GTX INC /DE/
Form 10-Q
May 11, 2015
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 March 31, 2015

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 May 5, 2015, 140,374,112 shares of the registrant s Common Stock were outstanding.

Table of Contents

GTx, INC.

FORM 10-Q FOR THE QUARTER ENDED MARCH 31, 2015

INDEX

Table of Contents

PART I: FINANCIAL INFORMATION

ITEM 1. FINANCIAL STATEMENTS

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

	March 31, 2015 (unaudited)	December 31, 2014
ASSETS		
Current assets:		
Cash and cash equivalents	$ 14,163	$ 17,880
Short-term investments	30,437	31,415
Prepaid expenses and other current assets	743	856
Total current assets	45,343	50,151
Property and equipment, net	16	29
Intangible and other assets, net	423	471
Total assets	$ 45,782	$ 50,651
LIABILITIES AND STOCKHOLDERS EQUITY		
Current liabilities:		
Accounts payable	$ 438	$ 512
Warrant liability	27,782	30,430
Accrued expenses and other current liabilities	1,647	1,850
Total current liabilities	29,867	32,792
Other long-term liabilities	20	30
Commitments and contingencies		
Stockholders equity:		
Common stock, $0.001 par value: 200,000,000 shares authorized at March 31, 2015 and December 31, 2014; 140,374,112 and 140,325,643 shares issued and outstanding at March 31, 2015 and December 31, 2014, respectively	140	140
Additional paid-in capital	512,910	512,460
Accumulated deficit	(497,155)	(494,771)
Total stockholders equity	15,895	17,829
Total liabilities and stockholders equity	$ 45,782	$ 50,651

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 March 31,	
	2015	**2014**
Expenses:		
Research and development expenses	$ 2,948	$ 6,360
General and administrative expenses	2,111	2,629
Total expenses	5,059	8,989
Loss from operations	(5,059)	(8,989)
Other income, net	27	2
Gain on change in fair value of warrant liability	2,648	
Net loss	$ (2,384)	$ (8,987)
Net loss per share -- basic and diluted	$ (0.02)	$ (0.14)
Weighted average shares outstanding:		
Basic and diluted	140,335,875	66,512,069

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

Table of Contents

GTx, Inc.
CONDENSED STATEMENTS OF CASH FLOWS

(in thousands)

(unaudited)

	Three Months Ended March 31,	
	2015	**2014**
Cash flows from operating activities:		
Net loss	$ (2,384)	$ (8,987)
Adjustments to reconcile net loss to net cash used in operating activities:		
Gain on change in fair value of warrant liability	(2,648)	
Depreciation and amortization	17	33
Share-based compensation	419	2,215
Directors deferred compensation	31	32
Changes in assets and liabilities:		
Prepaid expenses and other assets	157	(1,169)
Accounts payable	(74)	(189)
Accrued expenses and other liabilities	(213)	(22)
Net cash used in operating activities	(4,695)	(8,087)
Cash flows from investing activities:		
Purchase of property and equipment		(4)
Purchase of short-term investments, held to maturity	(10,202)	(5,145)
Proceeds from maturities of short-term investments, held to maturity	11,180	
Net cash provided by (used in) investing activities	978	(5,149)
Cash flows from financing activities:		
Net proceeds from the issuance of common stock and warrants		21,141
Payments on capital lease and financed equipment obligations		(2)
Net cash provided by financing activities		21,139
Net (decrease) increase in cash and cash equivalents	(3,717)	7,903
Cash and cash equivalents, beginning of period	17,880	14,529
Cash and cash equivalents, end of period	$ 14,163	$ 22,432

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, 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 treat other serious medical conditions where building lean body mass is important. The Company announced during the second quarter of 2014 positive 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. The Company plans to initiate a Phase 2 proof-of-concept clinical trial of enobosarm later in the second quarter of 2015 that is designed to evaluate the efficacy and safety of enobosarm in patients with advanced AR positive triple-negative breast cancer. The Company also plans to initiate a second Phase 2 clinical trial in the third quarter of 2015 evaluating enobosarm in patients with ER positive and AR positive advanced breast cancer. Additionally, the Company is evaluating enobosarm and other compounds in its SARM portfolio for indications outside of oncology where unmet medical needs in muscle related diseases may benefit from building muscle.

In March 2015, the Company entered into an exclusive license agreement with the University of Tennessee Research Foundation (UTRF) to develop UTRF s proprietary selective androgen receptor degrader, or SARD, technology which has the potential to provide compounds that can degrade multiple forms of AR for those patients who do not respond or are resistant to current therapies to inhibit tumor growth in patients with progressive castration-resistant prostate cancer (CRPC). The Company s evaluation of the licensed SARD technology is at a very early stage and any future preclinical or clinical development of the SARD technology, beyond identifying potential lead clinical compounds, will require us to obtain additional funding.

The Company is also developing GTx-758 (Capesaris®), an oral nonsteroidal selective ER alpha agonist, for the treatment of advanced prostate cancer. 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 CRPC with data from this clinical trial expected in the third quarter of 2015. The Company does not plan to dedicate further resources to this program after the conclusion of this Phase 2 clinical trial and is currently determining third party interest in partnering or acquiring this asset and other preclinical ER alpha agonist compounds in order to fund additional clinical development.

The Company estimates that its current cash, cash equivalents and short-term investments, together with interest thereon, will be sufficient to meet its projected operating requirements through the end of 2016 (during which time it expects, at a minimum, to obtain results from the patients enrolled in the first stage of each of our planned open-label Phase 2 clinical trials of enobosarm in patients with AR positive advanced breast cancer).

Basis of Presentation

The accompanying unaudited condensed financial statements reflect, in the opinion of management, all adjustments (consisting of normal recurring adjustments) necessary for a fair presentation of GTx s financial position, results of operations and cash flows for each period presented in accordance with accounting principles generally accepted in the United States for interim financial information and with the instructions to Form 10-Q and

Table of Contents

GTx, Inc.
NOTES TO THE CONDENSED FINANCIAL STATEMENTS

(in thousands, except share and per share data)

(unaudited)

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, 2014. Operating results for the three months ended March 31, 2015 are not necessarily indicative of the results that may be expected for the entire fiscal year ending December 31, 2015.

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.

Warrant Liability

In November 2014, the Company issued warrants to purchase 64,311,112 shares of its common stock. The Company classifies the warrants as a liability on its balance sheet since the warrants contain certain terms that could require the Company (or its successor) to purchase the warrants for cash in an amount equal to the value (as calculated utilizing a contractually-agreed Black-Scholes-Merton option pricing valuation model (Black-Scholes Model)) of the unexercised portion of the warrants in connection with certain change of control transactions occurring on or prior to December 31, 2016, with such cash payment capped at an amount equal to $0.125 per unexercised share underlying each warrant. In addition, each warrant was subject to net cash settlement if, at the time of any exercise, there was then an insufficient number of authorized and reserved shares of common stock to effect a share settlement of the warrant. Under the terms of the warrants, as of May 6, 2015, the net cash settlement feature of the warrants automatically became inoperative; accordingly, the warrants are exercisable only for shares of the Company s common stock. See *Subsequent Events*.

As a result of the provision of the warrant requiring cash settlement upon certain change of control transactions, the Company is required to account for these warrants as a liability at fair value and the estimated warrant liability is required to be revalued at each balance sheet date until the earlier of the exercise of the warrants or the expiration of the provision on December 31, 2016 that could require cash settlement upon certain change of control transactions. Upon the exercise of the warrants or the expiration of the provision on December 31, 2016 that could require cash settlement upon certain change of control transactions, the fair value of the warrants will be reclassified from a liability to stockholders equity on the Company s balance sheets and no further adjustment to the fair value would be made in subsequent periods. See Note 4, *Stockholders Equity*, for further information regarding these warrants and the Company s valuation of the warrant liability.

