond the second quarter of 2015 and to continue as a going concern, we would be required to, among other things, make further reductions in our

Table of Contents

workforce, abandon our plans to initiate our planned Phase 2 clinical trials of enobosarm in patients with AR positive advanced breast cancer, 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 severely harm our business and financial condition. Even if the private placement is consummated, we would need to obtain substantial additional funding to seek regulatory approval for enobosarm in patients with AR positive advanced breast cancer. In addition, if we decide to undertake any further development of GTx-758 and/or enobosarm for the prevention and treatment of muscle wasting in patients with NSCLC, we would need to obtain additional funding for such development, either through financing or by entering into collaborative arrangements or partnerships with third parties for such further development.

Our estimates of the period of time through which our financial resources or anticipated financial resources will be adequate to support our projected operating requirements and related development activities are forward-looking statements and involve 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. In addition, because of the numerous risks and uncertainties associated with the development of our product candidates and other research and development activities, we are unable to estimate with certainty the amounts of increased capital outlays and operating expenditures associated with our anticipated future clinical trials and other research and development activities. Our future funding requirements will depend on many factors, including:

- the scope, rate of progress and cost of our clinical development programs, including our ongoing, planned and any future clinical trials of our product candidates;
- 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, if any;
- the cost and timing of regulatory filings and/or approvals to commercialize our product candidates and any related restrictions, limitations, and/or warnings in the label of an approved product candidate;
- the effect of competing technological and market developments; and
- the cost of filing, prosecuting, defending and enforcing any patent claims and other intellectual property rights, and the cost of defending any other litigation claims.

While we have been able to fund our operations to date, we 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 also do not have any commitments for future external funding apart from those under our recently-announced private placement, the consummation of which has not yet occurred and is subject to the satisfaction of certain closing conditions. Until we can generate a sufficient amount of product revenue, which we may never do, we expect to finance future cash needs through potential collaboration, partnering or other strategic arrangements, as well as through public or private equity offerings or debt financings, or a combination of the foregoing. In October 2013, following our announcement that the POWER trials failed to achieve the results required by the FDA for us to file a new drug application, or NDA, for enobosarm, we announced a workforce reduction of approximately 60%. If we are unable to raise additional funds when needed, we may need to further reduce our expenditures, perhaps significantly, to preserve our cash. Cost-cutting measures that we may take in the future may not be sufficient to enable use to meet our cash requirements, and they may negatively affect our business and growth prospects.

To the extent that we raise additional funds through potential collaboration, partnering or other strategic arrangements, it may be necessary to relinquish rights to some of our technologies or product candidates, or grant licenses on terms that are not favorable to us, any of which could result in the stockholders of GTx having little or no continuing interest in our enobosarm and/or GTx-758 programs as stockholders or otherwise. To the extent we raise additional funds by issuing equity securities, our stockholders may experience significant dilution, particularly given our currently depressed stock price, and debt financing, if available, may involve restrictive covenants. For example, we completed a private placement of common stock and warrants in March 2014, which was substantially dilutive, and announced a subsequent private placement on November 10, 2014 that represents even greater dilution, and our stockholders may experience additional, perhaps substantial, dilution should we again raise additional funds by issuing equity securities. Any debt financing or additional equity that we raise may contain terms that are not favorable to us or our stockholders. Our ability to raise additional funds and the terms upon which we are able to raise such funds have been severely harmed by the failure of the two enobosarm Phase 3 clinical trials to meet both of the co-primary endpoints agreed upon with the FDA, and may in the future be adversely impacted by the uncertainty regarding the prospects of our planned development of enobosarm for the treatment of patients with AR positive advanced breast cancer and our ability to advance the development of enobosarm 3 mg and GTx-758, if at all. Our ability to raise additional funds and the terms upon which we are able to raise such funds may also be adversely affected by the uncertainties regarding our financial condition, the sufficiency of our capital resources, our ability to maintain the listing of our common stock on the NASDAQ Global Market and recent and potential future management turnover. As a result of these and other factors, we cannot be certain that additional funding will be available on acceptable terms, or at all. If adequate funds are not available when we need them, we may be required to delay, reduce the scope of or eliminate one or more of our research or development programs, including our enobosarm and GTx-758 programs, or conduct additional workforce or other expense reductions, any of which could have a material adverse effect on our business and our prospects.

NASDAQ Listing Compliance

On October 2, 2014, we received a letter from NASDAQ notifying us that for the previous 30 consecutive business days, the closing bid price for our common stock was below the minimum $1.00 per share requirement for continued listing on The NASDAQ Global Market, or the Bid Price Requirement. The notification had no immediate effect on the listing of our common stock.

In accordance with NASDAQ listing rules, we have been afforded 180 calendar days, or until March 31, 2015, to regain compliance. To regain compliance, the closing bid price of our common stock must be at least $1.00 per share for a minimum of ten consecutive business days at any time before March 31, 2015. If we do not demonstrate compliance with the Bid Price Requirement by March 31, 2015, we may be eligible for an additional 180 calendar

Table of Contents

day compliance period. To qualify, we would need to transfer the listing of our common stock to The NASDAQ Capital Market, provided that we meet The NASDAQ Capital Market initial inclusion criteria at that time, with the exception of the Bid Price Requirement, and would need to provide written notice of our intention to cure the deficiency during this second compliance period, by effecting a reverse stock split, if necessary. However, if it appears to the NASDAQ staff that we will not be able to cure the deficiency, or if we are otherwise not eligible, NASDAQ would notify us that our common stock would be subject to delisting. In the event of such a notification, we may appeal the NASDAQ's staff determination to delist our common stock, but there can be no assurance that any such appeal would be successful. In addition, we may be unable to meet other applicable NASDAQ listing requirements, including maintaining minimum levels of stockholders' equity or market values of our common stock in which case, our common stock could be delisted notwithstanding our ability to demonstrate compliance with the Bid Price Requirement. If our common stock is delisted, this would, among other things, substantially impair our ability to raise additional funds and could result in a loss of institutional investor interest and fewer development opportunities for us.

ITEM 3. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK

During the nine months ended September 30, 2014, there were no material changes to our market risk disclosures as set forth in Part II, Item 7A of our Annual Report on Form 10-K for the year ended December 31, 2013.

ITEM 4. CONTROLS AND PROCEDURES

We maintain disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) of the Securities and Exchange Act of 1934, as amended (the "Exchange Act")) that are designed to ensure that information required to be disclosed in the reports that we file or submit under the Exchange Act is recorded, processed, summarized, and reported within the time periods specified in the SEC's rules and forms and that such information is accumulated and communicated to our management, including our Principal Executive Officer and Principal Financial Officer, as appropriate, to allow for timely decisions regarding required disclosures.

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

There were no changes in our internal control over financial reporting during the third quarter of 2014 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 12, 2014.

