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

FORM 10-Q

(Mark One)

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

For the quarterly period ended June 30, 2017

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, a smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

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

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. o

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

As of August 09, 2017, 16,058,589 shares of the registrant's Common Stock were outstanding.

GTx, INC.
FORM 10-Q FOR THE QUARTER ENDED JUNE 30, 2017
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PART I: FINANCIAL INFORMATION

ITEM 1. FINANCIAL STATEMENTS

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

	June 30, 2017 (unaudited)	December 31, 2016
ASSETS		
Current assets:		
Cash and cash equivalents	$ 5,156	$ 8,910
Short-term investments	6,200	12,959
Prepaid expenses and other current assets	2,130	2,429
Total current assets	13,486	24,298
Property and equipment, net	65	81
Intangible assets, net	116	123
Total assets	$ 13,667	$ 24,502
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	$ 603	$ 1,220
Accrued expenses and other current liabilities	4,582	3,391
Total current liabilities	5,185	4,611
Commitments and contingencies		
Stockholders' equity:		
Common stock, $0.001 par value: 60,000,000 shares authorized at June 30, 2017 and December 31, 2016; 16,041,923 and 15,919,572 shares issued and outstanding at June 30, 2017 and December 31, 2016, respectively	16	16
Additional paid-in capital	552,322	551,073
Accumulated deficit	(543,856)	(531,198)
Total stockholders' equity	8,482	19,891
Total liabilities and stockholders' equity	$ 13,667	$ 24,502

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

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2017	2016	2017	2016
Expenses:				
Research and development expenses	$ 4,448	$ 4,058	$ 8,641	$ 8,029
General and administrative expenses	1,997	1,999	4,084	4,113
Total expenses	6,445	6,057	12,725	12,142
Loss from operations	(6,445)	(6,057)	(12,725)	(12,142)
Other income, net	40	5	67	33
Gain on change in fair value of warrant liability	—	—	—	8,163
Net loss	$ (6,405)	$ (6,052)	$ (12,658)	$ (3,946)
Net loss per share — basic and diluted	$ (0.40)	$ (0.43)	$ (0.79)	$ (0.28)
Weighted average shares outstanding:				
Basic and diluted	16,041,923	14,174,914	16,030,689	14,163,559

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

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

	Six Months Ended June 30,	
	2017	2016
Cash flows from operating activities:		
Net loss	$ (12,658)	$ (3,946)
Adjustments to reconcile net loss to net cash used in operating activities:		
Gain on change in fair value of warrant liability	—	(8,163)
Depreciation and amortization	23	9
Share-based compensation	1,322	1,499
Directors' deferred compensation	83	59
Changes in assets and liabilities:		
Prepaid expenses and other assets	299	569
Accounts payable	(617)	1,005
Accrued expenses and other liabilities	1,191	(298)
Net cash used in operating activities	(10,357)	(9,266)
Cash flows from investing activities:		
Purchase of property and equipment	—	(8)
Purchase of short-term investments, held to maturity	(11,200)	(17,200)
Proceeds from maturities of short-term investments, held to maturity	17,959	22,200
Net cash provided by investing activities	6,759	4,992
Cash flows from financing activities:		
Tax payments related to shares withheld for vested restricted stock units	(156)	(210)
Net cash used in financing activities	(156)	(210)
Net decrease in cash and cash equivalents	(3,754)	(4,484)
Cash and cash equivalents, beginning of period	8,910	14,056
Cash and cash equivalents, end of period	$ 5,156	$ 9,572

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

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1. Business and Basis of Presentation

Business and Going Concern

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 class of drugs that the Company believes has 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 the building of muscle mass may be important, such as stress urinary incontinence ("SUI") and Duchenne muscular dystrophy ("DMD").

In 2016, the Company initiated a Phase 2 open-label, non-placebo controlled, proof-of-concept clinical trial of enobosarm to treat postmenopausal women with SUI. In the second quarter of 2017, the Company announced positive preliminary data from this clinical trial and that an abstract addressing the preliminary data had been accepted for podium presentation at the annual meeting of the International Continence Society (ICS) to be held in Florence, Italy in September 2017. Based on the emerging data from this ongoing proof-of-concept clinical trial, the Company plans to initiate a placebo-controlled Phase 2 clinical trial of enobosarm to treat postmenopausal women with SUI in the second half of 2017.

The Company commenced enrollment in 2015 in a Phase 2 clinical trial designed to evaluate the efficacy and safety of a 9 mg and 18 mg dose of enobosarm in patients whose advanced breast cancer is both estrogen receptor ("ER") positive and androgen receptor ("AR") positive. The Company announced in November 2016 that enobosarm achieved the pre-specified primary efficacy endpoint in the 9 mg dose cohort and anticipates reporting top-line clinical results from this trial in the third quarter of 2017.