Fair Value of Financial Instruments and Warrant Liability

The carrying amounts of the Company s financial instruments (which include cash, cash equivalents, short-term investments, and accounts payable) and its warrant liability approximate their fair values. The fair value of the warrant liability is estimated using the Black-Scholes-Merton pricing valuation model. See Note 4, *Stockholders Equity*, for additional disclosure on the valuation methodology and significant assumptions. The Company s financial assets and liabilities are classified within a three-level fair value hierarchy that prioritizes the inputs used to measure fair value, which is defined as follows:

Level 1 Quoted prices in active markets for identical assets or liabilities that the Company has the ability to access at the measurement date

Table of Contents

GTx, Inc.
NOTES TO THE CONDENSED FINANCIAL STATEMENTS

(in thousands, except share and per share data)

(unaudited)

Level 2 Inputs other than quoted prices in active markets that are observable for the asset or liability, either directly or indirectly

Level 3 Inputs that are unobservable for the asset or liability

Asset and liabilities measured at fair value on a recurring basis as of March 31, 2015 and December 31, 2014 included only the Company s warrant liability of $27,782 and $30,430, respectively, which were classified within Level 3 of the hierarchy. A gain of $2,648 related to the change in the fair value of the warrant liability was recognized during the three months ended March 31, 2015 as a non-cash gain in the Company s condensed statement of operations.

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

Research and Development Expenses

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

Cash, Cash Equivalents and Short-term Investments

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

At March 31, 2015 and December 31, 2014, 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.

Income Taxes

The Company accounts for deferred taxes by recognition of deferred tax assets and liabilities for the expected future tax consequences of events that have been included in the financial statements or tax returns. Under this method, deferred tax assets and liabilities are determined based on the difference between the financial statement and tax basis of assets and liabilities using enacted tax rates in effect for the year in which the differences are expected to reverse. A valuation allowance is provided when it is more likely than not that some portion or all of the deferred tax assets will not be realized. Accordingly, at March 31, 2015 and December 31, 2014, 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, 2014.

Table of Contents

GTx, Inc.
NOTES TO THE CONDENSED FINANCIAL STATEMENTS

(in thousands, except share and per share data)

(unaudited)

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.

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.

Subsequent Events

The Company has evaluated all events or transactions that occurred after March 31, 2015 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 May 6, 2015 (the Authorized Share Increase Date), the Company filed a Certificate of Amendment to the Company s Restated Certificate of Incorporation with the Secretary of State of the State of Delaware to increase the number of authorized shares of the Company s common stock, par value $0.001 per share, from 200,000,000 shares to 400,000,000 shares. The foregoing amendment was approved by the Company s stockholders at the Company s 2015 Annual Meeting of Stockholders held on May 6, 2015 (the Stockholder Approval Date).

On the Stockholder Approval Date, the warrants the Company issued in November 2014 became exercisable and will continue to be exercisable for four years thereafter. Under the terms of these warrants, as of the Authorized Share Increase Date, the net cash settlement feature of the warrants automatically became inoperative; accordingly, the warrants are exercisable only for shares of the Company s common stock.

On April 13, 2015, the Company entered into a new office lease with respect to the Company s current office space at 175 Toyota Plaza, Memphis, Tennessee (the Office Lease). The Office Lease commenced on May 1, 2015 with 26,250 rentable square feet and a three year term ending on April 30, 2018 (the Initial Term). The approximate aggregate rent due over the Initial Term is $1.4 million. Additionally, certain taxes and operating expenses may be charged to the Company beginning January 1, 2016 as additional rent based upon increases in these taxes and operating expenses after the base year, as defined in the Office Lease.

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 director is required to provide service in exchange for the award. The Company s share-based compensation plans are described more fully in Note 3 to the Company s financial statements included in the Company s Annual Report on Form 10-K for the year ended December 31, 2014.

Table of Contents

GTx, Inc.
NOTES TO THE CONDENSED FINANCIAL STATEMENTS

(in thousands, except share and per share data)

(unaudited)

The following table summarizes share-based compensation expense included within the condensed statements of operations for the three months ended March 31, 2015 and 2014:

	Three Months Ended March 31,	
	2015	2014
Research and development expenses	$ 189	$ 1,380
General and administrative expenses	261	867
Total share-based compensation	$ 450	$ 2,247

Share-based compensation expense recorded as general and administrative expense for the three months ended March 31, 2015 and 2014 included share-based compensation expense related to deferred compensation arrangements for the Company s non-employee directors of $31 and $32, respectively.

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

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

	Three Months Ended March 31,	
	2015	2014
Expected price volatility	88.5%	87.9%
Risk-free interest rate	2.0%	2.3%
Weighted average expected life in years	7 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, 2014	8,104,434	$ 4.24
Options granted	35,000	0.76
Options expired	(123,100)	5.27
Options exercised		
Options outstanding at March 31, 2015	8,016,334	4.21

During the three months ended March 31, 2015, the Company granted 7,700,000 RSUs to employees of which a portion of each award vests annually over a three year period from the date of grant. The Company estimates the fair value of RSUs using the closing price of its stock on the grant date. The fair value of RSUs is amortized on a straight-line basis over the requisite service period of the awards. The non-vested RSUs had a weighted average grant date fair value per share of $0.67.

Table of Contents

GTx, Inc.
NOTES TO THE CONDENSED FINANCIAL STATEMENTS

(in thousands, except share and per share data)

(unaudited)

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 RSUs and common stock warrants.

Weighted average potential shares of common stock of 83,097,046 and 10,382,571 for the three months ended March 31, 2015 and 2014, respectively, were excluded from the calculations of diluted loss per share as inclusion of the potential shares would have had an anti-dilutive effect on the net loss per share for the periods. The increase in the weighted average potential shares of common stock excluded from the calculation of diluted net loss per share increased from the prior year due to the issuance of warrants under the two financing transactions that occurred during the year ended December 31, 2014.

4. Stockholders Equity

Common Stock and Associated Warrant Liability

On November 14, 2014, the Company completed a private placement of units consisting of an aggregate of 64,311,112 shares of common stock and warrants to purchase an aggregate of 64,311,112 shares of its common stock for net proceeds of $42,814, after deducting offering expenses. The purchasers in the private placement included certain existing GTx stockholders and certain members of the GTx management team and board of directors. The net proceeds from the private placement were allocated to the common stock and warrants based upon the fair value method. Similarly, the offering expenses were allocated between the common stock and warrants with the portion allocated to common stock offset against the proceeds allocated to stockholders equity, whereas the portion allocated to the warrants was expensed immediately. The warrants have a per share exercise price of $0.85, became exercisable on May 6, 2015 and will continue to be exercisable for four years thereafter. Prior to the Authorized Share Increase Date, each warrant was subject to net cash settlement if, at the time of any exercise, there was then an insufficient number of authorized and reserved shares of common stock to effect a share settlement of the warrant. Under the terms of the warrants, as of the Authorized Share Increase Date, the net cash settlement feature of the warrants automatically became inoperative; accordingly, the warrants are exercisable only for shares of the Company s common stock. The warrants, however, contain certain terms that could require the Company (or its successor) to purchase the warrants for cash in an amount equal to the value (as calculated utilizing a contractually-agreed Black-Scholes-Merton pricing valuation model) of the unexercised portion of the warrants in connection with certain change of control transactions occurring on or prior to December 31, 2016, with the cash payment capped at an amount equal to $0.125 per unexercised share underlying each warrant. Due to the provision of the warrants that could require cash settlement upon certain change of control transactions, the Company is required to account for these warrants as a liability at fair value using the Black-Scholes Model and the estimated warrant liability is required to be revalued at each balance sheet date until the earlier of the exercise of the warrants or the expiration of the provision on December 31, 2016 that could require cash settlement upon certain change of control transactions.