Table of Contents

Risks Related to Our Financial Condition and Need for Additional Financing

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

As of September 30, 2014, we had an accumulated deficit of $480.2 million. Our net loss for the nine months ended September 30, 2014 was $24.9 million. We expect to incur significant operating losses for the foreseeable future as we continue our clinical development activities and potentially seek regulatory approval of our product candidates. These losses, among other things, have had and will continue to have an adverse effect on our stockholders' equity and working capital.

Our current product candidates, enobosarm (GTx-024) and GTx-758 (Capesaris®), will require significant additional clinical development, financial resources and personnel in order to obtain necessary regulatory approvals for these product candidates and to develop them into commercially viable products. A substantial portion of our efforts and expenditures have been devoted to enobosarm 3 mg, which was the subject of our POWER 1 and POWER 2 Phase 3 clinical trials for the prevention and treatment of muscle wasting in patients with advanced non-small cell lung cancer, or NSCLC. The failure of the POWER trials to meet the primary statistical criterion for the co-primary endpoints agreed upon with the FDA significantly depressed our stock price and has harmed our future prospects. Although we have been evaluating the potential submission of a marketing authorization application, or MAA, to the European Medicines Agency, or EMA, seeking marketing approval of enobosarm 3 mg in the European Union, or EU, for the prevention and treatment of muscle wasting in patients with advanced NSCLC, based on recent input from the Medicines and Healthcare Products Regulatory Agency, or MHRA, we believe that the data from the POWER trials is not sufficient to support the filing and approval of a MAA without confirmatory data from another Phase 3

clinical trial of enobosarm 3 mg. As a result of this input, we do not intend to proceed with the submission of a MAA in the absence of such confirmatory data. In addition, since data from the two POWER trials failed to meet the primary statistical criterion pre-specified for the co-primary endpoints of lean body mass and physical function, the FDA will not accept a new drug application for enobosarm 3 mg for the prevention and treatment of muscle wasting in patients with advanced NSCLC. Accordingly, our strategy does not currently include further development of enobosarm for this indication in the U.S. or in Europe unless such development is part of a collaborative arrangement or strategic partnership. In the event we are unable to secure such arrangements, which we may never do, we could abandon further development of our enobosarm 3 mg program and forego any return on our investment from our enobosarm 3 mg program. Moreover, our current strategy is focused on the further development of enobosarm for the treatment of patients with androgen receptor, or AR, positive advanced breast cancer. However, the development of enobosarm for the treatment of patients with AR positive advanced breast cancer is at an early stage and is subject to the substantial risk of failure inherent in the development of early-stage product candidates.

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. In addition, we do not expect to obtain FDA or EMA approval, or any other regulatory approvals, to market any of our product candidates, including enobosarm, for the foreseeable future, and it is possible that none of our product candidates will ever receive any regulatory approvals.

We have funded our operations primarily through public offerings and private placements of our securities, as well as payments from our former collaborators. We also previously recognized product revenue from the sale of FARESTON®, the rights to which we sold to a third party in the third quarter of 2012. Currently, we have no ongoing collaborations for the development and commercialization of our product candidates, and as a result of the sale of our rights and certain assets related to FARESTON®, we also currently have no sources of revenue.

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

We may be unable to complete our recently-announced private placement of common stock and warrants, which could preclude us from executing on our stated business strategy.*

Table of Contents

On November 9, 2014, we entered into a securities purchase agreement providing for a private placement of units consisting of an aggregate 64.3 million shares of our common stock and warrants to purchase an aggregate of 64.3 million shares of our common stock for an aggregate purchase price of $43.4 million. The closing of the private placement, which has not yet occurred, is subject to the satisfaction of customary closing conditions. If we are unable to satisfy, or cause to be satisfied, the conditions to the consummation of the private placement, the securities purchase agreement could be terminated in which case, we would not receive any of the anticipated net proceeds from the private placement. In such event, we would not have funds sufficient to initiate our planned Phase 2 clinical trials of enobosarm in patients with AR positive advanced breast cancer and therefore our ability to execute on our stated business strategy could be precluded or, at a minimum, would be substantially impaired or delayed. This, in turn, would have a material adverse effect on our financial condition, business prospects and the price of our common stock.

We will need to raise substantial additional capital and may be unable to raise capital when needed, which would force us to delay, reduce or eliminate our product development programs and could cause us to discontinue our operations.*

We will need to raise substantial additional capital to:

- fund our operations and conduct clinical trials;
- continue our research and development;
- seek regulatory approval for our product candidates; and
- commercialize our product candidates, if any such product candidates receive regulatory approval for commercial sale.

We estimate that our current cash, cash equivalents and short-term investments, together with interest thereon, will be sufficient to meet our projected operating requirements only into the second quarter of 2015. While we estimate that the net proceeds from our private placement, if consummated, would, together with our current cash, cash equivalents and short-term investments, be sufficient to fund our operations through 2016, if we are unable to complete the private placement on the anticipated terms or at all, we will be unable to initiate our planned Phase 2 clinical trials of enobosarm in patients with AR positive advanced breast cancer, our business would be substantially harmed. If we are unable to complete the private placement or raise additional funds in an alternative transaction in the near term in order to fund our operations through and beyond the second quarter of 2015 and to continue as a going concern, we would be required to, among other things, make further reductions in our workforce, abandon our plans to initiate our planned Phase 2 clinical trials of enobosarm in patients with AR positive advanced breast cancer, 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 severely harm our business and financial condition. Even if the private placement is consummated, we would need to obtain substantial additional funding to seek regulatory approval for enobosarm in patients with AR positive advanced breast cancer. In addition, if we decide to undertake any further development of GTx-758 and/or enobosarm for the prevention and treatment of muscle wasting in patients with NSCLC, we would need to obtain additional funding for such development, either through financing or by entering into collaborative arrangements or partnerships with third parties for such further development.

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, planned and any future clinical trials of our product candidates;
- 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;

Table of Contents

- future clinical trial results, if any;
- the cost and timing of regulatory filings and/or approvals to commercialize our product candidates and any related restrictions, limitations, and/or warnings in the label of an approved product candidate;
- the effect of competing technological and market developments; and
- the cost of filing, prosecuting, defending and enforcing any patent claims and other intellectual property rights, and the cost of defending any other litigation claims.

While we have been able to fund our operations to date, we 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 also do not have any commitments for future external funding apart from those under our recently-announced private placement, the consummation of which has not yet occurred and is subject to the satisfaction of certain closing conditions. Until we can generate a sufficient amount of product revenue, which we may never do, we expect to finance future cash needs through potential collaboration, partnering or other strategic arrangements, as well as through public or private equity offerings or debt financings, or a combination of the foregoing. In October 2013, following our announcement that the POWER trials failed to achieve the results required by the FDA for us to file a new drug application, or NDA, for enobosarm, we announced a workforce reduction of approximately 60%. If we are unable to raise additional funds when needed, we may need to further reduce our expenditures, perhaps significantly, to preserve our cash. Cost-cutting measures that we may take in the future may not be sufficient to enable use to meet our cash requirements, and they may negatively affect our business and growth prospects.