During 2015, the Company also commenced enrollment in a Phase 2 proof-of-concept clinical trial designed to evaluate the efficacy and safety of enobosarm in patients with advanced AR positive triple-negative breast cancer ("TNBC"). During the third quarter of 2017, the Company completed its review of the data from the first stage of this clinical trial. While the review of the clinical trial data did not raise any safety concerns, it did confirm that there was insufficient efficacy demonstrated among the treated patients to proceed into the second stage of recruitment of this clinical trial.

The Company has also evaluated several SARM compounds, including enobosarm, in preclinical models of DMD where a SARM's ability to increase muscle mass may prove beneficial to patients suffering from DMD. Based on the Company's SARM data from these preclinical efforts, the Company has initiated discussions with potential collaboration partners to further develop a SARM for the treatment of DMD.

In 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 ("SARD") technology which may have the potential to provide compounds that can degrade multiple forms of AR to treat those patients who do not respond or are resistant to current therapies by inhibiting tumor growth in patients with progressive castration-resistant prostate cancer ("CRPC"). The Company has ongoing preclinical studies to select the most appropriate compound to move into a first-in-human clinical trial.

The Company has evaluated its capital resources and current business plans in accordance with Accounting Standards Update (ASU) No. 2014-15, *Disclosure of Uncertainties about an Entity's Ability to Continue as a Going Concern*, ("ASU 2014-15"). ASU 2014-15 requires the assessment of an entity's ability to continue as a going concern for a period of one year after the date the entity's financial statements are issued and to provide related

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

footnote disclosures, if necessary. As the Company currently estimates, based on its current business plan and assumptions, that its current cash, cash equivalents and short-term investments, together with interest thereon and the advances available to the Company under its loan agreement entered into on August 10, 2017 (described more fully in *Subsequent Events* of this Note 1), will be sufficient to meet its projected operating requirements only into the second quarter of 2018, there is substantial doubt raised about its ability to continue as a going concern. Accordingly, the Company is actively seeking additional funds through potential collaborations, partnering or other strategic arrangements, through additional public or private equity offerings or additional debt financings, or a combination of the foregoing.

In addition, the Company has based its cash sufficiency estimates on its current business plan and its assumptions that may prove to be wrong. The Company could utilize its available capital resources sooner than it currently expects, and it could need additional funding to sustain its operations even sooner than currently anticipated. The Company believes, based on its current estimates of clinical trial expenditures, that the Company's existing capital resources will be adequate to enable it to complete its ongoing Phase 2 clinical trial of enobosarm in patients with ER positive and AR positive advanced breast cancer and its ongoing open-label, non-placebo controlled, proof-of-concept Phase 2 clinical trial of enobosarm in postmenopausal women with SUI. However, the Company's existing capital resources will not be sufficient to allow it to complete its planned placebo-controlled Phase 2 clinical trial of enobosarm to treat postmenopausal women with SUI or to otherwise continue developing enobosarm for any other indication, which it may be unable to do in a timely manner or at all. Also, the Company's clinical trials may encounter technical, enrollment or other difficulties that could increase its development costs beyond its current estimates or delay its development timelines, and the Company could otherwise exhaust its available financial resources sooner than the Company expects. In any event, the Company will need to raise substantial additional capital in order to:

- complete its planned placebo-controlled Phase 2 clinical trial of enobosarm to treat postmenopausal women with SUI;
- undertake any further development of enobosarm in patients with ER positive and AR positive breast cancer beyond our ongoing Phase 2 clinical trial;
- undertake any additional preclinical or clinical development activities related to the development of SARMs as a potential treatment for DMD;
- initiate and complete human clinical studies of the Company's SARD program; and
- fund the Company's operations and any debt repayment and service obligations, and to continue as a going concern.

If the Company is unable to raise substantial additional capital in the near term to fund its operations through and beyond the second quarter of 2018 and to continue as a going concern thereafter, and to fund any debt repayment and service obligations, the Company could be required to, among other things, make further reductions in its workforce, reevaluate its plans to conduct its planned placebo-controlled Phase 2 clinical trial of enobosarm to treat postmenopausal women with SUI, discontinue further development of enobosarm and/or SARDs, liquidate all or a portion of its assets, and/or seek protection under the provisions of the U.S. bankruptcy code, all of which would have a material adverse effect on the Company's business and stock price.

These condensed financial statements do not include any adjustments or charges that might be necessary should the Company be unable to continue as a going concern, such as charges related to impairment of its assets, the recoverability and classification of assets or the amounts and classification of liabilities or other similar adjustments.