The fair value of the warrants at March 31, 2015 of $27,782 was estimated using the Black-Scholes-Merton pricing valuation model with the following assumptions: expected volatility of 95%, risk-free interest rate of 1.1%, expected life of approximately 4.1 years and no dividends. The fair value of the warrants at December 31, 2014 of $30,430 was estimated using the Black-Scholes Model with the following assumptions: expected volatility of 91%, risk-free interest rate of 1.5%, expected life of approximately 4.5 years and no dividends. The decrease in fair value from December 31, 2014 of $2,648 was recorded as a non-cash gain on the change in fair value of warrant liability in the Company s condensed statement of operations. Significant changes to the Company s market price for its common stock will impact the implied and/or historical volatility used to fair value the warrants. Any significant increases in the Company s stock price will likely create an increase to the fair value of the warrant liability. Similarly, any significant decreases in the Company s stock price will likely create a decrease to the fair value of the warrant liability.

Table of Contents

GTx, Inc.
NOTES TO THE CONDENSED FINANCIAL STATEMENTS

(in thousands, except share and per share data)

(unaudited)

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 net proceeds of $21,135, after deducting offering expenses. The net proceeds from the private placement were allocated to the common stock and warrants based upon their relative fair values. The warrants, which had a one year term, expired unexercised on March 6, 2015.

5. University of Tennessee Research Foundation License Agreements

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.

The Company and UTRF also entered into a license agreement (the SARD License Agreement) in March 2015 pursuant to which the Company was granted exclusive worldwide rights in all existing SARD technologies owned or controlled by UTRF, including all improvements thereto. Under the SARD License Agreement, the Company is obligated to employ active, diligent efforts to conduct preclinical research and development activities for the SARD program to advance one or more lead compounds into clinical development. The Company is also obligated to pay UTRF annual license maintenance fees, low single-digit royalties on net sales of products and additional royalties on sublicense revenues, depending on the state of development of a clinical product candidate at the time it is sublicensed.

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, and our ability to advance the development of, our product candidates and our SARD development program;

- the timing of regulatory discussions and submissions, and the anticipated timing, scope and outcome of related regulatory actions or guidance;

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

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

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

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

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

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

- our estimates regarding the sufficiency of our cash resources, expenses, capital requirements and needs for additional financing, and our ability to obtain additional financing.

In some cases, you can identify forward-looking statements by terms such as anticipates, believes, could, estimates, expects, intends, ma potential, predicts, projects, should, will, would and similar expressions intended to identify forward-looking statements. Forward-lookin statements reflect our current views with respect to future events, are based on assumptions and are subject to risks, uncertainties and other important factors. We discuss many of these risks in this Quarterly Report on Form 10-Q in greater detail in the section entitled Risk Factors under Part II, Item 1A below. Given these risks, uncertainties and other important factors, you should not place undue reliance on these forward-looking statements. Also, forward-looking statements represent our estimates and assumptions only as of the date of this Quarterly Report on Form 10-Q. You should read this Quarterly Report on Form 10-Q and the documents that we incorporate by reference in and have filed as exhibits to this Quarterly Report on Form 10-Q, completely and with the understanding that our actual future results may be materially different from what we expect. Except as required by law, we assume no obligation to update any forward-looking statements publicly, or to update the reasons actual results could differ materially from those anticipated in any forward-looking statements, even if new information becomes available in the future.

Table of Contents

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, and other serious medical conditions. Our current strategy is focused on the further development of 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 treat other serious medical conditions where building lean body mass is important. In April 2015, we announced that we have entered into an exclusive worldwide license agreement with the University of Tennessee Research Foundation, or UTRF, to develop its proprietary selective androgen receptor degrader, or SARD, technology, which has the potential to provide compounds that can degrade multiple forms of androgen receptor, or AR, for patients who do not respond or are resistant to current therapies to inhibit tumor growth in patients with progressive castration-resistant prostate cancer, or CRPC. We are also developing GTx-758, an oral nonsteroidal selective estrogen receptor alpha agonist, for the treatment of advanced prostate cancer.

Business Highlights

Our lead SARM candidate, enobosarm (GTx-024), 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 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 estrogen receptor, or ER, positive and AR positive metastatic breast cancer who have previously responded to hormonal therapy. Based on the positive results of the 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 advanced breast cancer, as well as for women with advanced AR positive triple-negative breast cancer, or TNBC.

We plan to initiate a Phase 2 proof-of-concept clinical trial of enobosarm later in the second quarter of 2015 that is designed to evaluate the efficacy and safety of enobosarm in patients with advanced AR positive TNBC. This planned open-label clinical trial is designed to enroll up to approximately 55 patients, who will be administered an 18 mg oral daily dose of enobosarm, and clinical benefit will be assessed at four months of treatment. We plan to conduct this clinical trial using a Simon s two-stage design, pursuant to which we will enroll approximately half of the patients in the first stage, and, assuming a certain pre-specified minimal response rate is achieved, we will proceed with enrollment of the second stage. We also plan to initiate a second Phase 2 clinical trial in the third quarter of 2015 evaluating enobosarm in patients with ER positive and AR positive advanced breast cancer. This second planned open-label clinical trial is designed to enroll up to approximately 118 patients whose cancer treatment has shown prior response to hormonal therapy but has subsequently progressed. This second planned open-label clinical trial will randomize patients to either a 9 mg or 18 mg oral daily dose of enobosarm, again using a Simon s two-stage design, and will assess clinical benefit at six months of treatment. For each of these two Phase 2 clinical trials, clinical benefit is defined as a complete response, partial response or stable disease. We currently estimate we have sufficient funding through the end of 2016 to allow us to obtain the results from at least the patients enrolled in stage one of each clinical trial, but our ability to enroll patients to stage two of the clinical trials and complete these clinical trials will require us to seek sufficient additional funding. We are also evaluating enobosarm and other compounds in our SARM portfolio for indications outside of oncology where unmet medical needs in muscle related diseases may benefit from building muscle.

In March 2015, we entered into an exclusive worldwide license agreement with the UTRF to develop SARD compounds that may be capable of degrading multiple forms of AR. We envision initially developing SARDs for those patients who do not respond or are resistant to currently approved therapies to inhibit tumor growth in patients with progressive CRPC. Although current therapies have improved overall survival in men with CRPC, approximately one-third of the CRPC patients do not respond to these therapies, due in part to the presence of splice variants, including ARv7. Splice variants of the androgen receptor have been identified in which the binding site for androgens, the ligand binding domain, necessary for the action of many of the current therapies, is lost. In addition,

Table of Contents

most patients who do initially respond to available treatments eventually progress due to the emergence of resistance to these therapies. It is believed that CRPC growth remains highly dependent on androgen receptor activity, although the mechanisms which underlie this resistance are not fully understood. A therapeutic agent that would safely degrade multiple forms of the androgen receptor, including those without the ligand binding domain, may be uniquely positioned to address this patient population. Our evaluation of the licensed SARD program is at a very early stage. We are currently preparing an appropriate development program for SARDs and have initiated research to identify one or more lead compounds that could potentially be advanced into preclinical and clinical development. However, to advance preclinical development of our SARD program through the the requisite preclinical studies to support initial human studies, we will require additional funding, which we hope to obtain through the licensing, partnering or sale of certain other assets, including GTx-758 and its ER alpha agonist family of compounds, or through a strategic partnership or collaboration for the development and commercialization of SARDs.

We are also developing GTx-758, an oral nonsteroidal selective estrogen receptor alpha agonist, for the treatment of advanced prostate cancer. We believe GTx-758 has the potential to reduce testosterone to levels lower than those attainable with androgen deprivation therapy, or ADT, alone while ameliorating estrogen deficiency side effects, such as bone loss and hot flashes, which are common with current androgen deprivation therapies for prostate cancer.