To the extent that we raise additional funds through potential collaboration, partnering or other strategic arrangements, it may be necessary to relinquish rights to some of our technologies or product candidates, or grant licenses on terms that are not favorable to us, any of which could result in the stockholders of GTx having little or no continuing interest in our enobosarm and/or GTx-758 programs as stockholders or otherwise. To the extent we raise additional funds by issuing equity securities, our stockholders may experience significant dilution, particularly given our currently depressed stock price, and debt financing, if available, may involve restrictive covenants. For example, we completed a private placement of common stock and warrants in March 2014, which was substantially dilutive, and announced a subsequent private placement on November 10, 2014 that represents even greater dilution, and our stockholders may experience additional, perhaps substantial, dilution should we again raise additional funds by issuing equity securities. Any debt financing or additional equity that we raise may contain terms that are not favorable to us or our stockholders. Our ability to raise additional funds and the terms upon which we are able to raise such funds have been severely harmed by the failure of the two enobosarm Phase 3 clinical trials to meet both of the co-primary endpoints agreed upon with the FDA, and may in the future be adversely impacted by the uncertainty regarding the prospects of our planned development of enobosarm for the treatment of patients with AR positive advanced breast cancer and our ability to advance the development of enobosarm 3 mg and GTx-758, if at all. Our ability to raise additional funds and the terms upon which we are able to raise such funds may also be adversely affected by the uncertainties regarding our financial condition, the sufficiency of our capital resources, our ability to maintain the listing of our common stock on the NASDAQ Global Market and recent and potential future management turnover. As a result of these and other factors, we cannot be certain that additional funding will be available on acceptable terms, or at all. If adequate funds are not available when we need them, we may be required to delay, reduce the scope of or eliminate one or more of our research or development programs, including our enobosarm and GTx-758 programs, or conduct additional workforce or other expense reductions, any of which could have a material adverse effect on our business and our prospects.

Risks Related to Development of Product Candidates

We are substantially dependent on the success of enobosarm and our failure to advance the development of enobosarm or to obtain regulatory approval of enobosarm would significantly harm our prospects.*

Our current strategy is focused on the further development of enobosarm for the treatment of patients with AR positive advanced breast cancer. However, the development of enobosarm for the treatment of patients with AR positive advanced breast cancer is at an early stage and is subject to the significant risk of failure inherent in the development of early-stage product candidates. Moreover, we still have only limited data from our preclinical

Table of Contents

models of breast cancer and our Phase 2 clinical proof-of-concept clinical trial of enobosarm in women with ER positive and AR positive metastatic breast cancer. As a result, we will need to conduct additional clinical trials of enobosarm for the treatment of patients with AR positive advanced breast cancer to determine whether enobosarm is an effective treatment for patients with AR positive advanced breast cancer. Additionally, if it is determined that enobosarm treatment is not superior to existing approved therapies for advanced breast cancer, or has an unacceptable safety profile, any regulatory approval of enobosarm could be denied or significantly delayed in this patient population.

Preclinical studies, including studies of SARMs in animal models of disease, may not accurately predict the results of subsequent human clinical trials of enobosarm, including the results our two planned Phase 2 clinical trials of enobosarm in patients with AR positive advanced breast cancer. Furthermore, the positive preliminary results of our Phase 2 clinical proof-of-concept clinical trial of enobosarm in women with ER positive and AR positive metastatic breast cancer does not ensure that our two planned Phase 2 clinical trials will be successful or that any later trials will be successful. A number of companies in the pharmaceutical industry, including us and those with greater resources and experience than we have, have suffered significant setbacks in Phase 3 and later-stage clinical trials, even after receiving encouraging results in earlier clinical trials. Due to the uncertain and time-consuming clinical development and regulatory approval process, we may not be successful in developing enobosarm for the treatment of patients with AR positive advanced breast cancer, or in developing any of our product candidates, and it is possible that none of our current product candidates will ever become commercial products.

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

statistically using responder analyses as required by the FDA. The failure of the POWER trials to meet the primary statistical criterion for the co-primary endpoints agreed upon with the FDA significantly depressed our stock price and has harmed our future prospects. Although we have been evaluating the potential submission of a MAA to the EMA seeking marketing approval of enobosarm 3 mg in the EU for the prevention and treatment of muscle wasting in patients with advanced NSCLC, based on recent input from the MHRA, we believe that the data from the POWER trials is not sufficient to support the filing and approval of a MAA without confirmatory data from another Phase 3 clinical trial of enobosarm 3 mg. As a result of this input, we do not intend to proceed with the submission of a MAA in the absence of such confirmatory data. In addition, since data from the two POWER trials failed to meet the primary statistical criterion pre-specified for the co-primary endpoints of lean body mass and physical function, the FDA will not accept a new drug application for enobosarm 3 mg for the prevention and treatment of muscle wasting in patients with advanced NSCLC. Accordingly, our strategy does not currently include further development of enobosarm for this indication in the U.S. or in Europe unless such development is part of a collaborative arrangement or strategic partnership. In the event we are unable to secure such arrangements, which we may never do, we could abandon further development of our enobosarm 3 mg program and forego any return on our investment from our enobosarm 3 mg program.

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

Significant additional clinical development and financial resources will be required to obtain necessary regulatory approvals for our product candidates and to develop them into commercially viable products. Preclinical and clinical testing is expensive, can take many years to complete and has an uncertain outcome. Success in preclinical testing and early clinical trials does not ensure that later clinical trials will be successful, and interim results of a clinical trial do not necessarily predict final results. Typically, the failure rate for development candidates is high. If a product candidate fails at any stage of development, we will not have the anticipated revenues from that product candidate to fund our operations, and we will not receive any return on our investment in that product candidate. For example, we announced in August 2013 that our POWER 1 and POWER 2 Phase 3 clinical trials evaluating enobosarm for the prevention and treatment of muscle wasting in patients with advanced NSCLC failed to meet the co-primary endpoints of lean body mass and physical function that were assessed statistically using responder analyses as agreed upon with the FDA. Although we have been evaluating the potential submission of a MAA to the EMA seeking marketing approval of enobosarm 3 mg in the EU for the prevention and

treatment of muscle wasting in patients with advanced NSCLC, based on recent input from the MHRA, we believe that the data from the POWER trials is not sufficient to support the filing and approval of a MAA without confirmatory data from another Phase 3 clinical trial of enobosarm 3 mg. As a result of this input, we do not intend to proceed with the submission of a MAA in the absence of such confirmatory data. In addition, since data from the two POWER trials failed to meet the primary statistical criterion pre-specified for the co-primary endpoints of lean body mass and physical function, the FDA will not accept a new drug application for enobosarm 3 mg for the prevention and treatment of muscle wasting in patients with advanced NSCLC. Accordingly, our strategy does not currently include further development of enobosarm for this indication in the U.S. or in Europe unless such development is part of a collaborative arrangement or strategic partnership. In the event we are unable to secure such arrangements, which we may never do, we could abandon further development of our enobosarm 3 mg program and forego any return on our investment from our enobosarm 3 mg program.