Basis of Presentation

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

statements prepared in accordance with accounting principles generally accepted in the United States have been condensed or omitted from the accompanying condensed financial statements. These interim condensed financial statements should be read in conjunction with the audited financial statements and related notes thereto, which are included in the Company's Annual Report on Form 10-K for the year ended December 31, 2016. Operating results for the three and six months ended June 30, 2017 are not necessarily indicative of the results that may be expected for the entire fiscal year ending December 31, 2017.

On December 5, 2016, the Company effected a one-for-ten reverse stock split of its common stock through an amendment to its restated certification of incorporation. As of the effective time of the reverse stock split, every ten shares of the Company's issued and outstanding common stock were automatically combined and reclassified into one issued and outstanding share of common stock, without any change in par value per share. The amendment to the Company's restated certification of incorporation also reduced the number of authorized shares of common stock from 400,000,000 to 60,000,000 shares. The reverse stock split affected all shares of the Company's common stock outstanding immediately prior to the effective time of the reverse stock split. Additionally, as a result of the reverse stock split, proportionate adjustments were made to the per share exercise price and/or the number of shares issuable upon the exercise or vesting of all stock options, restricted stock units and warrants issued by the Company and outstanding immediately prior to the effective time, which resulted in a proportionate decrease in the number of shares of the Company's common stock reserved for issuance upon exercise or vesting of such stock options, restricted stock units and warrants, and, in the case of stock options and warrants, a proportionate increase in the exercise price of all such stock options and warrants. In addition, the number of shares reserved for issuance under the Company's equity compensation plans immediately prior to the effective time was reduced proportionately. No fractional shares were issued as a result of the reverse stock split. Stockholders who have otherwise been entitled to receive a fractional share received a cash payment in lieu thereof. All references to shares of common stock, all per share data, and all warrant, stock option and restricted stock unit ("RSU") activity for all periods presented in these condensed financial statements and notes to the condensed financial statements have been adjusted to reflect the reverse stock split on a retroactive basis.

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 6,430,948 shares of its common stock. The Company classified these warrants as a liability on its balance sheet since the warrants contained certain terms that could have required 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 $1.25 per unexercised share underlying each warrant. As a result of the provision of the warrants requiring cash settlement upon certain change of control transactions, the Company was required to account for these warrants as a liability at fair value and the estimated warrant liability was required to be revalued at each balance sheet date until the earlier of the exercise of the warrants, the modification to remove the provision that could require cash settlement upon certain change of control transactions or the expiration of such provision on December 31, 2016. Effective

March 25, 2016, each of the warrants was amended by agreement of the warrant holders to remove the provision that could require cash settlement upon certain change of control transactions. These warrants were no longer accounted for as a liability subsequent to March 25, 2016. The Company recorded a non-cash reclassification

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of the warrant fair value to stockholders' equity based on the warrants' fair value as of the March 25, 2016 modification date, with no further adjustments to the fair value of these warrants being required.

Fair Value of Financial Instruments

The carrying amounts of the Company's financial instruments (which include cash, cash equivalents, short-term investments, and accounts payable) approximate their fair values. 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

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

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.

Cash, Cash Equivalents and Short-term Investments

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

At June 30, 2017 and December 31, 2016, 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

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deferred tax assets will not be realized. Accordingly, at June 30, 2017 and December 31, 2016, net of the valuation allowance, the net deferred tax assets were reduced to zero. Income taxes are described more fully in Note 8 to the Company's financial statements included in the Company's Annual Report on Form 10-K filed with the Securities and Exchange Commission on March 24, 2017.

Other Income, net

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

(in thousands, except share and per share data)

Subsequent Events

The Company has evaluated all events or transactions that occurred after June 30, 2017 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 August 10, 2017, the Company entered into a $15,000 loan agreement with each of The Pyramid Peak Foundation and J.R. Hyde, III, as described in more detail under Part II, Item 5 of this Quarterly Report on Form 10-Q.

2. Share-Based Compensation

Share-based payments include stock option grants and 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 filed with the Securities and Exchange Commission on March 24, 2017.

During the first quarter of 2017, the Company adopted the Financial Accounting Standards Board Accounting Standards Update 2016-09, *Improvements to Employee Share Based Payment Accounting*. This guidance addresses the income tax effects of stock-based payments and eliminates the windfall pool concept, as all of the tax effects related to stock-based payments will now be recorded at settlement (or expiration) through the income statement. The new guidance also permits entities to make an accounting policy election for the impact of forfeitures on the recognition of expense for stock-based payment awards, allowing for forfeitures to be estimated or recognized when they occur. The Company elected to prospectively adopt the policy that forfeitures be recorded when they occur. The adoption of this guidance did not have a material impact on our financial position or results of operations.