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 prostate specific antigen, or PSA, will be reduced in men with CRPC. The primary endpoint of the current Phase 2 clinical trial is the proportion of subjects with a 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 in the study subjects. In addition, the clinical trial is evaluating the ability of GTx-758 to treat certain estrogen deficiency side effects associated with luteinizing hormone releasing hormone agonists such as hot flashes and bone loss. The Phase 2 clinical trial is designed to allow us to assess the safety and tolerability of GTx-758 in these subjects, including monitoring for venous thromboembolic events (blood clots), or VTEs. We have completed enrollment of the clinical trial. Both the 125 mg and 250 mg doses have demonstrated dose dependent increases in SHBG, reductions in free testosterone, and reductions in PSA, confirming the mechanism of action of the compound. To date, there has been one reported incidence of a VTE and one reported incidence of a myocardial infarction in patients enrolled in the 250 mg arm, resulting in the discontinuation of both patients from active treatment. The study is ongoing and primary efficacy data from all patients in the study is expected early in the third quarter of 2015. While we do not plan to dedicate any further resources to this program after the conclusion of our Phase 2 clinical trial, we are gauging third party interest in partnering or acquiring this asset and other preclinical ER alpha agonist compounds.

Financial Highlights

Our net loss for the three months ended March 31, 2015 was $2.4 million. The net loss for the three months ended March 31, 2015 included a non-cash gain of $2.6 million due to revaluation of our warrant liability at March 31, 2015, which warrant liability resulted from the issuance of common stock and warrants in our November 2014 private placement discussed below. We expect to incur significant operating losses for the foreseeable future as we continue our clinical development activities and potentially seek regulatory approval of our product candidates. We have funded our operations primarily through the sale of equity securities, collaboration and license agreements, and prior to September 2012, product revenue from sales of FARESTON®, the rights to which we sold to a third party in the third quarter of 2012. We currently have no ongoing collaborations for the development and commercialization of our product candidates and no source of revenue, nor do we expect to generate revenue for the foreseeable future. We do not expect to obtain FDA or EMA approval, or any other regulatory approvals, to market any of our product candidates, including enobosarm, for the foreseeable future, and it is possible that none of our product candidates will ever receive any regulatory approvals.

At March 31, 2015, we had cash, cash equivalents and short-term investments of $44.6 million compared to $49.3 million at December 31, 2014. On March 6, 2014, we completed a private placement of units consisting of 12.0 million shares of common stock and warrants to purchase 10.2 million shares of our common stock for net proceeds to us of approximately $21.1 million, after deducting offering expenses. These warrants expired on March 6, 2015. On November 14, 2014, we completed a separate 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 net proceeds to us of $42.8 million, after deducting offering expenses.

Table of Contents

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 through the end of 2016 (during which time we expect, at a minimum, to obtain results from the patients enrolled in stage one of each of our planned open-label Phase 2 clinical trials of enobosarm in patients with AR positive advanced breast cancer, using a Simon s two-stage clinical trial design). While we estimate that our current cash, cash equivalents and short-term investments are sufficient to fund our operations through 2016, we will need to obtain substantial additional funding to commence and complete stage two of both of our planned open-label Phase 2 clinical trials of enobosarm in patients with AR positive advanced breast cancer and to otherwise conduct additional studies required of us 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 enobosarm beyond our ongoing and currently-planned clinical trials and/or to meaningfully advance the preclinical development of the licensed SARD program, beyond identifying potential lead clinical compounds, we would need to obtain additional funding for such development, either through a financing, a strategic sale or licensing of assets, 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. 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 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, GTx-758, or SARDs, 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 Capital 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, GTx-758, or SARD 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 and supplies associated with our research activities, screening and identification of product candidates, formulation and synthesis activities, manufacturing, preclinical studies, toxicology studies, clinical trials, regulatory and medical affairs activities, quality assurance activities and license fees. As a result of the October 2013 reduction in our workforce, we are no longer conducting in-house drug discovery activities.

We expect that our research and development expenses for fiscal year 2015 will decrease as compared to fiscal year 2014 as the prior year included employee retention expenses, which were a part of our efforts to retain essential employees continuing with us following the October 2013 workforce reduction.

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.

16

Table of Contents

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 advanced AR positive TNBC (18 mg)	SARM	Phase 2	Plan to initiate a Phase 2 open-label proof-of-concept clinical trial evaluating enobosarm in patients with advanced AR positive TNBC later in the second quarter of 2015.
Enobosarm Treatment of women with ER positive and AR positive advanced breast cancer (9 mg and 18 mg)	SARM	Phase 2	Plan to initiate a Phase 2 open-label clinical trial evaluating enobosarm in patients with ER positive and AR positive advanced breast cancer in the third quarter of 2015.
GTx-758 Secondary hormonal therapy in men with metastatic and non-metastatic CRPC	Selective ER alpha agonist	Phase 2	Primary efficacy data from all patients in the study is expected early in the third quarter of 2015.

General and Administrative Expenses

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

Critical Accounting Policies and Significant Judgments and Estimates

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

While our significant accounting policies are more fully described in Note 2 to our financial statements appearing in our Annual Report on Form 10-K for the year ended December 31, 2014 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.

Warrant Liability

In November 2014, we issued warrants to purchase 64.3 million shares of our common stock in a private placement to certain investors. We classify the warrants as a liability on our balance sheet since these warrants contain certain terms that could require us (or our successor) to purchase the warrants for cash in an amount equal to

Table of Contents

the value (as calculated utilizing a contractually-agreed Black-Scholes option pricing formula) of the unexercised portion of the warrants in connection with certain change of control transactions occurring on or prior to December 31, 2016, with such cash payment capped at an amount equal to $0.125 per unexercised share underlying each warrant. As a result of the provision of the warrants that could require cash settlement upon certain change of control transactions, we are required to account for these warrants as a liability at fair value, which is calculated using the Black-Scholes-Merton pricing valuation model. The Black-Scholes-Merton pricing valuation model requires that we use significant assumptions and judgment to determine appropriate inputs to the model. Some of the assumptions that we rely on include the volatility of our common stock over the life of the warrant and risk-free interest rate. Our warrant liability is influenced by these assumptions and the price of our common stock as of the balance sheet date. The estimated warrant liability is required to be revalued at each balance sheet date until the earlier of the exercise of the warrants or the expiration of the provision on December 31, 2016 that could require cash settlement upon certain change of control transactions. Upon the exercise of the warrants or the expiration of the provision on December 31, 2016 that could require cash settlement upon certain change of control transactions, the fair value of the warrants will be reclassified from a liability to stockholders equity on our balance sheets and no further adjustment to the fair value would be made in subsequent periods.

Research and Development Expenses

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

Share-Based Compensation

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

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

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

18

Table of Contents

The following table summarizes share-based compensation expense included within the condensed statements of operations for the three months ended March 31, 2015 and 2014:

	Three Months Ended March 31,	
	2015	2014
	(in thousands)	
Research and development expenses	$ 189	$ 1,380
General and administrative expenses	261	867
Total share-based compensation	$ 450	$ 2,247

Share-based compensation expense recorded in the condensed statement of operations as general and administrative expense for the three months ended March 31, 2015 and 2014 included share-based compensation expense related to deferred compensation arrangements for our non-employee directors of $31,000 and $32,000, respectively. At March 31, 2015, the total compensation cost related to non-vested stock options not yet recognized was approximately $3.9 million with a weighted average expense recognition period of 3.69 years. At March 31, 2015, the total compensation cost related to non-vested RSUs not yet recognized was approximately $4.8 million with a weighted average expense recognition period of 2.13 years.

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.