Significant delays in clinical testing could materially impact our product development costs. We do not know whether planned 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. In this regard, there has been one reported incidence of a VTE in a patient enrolled in the 250 mg cohort of our ongoing Phase 2 clinical trial of GTx-758, resulting in his discontinuation from active treatment, and we cannot assure you that we will not observe an unacceptable incidence of VTEs in this 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

Table of Contents

to find an appropriate dose that is both effective and safe for these patient populations. If an unacceptable incidence of venous thromboembolic events or other adverse events are observed in our current Phase 2 clinical trial of GTx-758, we may be required to abandon our development of GTx-758, in which case, we would not receive any return on our investment in that product candidate.

In our Phase 2 clinical trials for enobosarm for the treatment of muscle wasting in patients with cancer and healthy older males and postmenopausal females, we observed mild elevations of hepatic enzymes, which in certain circumstances may lead to liver failure, in a few patients in both the placebo and enobosarm treated groups. Reductions in high-density lipoproteins have also been observed in subjects treated with enobosarm. Lower levels of high-density lipoproteins could lead to increased risk of adverse cardiovascular events. In addition, in our Phase 2 proof-of-concept clinical trial evaluating enobosarm in a 9 mg daily dose for the treatment of patients with ER positive and AR positive metastatic breast cancer, a serious adverse event, bone pain of the chest cage, was assessed as possibly related to enobosarm. Alternations of bone health, including those potentially caused by hormonal therapies such as enobosarm, could lead to a skeletal-related event which is associated with multiple complications, could negatively affect quality of life and could reduce survival. Complications potentially associated with skeletal-related events include, but are not limited to, pathologic fractures and spinal cord compression, and surgery or radiation for palliation and treatment.

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 3 mg and GTx-758 programs if we are unable to raise sufficient funding for any additional clinical development of these product candidates through new collaborative arrangements with third parties or other financing alternatives. In this regard, if we decide to undertake any further development of GTx-758 and/or enobosarm for the prevention and treatment of muscle wasting in patients with

Table of Contents

NSCLC, we would need to obtain additional funding for such development, either through financing or by entering into collaborative arrangements or partnerships with third parties for any such further development. There can be no assurances that we will be successful in obtaining additional funding in any event. If we do not have sufficient funds, we will not be able to advance the development of our product candidates or otherwise 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 collaborative arrangements may place the development and commercialization of our product candidates

outside our control, may require us to relinquish important rights or may otherwise be on terms unfavorable to us.

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

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

- we may not be able to control the amount and timing of resources that our potential collaborators may devote to our product candidates;
- potential collaborations may experience financial difficulties or changes in business focus;
- we may be required to relinquish important rights such as marketing and distribution rights;
- should a collaborator fail to develop or commercialize one of our compounds or product candidates, we may not receive any future milestone payments and will not receive any royalties for the compound or product candidate;
- business combinations or significant changes in a collaborator's business strategy may also adversely affect a collaborator's willingness or ability to complete its obligations under any arrangement;
- under certain circumstances, a collaborator could move forward with a competing product candidate developed either independently or in collaboration with others, including our competitors; and
- collaborative arrangements are often terminated or allowed to expire, which could delay the development and may increase the cost of developing our product candidates.

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

Table of Contents

the FDA of an alternate supplier, would delay or prevent the clinical development and commercialization of these product candidates.

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

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

- reliance on the third party for regulatory compliance and quality assurance;
- the possible breach of the manufacturing agreement by the third party because of factors beyond our control;
- the possible termination or non-renewal of the agreement by the third party, based on its own business priorities, at a time that is costly or inconvenient for us; and
- drug product supplies not meeting the requisite requirements for clinical trial use.

If we are not able to obtain adequate supplies of our product candidates, it will be more difficult for us to develop our product candidates and compete effectively. Our product candidates and any products that we and/or our potential collaborators may develop may compete with other product candidates and products for access to manufacturing facilities.

Our present or future manufacturing partners may not be able to comply with FDA-mandated current Good Manufacturing Practice regulations, other FDA regulatory requirements or similar regulatory requirements outside the United States. Failure of our third-party manufacturers or us to comply with applicable regulations could result in sanctions being imposed on us, including fines, injunctions, civil penalties, failure of regulatory authorities to grant marketing approval of our product candidates, delays, suspension or withdrawal of approvals, license revocation, seizures or recalls of product candidates or products, operating restrictions and criminal prosecutions, any of which could significantly and adversely affect supplies of our product candidates.

If third parties on whom we rely do not perform as contractually required or expected, we may not be able to obtain regulatory approval for or successfully commercialize our product candidates.

We do not have the ability to independently conduct clinical trials for our product candidates, and we must rely on third parties, such as contract research organizations, or CROs, medical institutions, clinical investigators and contract laboratories to conduct our clinical trials. In addition, we rely on third parties to assist with our preclinical development of product candidates. If these third parties do not successfully carry out their contractual duties or regulatory obligations or meet expected deadlines, if the third parties need to be replaced, or if the quality or accuracy of the data they obtain is compromised due to the failure to adhere to our clinical protocols or regulatory requirements or for other reasons, our preclinical development activities or clinical trials may be extended, delayed, suspended or terminated, and we may not be able to obtain regulatory approval for or successfully commercialize our product candidates.

Risks Related to Our Intellectual Property

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

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

Table of Contents

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

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

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

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

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

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

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

There is a risk that we are infringing the proprietary rights of third parties because numerous United States and

foreign issued patents and pending patent applications, which are owned by third parties, exist in the fields that are the focus of our development and manufacturing efforts. Others might have been the first to make the inventions covered by each of our or our licensors' pending patent applications and issued patents and/or might have been the first to file patent applications for these inventions. In addition, because patent applications can take many years to issue, there may be currently pending applications, unknown to us or our licensors, which may later result in issued patents that cover the production, manufacture, synthesis, commercialization, formulation or use of our product candidates. In addition, the production, manufacture, synthesis, commercialization, formulation or use of our product candidates may infringe existing patents of which we are not aware. Defending ourselves against third-party claims, including litigation in particular, would be costly and time consuming and would divert management's attention from our business, which could lead to delays in our development or commercialization efforts. If third parties are successful in their claims, we might have to pay substantial damages or take other actions that are adverse to our business.

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

- be prohibited from selling or licensing any product that we and/or any potential collaborators may develop unless the patent holder licenses the patent to us, which the patent holder is not required to do;
- be required to pay substantial royalties or other amounts, or grant a cross license to our patents to another patent holder; or
- be required to redesign the formulation of a product candidate so that it does not infringe, which may not be possible or could require substantial funds and time.

Risks Related to Regulatory Approval of Our Product Candidates

If we or any potential collaborators are not able to obtain required regulatory approvals, we or such collaborators will not be able to commercialize our product candidates, and our ability to generate revenue will be materially impaired.*

Our product candidates and the activities associated with their development and commercialization are subject to comprehensive regulation by the FDA, other regulatory agencies in the United States and by comparable authorities in other countries, including the EMA. Failure to obtain regulatory approval for a product candidate will prevent us or any potential collaborator from commercializing the product candidate. We have not received regulatory approval to market any of our product candidates in any jurisdiction, and we do not expect to obtain FDA, EMA or any other regulatory approvals to market any of our product candidates for the foreseeable future, if at all. The process of obtaining regulatory approvals is expensive, often takes many years, if approval is obtained at all, and can vary substantially based upon the type, complexity and novelty of the product candidates involved.

Changes in the regulatory approval policy during the development period, changes in or the enactment of additional regulations or statutes, or changes in regulatory review for each submitted product application may cause delays in the approval or rejection of an application. Even if the FDA or the EMA approves a product candidate, the approval may impose significant restrictions on the indicated uses, conditions for use, labeling, advertising, promotion, marketing and/or production of such product, and may impose ongoing requirements for post-approval studies, including additional research and development and clinical trials. Any FDA approval may also impose risk evaluation mitigation strategies, or REMS, on a product if the FDA believes there is a reason to monitor the safety of the drug in the market place. REMS may include requirements for additional training for health care professionals, safety communication efforts and limits on channels of distribution, among other things. The sponsor would be required to evaluate and monitor the various REMS activities and adjust them if need be. The FDA and EMA also may impose various civil or criminal sanctions for failure to comply with regulatory requirements, including withdrawal of product approval.

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

The FDA, the EMA and other foreign regulatory authorities have substantial discretion in the approval process and may refuse to accept any application or may decide that our data is insufficient for approval and require additional preclinical, clinical or other studies. For example, in October 2009, we received a Complete Response Letter from the FDA regarding our NDA for toremifene 80 mg to reduce fractures in men with prostate cancer on ADT notifying us that the FDA would not approve our NDA as a result of certain clinical deficiencies identified in the Complete Response Letter. We have since discontinued our toremifene 80 mg development program, as well as our other toremifene-based products and terminated our license and supply agreement with Orion for toremifene products. Although we have been evaluating the potential submission of a MAA to the EMA seeking marketing approval of enobosarm 3 mg in the EU for the prevention and treatment of muscle wasting in patients with advanced NSCLC, based on recent input from the MHRA, we believe that the data from the POWER trials is not sufficient to support the filing and approval of a MAA without confirmatory data from another Phase 3 clinical trial of enobosarm 3 mg. As a result of this input, we do not intend to proceed with the submission of a MAA in the absence of such confirmatory data. In addition, since data from the two POWER trials failed to meet the primary statistical criterion pre-specified for the co-primary endpoints of lean body mass and physical function, the FDA will not accept a new drug application for enobosarm 3 mg for the prevention and treatment of muscle wasting in patients with advanced NSCLC. Accordingly, our strategy does not currently include further development of enobosarm for this indication in the U.S. or in Europe unless such development is part of a collaborative arrangement or strategic partnership. In the event we are unable to secure such arrangements, which we may never do, we could abandon further development of our enobosarm 3 mg program and forego any return on our investment from our enobosarm 3 mg program.

Additionally, there can be no assurance that the FDA will determine that the data from our ongoing, planned or potential future clinical trials of enobosarm for the treatment of patients with AR positive advanced breast cancer or GTx-758 will be sufficient for approval of these product candidates in any indications. For example, we may

observe an unacceptable incidence of adverse events in our ongoing, planned or potential clinical trials of enobosarm or GTx-758, which could require us to abandon the development of the affected product candidate.

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

We do not expect to receive regulatory approval for the commercial sale of any of our product candidates that are in development for the foreseeable future, if at all. The inability to obtain approval from the FDA, the EMA and other foreign regulatory authorities for our product candidates would prevent us or any potential collaborators from commercializing these product candidates in the United States, the EU, or other countries. See the section entitled "Business — Government Regulation" under Part 1, Item 1 of our Annual Report on Form 10-K, filed with the SEC on March 12, 2014, for additional information regarding risks associated with marketing approval, as well as risks related to potential post-approval requirements.

Risks Related to Commercialization

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, including enobosarm, may not gain market acceptance for its stated indication among physicians, patients, health care payors and the medical community. If these products do not achieve an adequate level of acceptance, we may not generate material product revenues or receive royalties to the extent we currently anticipate, and we may not become profitable. The degree of market acceptance of our product candidates, if approved for commercial sale, will depend on a number of factors, including:

- efficacy and safety results in clinical trials;
- the prevalence and severity of any side effects;
- potential advantages over alternative treatments;
- whether the products we commercialize remain a preferred course of treatment;
- the ability to offer our product candidates for sale at competitive prices;

Table of Contents

- relative convenience and ease of administration;
- the strength of marketing and distribution support; and
- sufficient third-party coverage or reimbursement.

For example, if we are able to raise sufficient funding for any additional clinical development of enobosarm 3 mg through new collaborative arrangements with third parties or other financing alternatives and a MAA is submitted to the EMA for the marketing approval of enobosarm 3 mg in the EU for the more narrow indication of the prevention and treatment of muscle wasting in patients with advanced NSCLC treated with platinum plus taxane chemotherapy and marketing approval is obtained, we anticipate that the commercial prospects for enobosarm 3 mg could be diminished as a result of this more limited product indication.

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

Table of Contents

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

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

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

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

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

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

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

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

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

Table of Contents

- costs to defend the related litigation;
- substantial monetary awards to trial participants or patients;
- loss of revenue; and
- the inability to commercialize any products for which we obtain or hold marketing approvals.

We have product liability insurance that covers our clinical trials and any commercial products up to a $20 million annual aggregate limit. Insurance coverage is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost, and we may not be able to obtain insurance coverage that will be adequate to satisfy any liability that may arise.