Table of Contents

Results of Operations

Three Months Ended March 31, 2015 and 2014

Research and Development Expenses

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

Proposed Candidate / Proposed Indication	Program	Three Months Ended March 31, 2015	2014
		(in thousands)	
Enobosarm Treatment of women with AR positive TNBC (18 mg)	**SARM**	$ 1,181	$
Enobosarm Treatment of women with ER positive and AR positive advanced breast cancer (9 mg and 18 mg)	**SARM**	839	863
Enobosarm Prevention and treatment of muscle wasting in patients with advanced non-small cell lung cancer (3 mg)	**SARM**		3,987
GTx-758 Secondary hormonal therapy in men with metastatic and non-metastatic CRPC	**Selective ER alpha agonist**	555	1,462
Other research and development		373	48
Total research and development expenses		$ 2,948	$ 6,360

Research and development expenses decreased to $2.9 million for the three months ended March 31, 2015 from $6.4 million for the three months ended March 31, 2014.

Research and development expenses for enobosarm for the treatment of women with AR positive TNBC increased from the prior year period due to preparatory activities related to the planned Phase 2 clinical trial for the treatment of women with AR positive TNBC, which preparatory activities began in the fourth quarter of 2014 and continued into the first quarter of 2015.

Research and development expenses for enobosarm for the prevention and treatment of AR positive and ER positive metastatic breast cancer during the current year period consisted of expenses for preparatory activities related to the planned Phase 2 clinical trial for the treatment of women with ER positive and AR positive advanced breast cancer, which preparatory activities began in the fourth quarter of 2014 and continued into the first quarter of 2015, as well as expenses related to our ongoing Phase 2 proof-of-concept clinical trial evaluating enobosarm 9 mg for the treatment of AR positive and ER positive metastatic breast cancer in women who have previously responded to hormonal therapy for the treatment of their metastatic breast cancer. The prior year period consisted only of expenses related to our ongoing Phase 2 proof-of-concept clinical trial that began in the second quarter of 2013.

Table of Contents

Research and development expenses for enobosarm for the prevention and treatment of muscle wasting in patients with advanced NSCLC decreased by $4.0 million from the prior year period as activities related to satisfying the prerequisites necessary for our then-planned regulatory submission in Europe for enobosarm 3 mg, including conducting seven Phase 1 clinical trials, were being performed in the first quarter of 2014 and were completed by the end of 2014.

Research and development expenses related to the ongoing Phase 2 clinical trial to evaluate GTx-758 as secondary hormonal therapy in men with metastatic CRPC decreased by $907,000 for the three months ended March 31, 2015 compared to the prior year period due to the timing of patient activities and related management expenses as this trial was initiated in the third quarter of 2012 and enrollment was completed during the first quarter of 2015.

Additionally, research and development expenses for each product candidate in the prior year included expenses related to cash bonuses and stock option and RSU grants made to the employees as part of our efforts to retain the essential employees continuing with us following the October 2013 workforce reduction.

General and Administrative Expenses

General and administrative expenses decreased 20% to $2.1 million for the three months ended March 31, 2015 from $2.6 million for the three months ended March 31, 2014, which was due primarily to expenses in the prior year period related to cash bonuses and stock option and RSU grants made to the employees as part of our efforts to retain the essential employees continuing with us following the October 2013 workforce reduction.

Gain on Change in Fair Value of Warrant Liability

We recognized a warrant liability due to certain provisions of the warrants issued as part of the November 2014 private placement of common stock and warrants. The warrants are required to be accounted for as a liability at fair value and the fair value must be revalued at each balance sheet date until the earlier of the exercise of the warrants or the expiration of the provision on December 31, 2016 that could require cash settlement upon certain change of control transactions. The resulting non-cash gain or loss on the fair value revaluation at each balance sheet date is recorded as non-operating income in our condensed statement of operations.

These warrants were revalued at fair value as of March 31, 2015 and the decrease in fair value of $2.6 million was recorded as a non-cash gain on the change in fair value of warrant liability in the Company s condensed statement of operations.

Liquidity and Capital Resources

At March 31, 2015, we had cash, cash equivalents and short-term investments of $44.6 million compared to $49.3 million at December 31, 2014. Net cash used in operating activities was $4.7 million and $8.1 million for the three months ended March 31, 2015 and 2014, respectively, and resulted primarily from funding our operations.

Net cash provided by investing activities was $978,000 for the three months ended March 31, 2015 and resulted from the maturities of short-term investments of $11.2 million offset by the purchase of short-term investments of $10.2 million. Net cash used in investing activities was $5.1 million for the three months ended March 31, 2014 and resulted primarily from the purchase of short-term investments.

Net cash provided by financing activities was $0 and $21.1 million for the three months ended March 31, 2015 and 2014, respectively. Net cash provided by financing activities for the three months ended March 31, 2014 reflects proceeds from the issuance of common stock and warrants, partially offset by payments on capital lease obligations.

We estimate that our current cash, cash equivalents and short-term investments, together with interest thereon, will be sufficient to meet our projected operating requirements through the end of 2016 (during which time we expect, at a minimum, to obtain results from the patients enrolled in stage one of each of our planned open-label Phase 2 clinical trials of enobosarm in patients with AR positive advanced breast cancer, using a Simon s two-stage clinical trial design). While we estimate that our current cash, cash equivalents and short-term investments are sufficient to fund our operations through 2016, we will need to obtain substantial additional funding to commence and complete stage two of both of our planned open-label Phase 2 clinical trials of enobosarm in patients with AR

Table of Contents

positive advanced breast cancer and to otherwise conduct additional studies required of us 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 enobosarm beyond our ongoing and currently-planned clinical trials and/or to meaningfully advance the preclinical development of the licensed SARD program, beyond identifying potential lead clinical compounds, we would need to obtain additional funding for such development, either through a financing, a strategic sale or licensing of assets, or by entering into collaborative arrangements or partnerships with third parties for such further development.

Our estimate of the period of time or events through which our financial resources will be adequate to support our projected operating requirements is a forward-looking statement and involves risks and uncertainties, and actual results could vary as a result of a number of factors, including the factors discussed under Part II, Item 1A Risk Factors section of this Quarterly Report on Form 10-Q. Because of the numerous risks and uncertainties associated with the development and potential commercialization of our product candidates and other research and development activities, including risks and uncertainties that could impact the rate of progress of our development activities, we are unable to estimate with certainty the amounts of increased capital outlays and operating expenditures associated with the future development of our product candidates, if any. Our future funding requirements will depend on many factors, including:

- the scope, rate of progress and cost of our preclinical and 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;

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

- the effect of competing technological and market developments; and

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

We do not currently have any commitments for future external funding nor do we currently have any sources of revenue. Until we can generate a sufficient amount of product revenue, which we may never do, we expect to finance future cash needs through 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 submit a NDA for enobosarm, we announced and implemented a workforce reduction of approximately 60%. If we are unable to raise additional funds when needed, we may need to further reduce our expenditures, perhaps significantly, to preserve our cash. Cost-cutting measures that we may take in the future may not be sufficient to enable 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, GTx-758 and/or SARDs 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 completed a subsequent private placement in November 2014 that represented 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

Table of Contents

AR positive advanced breast cancer and our ability to advance the development of enobosarm, GTx-758, or SARDs, 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 Capital 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, GTx-758, or SARD programs, or conduct additional workforce or other expense reductions, any of which could have a material adverse effect on our business and our prospects.

Contractual Obligations

Our future minimum contractual obligations were reported in our Annual Report on Form 10-K for the year ended December 31, 2014, as filed with the SEC. Although there were no material changes during the first quarter of 2015 from the contractual obligations previously disclosed in our Annual Report on Form 10-K for the year ended December 31, 2014, we entered into a new office lease on April 13, 2015 with respect to our current office space. The new office lease term commenced on May 1, 2015 with a three year term ending on April 30, 2018. Operating lease obligations under the new office lease include aggregate future minimum payments of approximately $1.4 million.

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.