If our competitors are better able to develop and market products than any products that we and/or any potential collaborators may develop, our commercial opportunity will be reduced or eliminated.*

We face competition from commercial pharmaceutical and biotechnology enterprises, as well as from academic institutions, government agencies and private and public research institutions. Our commercial opportunities will be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer side effects or are less expensive than any products that we and/or any potential collaborators may develop. Competition could result in reduced sales and pricing pressure on our product candidates, if approved, which in turn would reduce our ability to generate meaningful revenue and have a negative impact on our results of operations. In addition, significant delays in the development of our product candidates could allow our competitors to bring products to market before us and impair any ability to commercialize our product candidates.

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

With respect to our SARM program, there are other SARM product candidates in development that may compete with enobosarm and any future SARM product candidates, if approved for commercial sale. Competition for enobosarm 3 mg, which is being developed for the prevention and treatment of muscle wasting in patients with advanced NSCLC, includes SARMs in development by 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 3 mg 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 also plan to advance the development of enobosarm for the treatment of patients with ER positive and AR positive metastatic breast cancer. No other SARMs are currently in development for this indication; although SARMs in development for muscle wasting and cachexia could enter into a breast cancer program in the future. For example, Radius Health, Inc. has stated that it may test its compound, RAD140, in a breast cancer indication in the future. A number of other compounds targeting the androgen axis in breast cancer could compete with enobosarm if one or more are approved for commercial sale in the indications for which enobosarm is being developed. These compounds fall into two categories, androgen synthesis inhibitors, or ASIs, and androgen receptor antagonists, or ARAs. ASIs in development include orteronel being developed by Takeda Pharmaceuticals and Zytiga® being developed by Janssen Pharmaceuticals. ARAs in development include XTANDI® (enzalutamide) being developed by Medivation and Astellas Pharma, and generic bicalutamide. Agents targeting pathways outside of the androgen axis also may compete with enobosarm in breast cancer as they are directed towards similar patient populations that may benefit from enobosarm. Additionally, we plan to initiate a proof of concept study in advanced AR positive TNBC patients for which there are no currently approved therapies, beyond chemotherapy. However, a number of

Table of Contents

approaches for the treatment of TNBC are currently under investigation. Agents also targeting the androgen axis include XTANDI® (enzalutamide) being developed by Medivation and Astellas Pharma, orteronel (TAK-700) being developed by Takeda, and CR-1447 being developed by Curadis. Only a subset of the total TNBC population is AR positive; therefore, agents targeting TNBC as a whole may also compete with enobosarm if approved for commercial sale. These agents include: PI3K/AKT inhibitors (BKM120 being developed by Novartis), IL6/JAK/Stat inhibitors (ruxolitinib being developed by Incyte), mTOR inhibitors (Neratinib being developed by Puma), and PARP inhibitors (Velaparib being developed by AbbVie).

We are developing GTx-758 for secondary hormonal therapy in men with 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 has received approval for XTANDI® (enzalutamide), an oral androgen receptor antagonist, for the treatment of metastatic castration-resistant prostate cancer in men previously treated with docetaxel. Medivation and Astellas Pharma continue to develop XTANDI® (enzalutamide) for men with metastatic CRPC prior to receiving chemotherapy. Zytiga®, sold by Johnson & Johnson, has been approved for the treatment of metastatic CRPC prior in patients who have received prior chemotherapy and recently received approval for the treatment of metastatic castrate resistant prostate cancer prior to chemotherapy. Johnson & Johnson acquired Aragon Pharmaceuticals, Inc., which developed a second generation anti-androgen (ARN-509) that is currently being evaluated in Phase 2 studies in men with progressive, advanced prostate cancer. Millennium: The Takeda Oncology Company is developing TAK-700 for the treatment of men with metastatic castrate resistant prostate cancer prior to chemotherapy. GTx-758 is being developed as a treatment for this same patient population prior to the initiation of chemotherapy.

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

Risks Related to Employees, Growth and Other Aspects of Operations

Management transition creates uncertainties and could harm our business.*

We have recently had significant changes in executive leadership, and more could occur. Effective December 31, 2013, Mark Mosteller resigned as our Chief Financial Officer. In connection with Mr. Mosteller's resignation, Marc S. Hanover, who was then serving as our President and Chief Operating Officer,

was appointed as our acting principal financial officer and Jason T. Shackelford, who was then serving as our Corporate Controller and Director of Accounting, was appointed as our principal accounting officer. On April 3, 2014, Mitchell S. Steiner resigned as our Vice Chairman and Chief Executive Officer. On April 3, 2014, Mr. Hanover was appointed as our interim Chief Executive Officer. Upon the appointment of Mr. Hanover as interim Chief Executive Officer, Mr. Hanover ceased to perform the duties of our principal financial officer, which duties were assigned to Mr. Shackelford. Additionally, James T. Dalton, our former Chief Scientific Officer, resigned effective August 31, 2014.

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

Table of Contents

organization as a result of executive management transition may have a detrimental impact on our ability to implement our strategy and could have a material adverse effect on our business, financial condition and results of operations.

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

Our internal computer and information technology systems, or those of our CROs or other contractors or consultants, may fail or suffer security breaches, or could otherwise face serious disruptions, which could result in a material disruption of our product development efforts.*

Despite the implementation of security measures, our internal computer systems and those of our CROs and other contractors and consultants are vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, and telecommunication and electrical failures. Such events could cause interruptions of our operations. For instance, the loss of preclinical data or data from our ongoing, planned and potential future clinical trials involving our product candidates could result in delays in our development and regulatory filing efforts and significantly increase our costs. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data, or inappropriate disclosure of confidential or proprietary information, we could incur liability and the development of our product candidates could be delayed. In addition, our information technology and other internal infrastructure systems, including corporate firewalls, servers, leased lines and connection to the Internet, face the risk of systemic failure that could disrupt our operations. A significant disruption in the availability of our information technology and other internal infrastructure systems could cause delays in our research and development work and could otherwise adversely affect our business.

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

Our success depends on our continued ability to attract, retain and motivate highly qualified management, clinical and scientific personnel and on our ability to develop and maintain important relationships with leading academic institutions, clinicians and scientists. If we are not able to attract and keep senior management and key scientific personnel, we may not be able to successfully develop or commercialize our product candidates. All of our employees are at-will employees and can terminate their employment at any time.

In October 2013, we announced a reduction of approximately 60% of our workforce following our announcement that our POWER trials failed to achieve the results required by the FDA to file a NDA for enobosarm 3 mg for the prevention and treatment of muscle wasting in patients with advanced NSCLC. In addition, since our October 2013 workforce reduction, our former Chief Executive Officer, former Chief Financial Officer and former Chief Scientific Officer have resigned. Primarily as a result of our October 2013 workforce reduction, only 25 employees remained as employees of GTx as of September 30, 2014. Accordingly, we have been and are operating with a shortage of resources and may not be able to effectively conduct our operations with this limited number of employees. In addition, we announced past workforce reductions in each of December 2009 and June 2011, and our history of implementing workforce reductions, along with the potential for future workforce reductions, may negatively affect our ability to retain or attract talented employees. Further, to the extent we experience additional management transition, competition for top management is high and it may take many months to find a candidate that meets our requirements. If we are unable to attract and retain qualified management personnel, our business could suffer.

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

As of September 30, 2014, we had only 25 employees, and we will need to hire experienced personnel to develop and commercialize our product candidates and to otherwise grow our business, and we will need to expand

Table of Contents

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

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. On October 2, 2014, we received a letter from NASDAQ notifying us that for the previous 30 consecutive business days, the closing bid price for our common stock was below the minimum $1.00 per share requirement for continued listing on The NASDAQ Global Market, or the Bid Price Requirement. In accordance with NASDAQ listing rules, we have been afforded 180 calendar days, or until March 31, 2015, to regain compliance. To regain compliance, the closing bid price of our common stock must be at least $1.00 per share for a minimum of ten consecutive business days at any time before March 31, 2015. If we do not demonstrate compliance with the Bid Price Requirement by March 31, 2015, we may be eligible for an additional 180 calendar day compliance period. To qualify, we would need to transfer the listing of our common stock to The NASDAQ Capital Market, provided that we meet The NASDAQ Capital Market initial inclusion criteria at that time, with the exception of the Bid Price Requirement, and would need to provide written notice of our intention to cure the deficiency during this second compliance period, by effecting a reverse stock split, if necessary. However, if it appears to the NASDAQ staff that we will not be able to cure the deficiency, or if we are otherwise not eligible, NASDAQ would notify us that our common stock would be subject to delisting. In the event of such a notification, we may appeal the NASDAQ's staff determination to delist our common stock, but there can be no assurance that any such appeal would be successful. In addition, we may be unable to meet other applicable NASDAQ listing requirements, including maintaining minimum levels of stockholders' equity or market values of our common stock in which case, our common stock could be delisted notwithstanding our ability to demonstrate compliance with the Bid Price Requirement.

If our common stock is delisted, we would expect our common stock to be traded in the over-the-counter market, which could adversely affect the liquidity of our common stock. Additionally, we could face significant material adverse consequences, including:

- a limited availability of market quotations for our common stock;
- a reduced amount of news and analyst coverage for us;
- a decreased ability to issue additional securities and a concomitant substantial impairment in our ability to obtain sufficient additional capital to fund our operations and to continue as a going concern;
- reduced liquidity for our stockholders;
- potential loss of confidence by employees and potential future partners or collaborators; and
- loss of institutional investor interest and fewer business development opportunities.

The market price of our common stock has been has been volatile and may continue to be volatile in the future. This volatility may cause our stock price and the value of your investment to decline.*

The market prices for securities of biotechnology companies, including ours, have been highly volatile and may continue to be so in the future. In this regard, the market price for our common stock has varied between a high of $2.35 on January 17, 2014 and a low of $0.69 on September 23, 2014 in the twelve-month period ended September 30, 2014. The market price of our common stock is likely to continue to be volatile and subject to significant price and volume fluctuations. 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:

Table of Contents

- our ability to complete our recently-announced private placement in a timely manner, on the anticipated terms, or at all;
- delays in the initiation, enrollment and/or completion of our ongoing, planned and any future clinical trials of enobosarm and GTx-758, or negative, inconclusive or mixed results reported in any of our ongoing, planned and any future clinical trials of enobosarm and GTx-758;
- announcements regarding any plans to abandon the development of our enobosarm 3 mg program;
- announcements regarding our ability to determine, in consultation with the FDA, a feasible pathway forward to seek marketing approval for enobosarm 3 mg for the prevention and treatment of muscle wasting or cachexia in patients with NSCLC;
- our ability to raise additional capital in the future to carry through with our clinical development plans and current and future operations and the terms of any related financing arrangements;
- reports of unacceptable incidences of adverse events observed in any of our ongoing and planned clinical trials of enobosarm and GTx-758;
- announcements regarding further cost-cutting initiatives or restructurings;
- uncertainties created by our recent and potential future management turnover;
- our ability to enter into new collaborative, licensing or other strategic arrangements with respect to our product candidates;

- the terms and timing of any future collaborative, licensing or other arrangements that we may establish;
- the timing of achievement of, or failure to achieve, our and any potential collaborators' clinical, regulatory and other milestones, such as the commencement of clinical development, the completion of a clinical trial or the receipt of regulatory approval;
- announcement of FDA approval or non-approval of our product candidates or delays in or adverse events during the FDA review process;
- actions taken by regulatory agencies with respect to our product candidates or products, our clinical trials or our sales and marketing activities, including regulatory actions requiring or leading to restrictions, limitations and/or warnings in the label of an approved product candidate;
- the commercial success of any product approved by the FDA or its foreign counterparts;
- introductions or announcements of technological innovations or new products by us, our potential collaborators, or our competitors, and the timing of these introductions or announcements;
- market conditions for equity investments in general, or the biotechnology or pharmaceutical industries in particular;
- announcements regarding our ability to comply with the minimum listing requirements of The NASDAQ Stock Market LLC;
- regulatory developments in the United States and foreign countries;

Table of Contents

- 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;
- the trading volume of our common stock;
- changes in accounting principles; and
- additional losses of any of our key scientific or management personnel.

In addition, the stock markets in general, and the markets for biotechnology stocks in particular, have experienced significant volatility that has often been unrelated to the operating performance of particular companies. These broad market fluctuations may adversely affect the trading price of our common stock.

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

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

As of September 30, 2014, our executive officers, directors and holders of 5% or more of our outstanding common stock, including their affiliated or associated entities, held approximately 57.7% of our outstanding common stock, and our executive officers and directors alone, including their affiliated or associated entities, held approximately 33.5% of our outstanding common stock. In addition, if our recently-announced private placement is completed, this concentration of ownership among our executive officers, directors and holders of 5% or more of our outstanding common stock will increase. 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.

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

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

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

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

Table of Contents

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

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

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

For the 12-month period ended September 30, 2014, the average daily trading volume of our common stock on The NASDAQ Global Market was 532,665 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, 2014, we had 76,014,531 shares of common stock outstanding. In addition, as a result of the relatively low trading volume of our common stock, the trading of relatively small quantities of shares by our stockholders may disproportionately influence the market price of our common stock in either direction. The price for our shares could, for example, decline significantly in the event that a large number of our common shares are sold on the market without commensurate demand, as compared to an issuer with a higher trading volume that could better absorb those sales without an adverse impact on its stock price.