We had requested and, on March 16, 2015, received approval from The NASDAQ Stock Market to transfer our listing from The NASDAQ Global Market to The NASDAQ Capital Market. The transfer was effective at the opening of trading on March 19, 2015, and our common stock continues to trade under the symbol GTXI. On April 1, 2015, we were afforded an additional 180-day grace period, through September 28, 2015, to comply with the Bid Price Requirement, by which date our common stock must trade above $1.00 for at least ten consecutive business days. In this regard, we have provided written notice to NASDAQ of our intention to cure the Bid Price Requirement deficiency during this second 180 calendar day compliance period by effecting a reverse stock split, if necessary. If we do not regain compliance by September 28, 2015, then NASDAQ will provide written notice that our common stock will be subject to delisting from The NASDAQ Capital Market. In the event we do not regain compliance, we may appeal the decision to a NASDAQ Listing Qualifications Panel, 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 three months ended March 31, 2015, 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, 2014.

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

Table of Contents

and communicated to our management, including our Principal Executive Officer and Principal Financial Officer, as appropriate, to allow for timely decisions regarding required disclosures.

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

There were no changes in our internal control over financial reporting during the first quarter of 2015 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 16, 2015.

Risks Related to Our Financial Condition and Need for Additional Financing

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

As of December 31, 2015, we had an accumulated deficit of $497.2 million. Our net loss for the three months ended March 31, 2015 was $2.4 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 U.S. Food and Drug Administration, or FDA, significantly depressed our stock price and has harmed our future prospects. Although we evaluated 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 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 submit 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

Table of Contents

function, the FDA will not accept a new drug application, or NDA, 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. 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. While we do not intend to commit additional internal resources for the development of GTx-758 once we have completed the efficacy and safety analysis of our ongoing Phase 2 clinical trial, our newly in-licensed selective androgen receptor degrader, or SARD, technology will require significant additional financial resources and personnel to continue development of these preclinical compounds. 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, GTx-758, or SARD technology, if development is further delayed or is eliminated, or if sales revenue from enobosarm, GTx-758, or SARD technology upon receiving marketing approval, if ever, is insufficient, we may never become profitable and we will not be successful.

We will need to raise substantial additional capital and may be unable to raise capital when needed, which would force us to delay, reduce or eliminate our product development programs 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 through the end of 2016 (during which time we expect, at a minimum, to obtain results from the patients enrolled in stage one of each of our planned open-label Phase 2 clinical trials of enobosarm in patients with AR positive advanced breast cancer, using a Simon s two-stage clinical trial design). While we estimate that our current cash, cash equivalents and short-term investments are sufficient to fund our operations through 2016, we will need to obtain substantial additional funding to commence and complete stage two of both of our planned open-label Phase 2 clinical trials of enobosarm in patients with AR positive advanced breast cancer and to otherwise conduct additional studies required of us 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 enobosarm beyond our ongoing and currently-planned clinical trials and/or to meaningfully advance the preclinical development of the licensed SARD program, beyond identifying potential lead clinical compounds, we would need to obtain additional funding for such development, either through a financing, a strategic sale or licensing of assets, or by entering into collaborative arrangements or partnerships with third parties for such further development.

Table of Contents

Our future funding requirements will depend on many factors, including:

- the scope, rate of progress and cost of our preclinical and 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;

- 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. 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 submit a NDA for enobosarm, we announced and implemented a workforce reduction of approximately 60%. If we are unable to raise additional funds when needed, we may need to further reduce our expenditures, perhaps significantly, to preserve our cash. Cost-cutting measures that we may take in the future may not be sufficient to enable 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, GTx-758 and/or our recently-licensed selective androgen receptor degrader, or SARD, 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 completed a subsequent private placement in November 2014 that represented 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, GTx-758, or SARDs, 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 Capital 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, GTx-758, or SARD programs, or conduct additional workforce or other expense reductions, any of which could have a material adverse effect on our business and our prospects.

Table of Contents

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 models of breast cancer and our ongoing Phase 2 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 advanced AR positive TNBC and ER positive and AR positive advanced breast cancer.

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 of our two planned Phase 2 clinical trials of enobosarm in patients with AR positive advanced breast cancer. Furthermore, the positive results from our ongoing Phase 2 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 evaluated 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 submit 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 NDA 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 addition, we do not currently have any further clinical development plans for GTx-758 and we do not in any event have sufficient funds to enable further clinical development of GTx-758. Likewise, our evaluation of the recently-licensed SARD program is at a very early stage and any meaningful preclinical and clinical development, beyond identifying potential lead clinical compounds, of our SARD program will require us to obtain additional funding. Accordingly, our current strategy and near-term prospects are substantially dependent on the successful development of enobosarm for the treatment of patients with AR positive advanced breast cancer.

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

Table of Contents

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 evaluated 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 submit 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 NDA 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 addition, in the first quarter of 2015, we entered into an exclusive worldwide license agreement with the University of Tennessee Research Foundation, or UTRF, to develop its proprietary SARD technology. However, our evaluation of the licensed SARD program is at a very early stage and it is possible that we may determine not to move forward with any meaningful preclinical development of our SARD program, beyond identifying potential lead clinical compounds. Even if we do determine to move forward with any meaningful preclinical development of our SARD program, to advance preclinical development of our SARD program through the requisite preclinical studies to support initial human studies, we will require additional funding. Accordingly, as a result of our unsuccessful research and preclinical development and/or our inability to obtain sufficient funding to meaningfully advance preclinical development of our SARD program, we may fail to realize the anticipated benefits of our licensing of this 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 modified or will be completed on schedule, if at all. For example, both of our planned Phase 2 clinical trials of enobosarm in patients with AR positive advanced breast cancer are designed to be conducted using a Simon s two-stage design, pursuant to which we plan to enroll approximately half of the patients in the first stage, and, upon achievement of a pre-specified minimal response rate, we plan to proceed with enrollment of the second stage. However, even if we achieve the pre-specified minimal response rate, our ability to proceed with enrollment of and to complete the second stage in both trials is subject to our ability to obtain additional funding, which we may be unable to do. In any event, 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

Table of Contents

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 serious adverse events, or SAEs, in the current Phase 2 clinical trial. In this regard, there has been one reported incidence of a VTE and one reported incidence of a myocardial infarction (MI) in patients enrolled in the 250 mg arm of our ongoing Phase 2 clinical trial of GTx-758, resulting in the discontinuation of both patients from active treatment, and we cannot assure you that we will not observe additional SAEs in this trial. If an unacceptable incidence of VTEs, MIs, or other SAEs 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, or HDL, have also been observed in subjects treated with enobosarm. Lower levels of HDL could lead to increased risk of adverse cardiovascular events. In addition, in our ongoing 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, bone pain of the chest cage was assessed as possibly related to enobosarm. Although doses up to 30 mg have been evaluated in short duration studies, doses higher than the 9 mg dose currently being tested in our Phase 2 clinical trials may increase the risk or incidence of known potential side effects of SARMs including elevations in hepatic enzymes and further reductions in HDL, in addition to the emergence of side effects that have not been seen to date. Although no evidence of virilization has been seen to date with any dose of enobosarm, higher doses for longer duration may increase the risk of hair growth and masculinization in some women.

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

Table of Contents

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 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 enobosarm beyond our ongoing and currently-planned clinical trials, 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. Moreover, both of the planned Phase 2 clinical trials of enobosarm are designed to be conducted using a Simon s two-stage design, pursuant to which we plan to enroll approximately half of the patients in the first stage, and, upon achievement of a pre-specified minimal response rate, we plan to proceed with enrollment of the second stage. However, even if we achieve the pre-specified minimal response rate, our ability to proceed with enrollment of and to complete the second stage in both trials is subject to our ability to obtain additional funding, which we may be unable to do. Likewise, any meaningful preclinical development, beyond identifying potential lead clinical compounds, of our SARD program will require us to obtain additional funding. 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,

Table of Contents

we may not receive any future milestone payments and will not receive any royalties for the compound or product candidate;

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

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

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

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

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

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

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

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

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

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

- the possible termination or non-renewal of the agreement by the third party, based on its own business priorities, at a time that is costly or inconvenient for us; and

- drug product supplies not meeting the requisite requirements for clinical trial use.