In November 2014, we entered into a securities purchase agreement under which we agreed to issue and sell in a private placement 64.3 million shares of our common stock and warrants to purchase 64.3 million shares of our common stock, the consummation of which has not yet occurred and is subject to customary closing conditions. Similarly, in March 2014 we completed a private placement of 11,976,048 shares of our common stock and warrants to purchase 10,179,642 shares of our common stock. Pursuant to the terms of a registration rights agreement we entered into in connection with the March 2014 private placement, we filed a registration statement under the Securities Act registering the resale of the 11,976,048 shares of common stock we issued to the investors in the private placement, which include J.R. Hyde, III, our largest stockholder, as well as the 10,179,642 shares of common stock underlying the warrants we issued to those investors. Likewise, pursuant to the terms of the securities purchase agreement we entered into in connection with the November 2014 securities purchase agreement, we agreed to file registration statements under the Securities Act registering the resale of the 64.3 million shares of common stock we agreed to issue to the investors in the private placement, which include J.R. Hyde, III, as well as the 64.3 million shares of common stock underlying the warrants we will issue to those investors if the private placement is consummated. Moreover, J.R. Hyde, III and certain of his affiliates, have rights under a separate registration rights agreement with us to require us to file resale registration statements covering an additional 7.9 million shares of common stock held in the aggregate or to include these shares in registration statements that we may file for ourselves or other stockholders. If Mr. Hyde or his affiliates or any of our other significant stockholders, including the other investors in our 2014 private placements, 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.

Table of Contents

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 10, 2014

By: /s/ Marc S. Hanover
Marc S. Hanover, President, Chief Operating Officer

and interim Chief Executive Officer
(Principal Executive Officer)

Date: November 10, 2014

By: /s/ Jason T. Shackelford
Jason T. Shackelford, Senior Director,
Accounting and Corporate Controller
Acting Principal Financial and Accounting Officer
(Principal Financial and Accounting Officer)

Table of Contents

EXHIBIT INDEX

Exhibit Number	Exhibit Description	Incorporation By Reference			
		Form	SEC File No.	Exhibit	Filing Date
2.1	Asset Purchase Agreement dated as of September 28, 2012 between the Registrant and Strakan International S.à r.l.	8-K	000-50549	2.1	10/03/2012
3.1	Restated Certificate of Incorporation of GTx, Inc.	S-3	333-127175	4.1	08/04/2005
3.2	Certificate of Amendment of Restated Certificate of Incorporation of GTx, Inc.	8-K	000-50549	3.2	05/06/2011
3.3	Certificate of Amendment of Restated Certificate of Incorporation of GTx, Inc.	8-K	000-50549	3.3	05/09/2014
3.4	Amended and Restated Bylaws of GTx, Inc.	8-K	000-50549	3.2	07/26/2007
4.1	Reference is made to Exhibits 3.1, 3.2, 3.3 and 3.4				
4.2	Specimen of Common Stock Certificate	S-1	333-109700	4.2	12/22/2003
4.3	Amended and Restated Registration Rights Agreement between Registrant and J. R. Hyde, III dated August 7, 2003	S-1	333-109700	4.4	10/15/2003
4.4	Consent, Waiver and Amendment between Registrant and J. R. Hyde, III and Pittco Associates, L.P. dated December 3, 2007	S-3	333-148321	4.6	12/26/2007
4.5	Waiver and Amendment Agreement among Registrant, J.R. Hyde, III and Pittco Associates, L.P. dated March 6, 2014	10-K	000-50549	4.5	03/12/2014
4.6	Amended and Restated Registration Rights Agreement among Registrant, J.R. Hyde, III and The Pyramid Peak Foundation, dated August 4, 2014	10-Q	000-50549	4.6	08/05/2014
4.7	Form of Common Stock Warrant, issued by Registrant pursuant to the Securities Purchase Agreement, dated March 3, 2014, among the Registrant, J.R. Hyde, III and The Pyramid Peak Foundation	10-K	000-50549	4.7	03/12/2014
4.8	Consent, Waiver and Amendment Agreement between Registrant and J.R. Hyde, III and Pittco Associates, L.P., dated August 4, 2014	10-Q	000-50549	4.8	08/05/2014
31.1+	Certification of Principal Executive Officer, as required by Rule 13a-14(a) or Rule 15d-14(a)	—	—	—	—
31.2+	Certification of Principal Financial Officer, as required by Rule 13a-14(a) or Rule 15d-14(a)	—	—	—	—
32.1+	Certification of Principal Executive Officer, as required by Rule 13a-14(b) or Rule 15d-14(b) and Section 1350 of Chapter 63 of Title 18 of the United States Code (18 U.S.C. §1350)(1)	—	—	—	—
32.2+	Certification of Principal Financial Officer, as required by Rule 13a-14(b) or Rule 15d-14(b) and Section 1350 of Chapter 63 of Title 18 of the United States Code (18 U.S.C. §1350)(1)	—	—	—	—
101.INS+	XBRL Instance Document	—	—	—	—
101.SCH+	XBRL Taxonomy Extension Schema Document	—	—	—	—
101.CAL+	XBRL Taxonomy Extension Calculation Linkbase Document	—	—	—	—
101.DEF+	XBRL Taxonomy Extension Definition Linkbase Document	—	—	—	—
101.LAB+	XBRL Taxonomy Extension Labels Linkbase Document	—	—	—	—
101.PRE+	XBRL Taxonomy Extension Presentation Linkbase Document	—	—	—	—

\+ 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.

EXHIBIT 31.1

PRINCIPAL EXECUTIVE OFFICER CERTIFICATION

I, Marc S. Hanover, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of GTx, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

   (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   (b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

   (c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   (d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

   (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: November 10, 2014

/s/ Marc S. Hanover

Marc S. Hanover
President, Chief Operating Officer and interim Chief Executive Officer
(Principal Executive Officer)

EXHIBIT 31.2

PRINCIPAL FINANCIAL OFFICER CERTIFICATION

I, Jason T. Shackelford, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of GTx, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

   (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   (b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

   (c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   (d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

   (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: November 10, 2014

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

EXHIBIT 32.1

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

In connection with the Quarterly Report of GTx, Inc. (the "Company") on Form 10-Q for the three months ended September 30, 2014, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I, Marc S. Hanover, Chief Executive Officer of the Company certify, pursuant to Rule 13a-14(b) or Rule 15d-14(b) and 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that, to the best of my knowledge:

1. The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

2. The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: November 10, 2014

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

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

EXHIBIT 32.2

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

In connection with the Quarterly Report of GTx, Inc. (the "Company") on Form 10-Q for the three months ended September 30, 2014, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I, Jason T. Shackelford, Principal Financial Officer of the Company certify, pursuant to Rule 13a-14(b) or Rule 15d-14(b) and 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that, to the best of my knowledge:

3. The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

4. The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: November 10, 2014

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

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