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

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

Table of Contents

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 licenses from 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. Our license agreements with UTRF, under which we were granted rights to SARM compounds and technologies, including enobosarm, and more recently, to SARD compounds and technology, may be terminated by UTRF if we are in breach of our obligations under, or fail to perform any terms of, the relevant agreement and fail to cure that breach. If one or both of these agreements are terminated, then we may lose our rights to utilize the SARM and/or SARD technology and intellectual property covered by those agreements to market, distribute and sell licensed products, which may prevent us from continuing a substantial part of our business and may result in a material and serious adverse effect on our financial condition, results of operations and any prospects for growth.

If some or all of our or our licensor s 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 licensor owns or controls 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 licensor s 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 licensor, 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 licensor 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

Table of Contents

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 licensor s 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 take many months to publish and patent applications can take many years to issue, there may be currently pending applications, unknown to us or our licensor, 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

Table of Contents

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 evaluated 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 submit 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 NDA 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.

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 this Annual Report on Form 10-K for additional information regarding risks associated with marketing approval, as well as risks related to potential post-approval requirements.

Risks Related to Commercialization

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

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

Table of Contents

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

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

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).

Table of Contents

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

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

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

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

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

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

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

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:

Table of Contents

- decreased demand for any product candidates or products;
- injury to our reputation;
- withdrawal of clinical trial participants;
- costs to defend the related litigation;
- substantial monetary awards to trial participants or patients;
- loss of revenue; and
- the inability to commercialize any products for which we obtain or hold marketing approvals.

We have product liability insurance that covers our clinical trials and any commercial products up to a $25 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. We plan to advance the development of enobosarm for the treatment of patients with AR positive advanced breast cancer. To our knowledge, 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 SARM 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 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), PD-1 inhibitors (pembrolizumab) being developed by Merck & Co. and MPDL3280A being developed by Roche.

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.

Table of Contents

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. Provenge®, which was recently acquired by Valeant Pharmaceuticals, is an autologous cellular immunotherapy for the treatment of asymptomatic or minimally symptomatic metastatic castrate resistant prostate cancer. Medivation and Astellas Pharma market XTANDI® (enzalutamide), an oral androgen receptor antagonist, for the treatment of metastatic castration-resistant prostate cancer in men previously treated with docetaxel as well as those that have not yet received chemotherapy. Zytiga®, sold by Johnson & Johnson, has been approved for the treatment of metastatic CRPC 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. Bayer HealthCare and Orion Corporation are currently performing a Phase 3 study of ODM-201 in men with CRPC without metastases and with a rising PSA examining safety and efficacy by measuring metastatic free survival. Millennium: The Takeda Oncology Company is developing TAK-700 for the treatment of men with metastatic CRPC prior to chemotherapy.

We recently announced that we have entered into an exclusive worldwide license agreement with UTRF to develop its proprietary SARD technology which has the potential to provide compounds that can degrade multiple forms of AR for patients who do not respond or are resistant to current therapies to inhibit tumor growth in patients with progressive CRPC. We anticipate developing SARD compounds initially to target those men who are not responsive and/or develop resistance to currently available agents to treat men with CRPC. Drugs in development having potentially similar mechanisms of action to our SARD compounds include Androscience Corporation s androgen receptor degrader enhancer, or ARD, currently in development for acne and alopecia with the potential for development in prostate cancer. In addition to this specific potential mechanistic competition, there are various products approved or under clinical development in the broader space of treating men with advanced prostate cancer who have metastatic CRPC which may compete with our proposed initial clinical objective for our SARD compounds, as set forth above in the paragraph relating to GTx-758. Additionally, it has been reported that two other companies are developing drugs to treat men with CRPC who are resistant to current therapies: Tokai Pharmaceuticals is developing TOK-001 (Galeterone) with a principal mechanism of action as a CYP17 lyase inhibitor and AR antagonist and Essa Pharma Inc. is beginning early studies with EPI-506, an AR antagonist that targets the N-terminal domain of the AR.

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 and on February 12, 2015, Mr. Hanover was appointed as our permanent 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. Finally, on March 2,

2015, Robert J. Wills was appointed as our Executive Chairman.

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 our Chief Executive Officer in addition to the role he served

Table of Contents

when functioning as our President and Chief Operating Officer, positions that were previously occupied by two persons. In addition, while Dr. Wills role as our Executive Chairman is, in part, to support Mr. Hanover in his role as our permanent Chief Executive Officer, the position of Executive Chairman is new to us and it may be some time before we can assess how much assistance he will provide to Mr. Hanover. 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 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 27 employees remained

as employees of GTx as of March 31, 2015. 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.

Table of Contents

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 March 31, 2015, we had only 27 employees, and we will need to hire experienced personnel to develop and commercialize our product candidates and to otherwise grow our business, and we will need to expand the number of our managerial, operational, financial and other employees to support that growth. Competition exists for qualified personnel in the biotechnology field.

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

Risks Related to Our Common Stock

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. We had requested and, on March 16, 2015, received approval from The NASDAQ Stock Market to transfer our listing from The NASDAQ Global Market to The NASDAQ Capital Market. The transfer was effective at the opening of trading on March 19, 2015, and our common stock continues to trade under the symbol GTXI. On April 1, 2015, we were afforded an additional 180-day grace period, through September 28, 2015, to comply with the Bid Price Requirement, by which date our common stock must trade above $1.00 for at least ten consecutive business days. In this regard, we have provided written notice to NASDAQ of our intention to cure the Bid Price Requirement deficiency during this second 180 calendar day compliance period by effecting a reverse stock split, if necessary. On May 6, 2015, we received approval from our stockholders to implement a proposed reverse stock split. However, we cannot assure you that the proposed reverse stock split, if effected, will result in a sustained increase to our stock price and have the desired effect of maintaining compliance with Bid Price Requirement or other applicable NASDAQ listing requirements. In addition, the liquidity of our common stock may be harmed by the proposed reverse stock split given the reduced number of shares that would be outstanding after the reverse stock split, particularly if our stock price does not increase as a result of the reverse stock split. In any event, if we do not regain compliance by September 28, 2015, then NASDAQ will provide written notice that our common stock will be subject to delisting from The NASDAQ Capital Market. In the event we do not regain compliance, we may appeal the decision to a NASDAQ Listing Qualifications Panel, 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.

Table of Contents

The market price of our common stock 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 $1.70 on May 30, 2014 and a low of $0.41 on October 14, 2014 in the twelve-month period ended March 31, 2015. 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:

- 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;

- our ability to raise additional capital in the future to carry through with our preclinical and clinical development plans, including to commence and complete stage two of both of our planned Phase 2 clinical trials of enobosarm, as well as our 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 past 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 our clinical trials, including regulatory actions requiring or leading to a delay or stoppage of our ongoing or planned clinical trials;

- the potential negative effects, or the perception of potential negative effects, resulting from a proposed reverse stock split approved by our stockholders in May 2015;

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

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

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

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

- regulatory developments in the United States and foreign countries;

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

- any intellectual property infringement lawsuit involving us;

- actual or anticipated fluctuations in our results of operations;

- changes in financial estimates or recommendations by securities analysts;

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

41

Table of Contents

- 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 March 31, 2015, our executive officers, directors and holders of 5% or more of our outstanding common stock, including their affiliated or associated entities, held approximately 75.7% of our outstanding common stock, and our executive officers and directors alone, including their affiliated or associated entities, held approximately 35.8% of our outstanding common stock as well as warrants to purchase up to an additional 24.8 million shares of common stock. As a result, these stockholders, acting together, have the ability to control all matters requiring approval by our stockholders, including the election of directors and the approval of mergers or other business combination transactions. The interests of this group of stockholders may not always coincide with our interests or the interests of other stockholders.

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 completed a study in 2014 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:

- 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

Table of Contents

Directors; and

- limitations on the removal of directors.

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

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

For the 12-month period ended March 31, 2015, the average daily trading volume of our common stock on The NASDAQ Global Market was 269,066 shares. As a result, future sales of a substantial number of shares of our common stock in the public market, or the perception that such sales may occur, could adversely affect the then-prevailing market price of our common stock. As of March 31, 2015, we had 140,374,112 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 completed a private placement 64.3 million shares of our common stock and warrants to purchase 64.3 million shares of our common stock. Similarly, in March 2014 we completed a private placement of 12.0 million shares of our common stock and warrants to purchase 10.2 million 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 12.0 million shares of common stock we issued to the investors in the March 2014 private placement, which include J.R. Hyde, III, our largest stockholder, as well as the 10.2 million 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 private placement, we filed a registration statement under the Securities Act registering the resale of the 64.3 million shares of common stock we issued to the investors in the November 2014 private placement, which included J.R. Hyde, III, and we also agreed to file one or more registration statements covering the resale of the 64.3 million shares of common stock subject to the warrants we issued to the investors in the November 2014 private placement. 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 5. OTHER INFORMATION

Charter Amendment

On May 6, 2015, at the Company s 2015 Annual Meeting of Stockholders (the Annual Meeting), the Company s stockholders approved an amendment to GTx s Restated Certificate of Incorporation to increase the number of authorized shares of GTx s common stock from 200,000,000 shares to 400,000,000 shares. The increase in the number of authorized shares of the Company s common stock was effected pursuant to a Certificate of Amendment of Restated Certificate of Incorporation (the Certificate of Amendment) filed with the Secretary of State of the State of Delaware on May 6, 2015 and was effective as of such date. A copy of the Certificate of Amendment is attached as Exhibit 3.4 hereto.

Amendment and Restatement of the GTx, Inc. 2013 Equity Incentive Plan

At the Annual Meeting held on May 6, 2015, the Company s stockholders approved the amendment and restatement of the Company s 2013 Equity Incentive Plan (the 2013 Plan) to:

Table of Contents

- increase the maximum number of shares of the Company s common stock subject to stock options, stock appreciation rights and other stock awards whose value is determined by reference to an increase over an exercise price or strike price of at least 100% of the fair market value of the Company s common stock on the date of grant that may be granted to any participant during any calendar year from 1,000,000 shares to 5,000,000 shares (subject to adjustment for changes in the Company s capitalization); and

- increase the maximum number of shares of the Company s common stock subject to performance stock awards that may be granted to any participant during any calendar year from 1,000,000 shares to 5,000,000 shares (subject to adjustment for changes in the Company s capitalization).

The amendment and restatement of the 2013 Plan (as so amended and restated, the Restated 2013 Plan), previously had been approved, subject to stockholder approval, by the Board of Directors of the Company. The Restated 2013 Plan became effective immediately upon stockholder approval at the Annual Meeting.

A more detailed summary of the material features of the Restated 2013 Plan is set forth in the Company s definitive proxy statement for the Annual Meeting filed with the Securities and Exchange Commission on March 26, 2015 (the Proxy Statement). That summary and the foregoing description is qualified in its entirety by reference to the text of the Restated 2013 Plan, which is attached as Annex C to the Proxy Statement.

Submission of Matters to a Vote of Security Holders

At the Annual Meeting held on May 6, 2015 at the Company s corporate offices in Memphis, Tennessee, the Company s stockholders voted on the following five proposals:

(1) Proposal to elect the three nominees for Class II director named below to serve until the 2018 Annual Meeting of Stockholders and until their successors have been duly elected and qualified. Each of the three named nominees was so elected, with the votes thereon at the Annual Meeting as follows:

	Final Voting Results		
Nominee	**For**	**Withheld**	**Broker Non-Vote**
J. Kenneth Glass	117,267,037	638,107	16,005,129
Marc S. Hanover	100,043,148	17,861,996	16,005,129
Robert J. Wills, Ph.D.	117,689,763	217,881	16,002,629

The Company s Class III directors, Michael G. Carter, M.D., Ch.B., F.R.C.P. and J. R. Hyde, III, will each continue to serve on the Company s Board of Directors until the Company s 2016 Annual Meeting of Stockholders and until his successor is elected and has qualified, or until his earlier death, resignation or removal. The Company s Class I director, Kenneth S. Robinson, M.D., M.Div., will continue to serve on the Company s Board of Directors until the Company s 2017 Annual Meeting of Stockholders and until his successor is elected and has qualified, or until his earlier death, resignation or removal.

(2) Proposal to approve an amendment to GTx s Restated Certificate of Incorporation to increase the number of authorized shares of GTx s common stock from 200,000,000 shares to 400,000,000 shares. This proposal was approved, with the votes thereon at the Annual Meeting as follows:

Final Voting Results			
For	**Against**	**Abstain**	**Broker Non-Vote**
129,970,307	3,625,372	314,594	

Table of Contents

(3) Proposal to approve a series of alternate amendments to GTx s Restated Certificate of Incorporation to effect, at the discretion of the Board of Directors, a reverse stock split at a reverse stock split ratio ranging from one-for-five (1:5) and one-for-fifteen (1:15), inclusive, and a corresponding reduction in the total number of authorized shares of GTx s common stock, as more specifically described in the Proxy Statement. This proposal was approved, with the votes thereon at the Annual Meeting as follows:

Final Voting Results			
For	**Against**	**Abstain**	**Broker Non-Vote**
130,944,622	2,124,535	841,116	

(4) Proposal to approve the amendment and restatement of the GTx, Inc. 2013 Equity Incentive Plan to increase the maximum number of shares of GTx s common stock subject to appreciation and performance-based awards granted thereunder, as more specifically described in the Proxy Statement. This proposal was approved, with the votes thereon at the Annual Meeting as follows:

Final Voting Results			
For	**Against**	**Abstain**	**Broker Non-Vote**
116,328,892	1,541,708	38,044	16,001,629

(5) Proposal to ratify the appointment of Ernst & Young LLP as GTx s independent registered public accounting firm for the fiscal year ending December 31, 2015. This proposal was approved, with the votes thereon at the Annual Meeting as follows:

Final Voting Results			
For	**Against**	**Abstain**	**Broker Non-Vote**
133,685,393	186,780	38,100	

Table of Contents

ITEM 6. EXHIBITS

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

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned thereunto duly authorized.

GTx, Inc.

Date: May 11, 2015	By:	/s/ Marc S. Hanover Marc S. Hanover, President, Chief Executive Officer ***(Principal Executive Officer)***
Date: May 11, 2015	By:	/s/ Jason T. Shackelford Jason T. Shackelford, Senior Director of Accounting and Corporate Controller and 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+	Certificate of Amendment of Restated Certificate of Incorporation of GTx, Inc.				
3.5	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, 3.4 and 3.5				
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	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
4.8	Form of Common Stock Warrant, issued by Registrant pursuant to the Purchase Agreement, dated November 9, 2014, between Registrant and the purchasers identified in Exhibit A therein	10-K	000-50549	4.9	03/16/2015
10.1	Amended and Restated Employment Agreement dated February 12, 2015, between Registrant and Marc S. Hanover	10-K	000-50549	10.25	03/16/2015
10.2		10-K	000-50549	10.35	03/16/2015

	2015 Compensation Information for Registrant s Executive Officers				
10.3	Non-Employee Director Compensation Policy of GTx, Inc., effective February 12, 2015	10-K	000-50549	10.39	03/16/2015
10.4+	Employment Agreement dated February 12, 2015, between Registrant and Robert J. Wills				
10.5+	Form of Restricted Stock Unit Grant Notice and Restricted Stock Unit Award Agreement under the GTx, Inc. 2013 Equity Incentive Plan				
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)				

Table of Contents

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.