The following table summarizes share-based compensation expense included within the condensed statements of operations for the three and six months ended June 30, 2017 and 2016:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2017	2016	2017	2016
Research and development expenses	$ 181	$ 369	$ 414	$ 663
General and administrative expenses	520	445	991	895
Total share-based compensation	$ 701	$ 814	$ 1,405	$ 1,558

Share-based compensation expense recorded as general and administrative expense for the three months ended June 30, 2017 and 2016 included share-based compensation expense related to deferred compensation arrangements

for the Company's non-employee directors of $42 and $29, respectively. Share-based compensation expense recorded as general and administrative expense for the six months ended June 30, 2017 and 2016 included share-based compensation expense related to deferred compensation arrangements for the Company's non-employee directors of $83 and $59, 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 fair value of options granted was estimated using the following assumptions for the periods presented:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2017	2016	2017	2016
Expected price volatility	91.0%	90.5%	88.5%	91.3%
Risk-free interest rate	2.0%	1.4%	2.2%	2.1%
Weighted average expected life in years	6 years	6 years	7 years	7 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, 2016	1,089,980	$ 27.13
Options granted	942,350	4.69
Options forfeited or expired	(130,775)	30.52
Options outstanding at June 30, 2017	1,901,555	15.77

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.

(in thousands, except share and per share data)

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

	Number of Shares
Nonvested RSUs at December 31, 2016	584,999
RSUs granted	—
RSUs vested	(151,833)
RSUs forfeited	(36,000)
Nonvested RSUs at June 30, 2017	397,166

3. **Basic and Diluted Net Loss Per Share**

Basic and diluted net income (loss) per share attributable to common stockholders is calculated based on the weighted average number of common shares outstanding during the period. Diluted net income (loss) per share

gives effect to the dilutive potential of common stock consisting of stock options, unvested RSUs and common stock warrants.

Weighted average potential shares of common stock of 8,720,616 and 8,160,128 for the three months ended June 30, 2017 and 2016, respectively, and 8,490,229 and 8,179,040 for the six months ended June 30, 2017 and 2016, respectively, were excluded from the calculations of diluted net 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.

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 6,431,111 shares of common stock and warrants to purchase an aggregate of 6,430,948 shares of its common stock for net proceeds of $42,814, after deducting offering expenses. 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 $8.50, became exercisable on May 6, 2015 and will continue to be exercisable for four years thereafter. Prior to May 6, 2015, 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. The warrants, however, also contained certain terms that could have required 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 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 $1.25 per unexercised share underlying each warrant. Due to the provision of the warrants that could have required cash settlement upon certain change of control transactions, the Company was required to account for these warrants as a liability at fair value using the Black-Scholes Model and the estimated warrant liability was required to be revalued at each balance sheet date until the earlier of the exercise of the warrants, the modification to remove the provision that could require cash settlement upon certain change of control transactions or the expiration of such provision on December 31, 2016. Effective March 25, 2016, each of the warrants was amended by agreement of the warrant holders to remove the provision that could require cash settlement upon certain change of control transactions. These warrants were no longer accounted for as a liability at March 31, 2016. The Company recorded a non-cash reclassification of the warrant fair value to stockholders' equity based on the warrants' fair value as of the March 25, 2016 modification date, with no further adjustments to the fair value of these warrants being required. The fair value of the warrants on the March 25, 2016 modification date of $19,186 was estimated using the Black-Scholes Model with the following assumptions: expected volatility of 101%, risk-free interest rate of 1.1%, expected life of approximately 3.1 years and no dividends.

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.

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

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

Forward-Looking Information

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

- the implementation of our business strategies, including our ability to preserve or realize any significant value from our selective androgen receptor modulator, or SARM, and selective androgen receptor degrader, or SARD, programs;
- the therapeutic and commercial potential of, and our ability to advance the development of, SARMs and our SARD program;
- 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 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 preclinical and clinical programs, including whether our ongoing or planned clinical trials will achieve clinically relevant results;
- the timing of regulatory discussions and submissions, and the anticipated timing, scope and outcome of related regulatory actions or guidance;
- our ability to obtain and maintain regulatory approvals of our product candidates and any related restrictions, limitations, and/or warnings in the label of an approved product candidate;
- our ability to market, commercialize and achieve market acceptance for our product candidates;
- our ability to protect our intellectual property and operate our business without infringing upon the intellectual property rights of others; 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," "envisions," "estimates," "expects," "intends," "may," "plans," "potential," "predicts," "projects," "should," "will," "would" and similar expressions intended to identify forward-looking statements. Forward-looking statements reflect our current views with respect to future events, are based on assumptions and are subject to risks, uncertainties and other important factors. We discuss many of these risks in this Quarterly Report on Form 10-Q in greater detail in the section entitled "Risk Factors" under Part II, Item 1A below. Given these risks, uncertainties and other important factors, you should not place undue reliance on these forward-looking statements. Also, forward-looking statements represent our estimates and assumptions only as of the date of this Quarterly Report on Form 10-Q. You should read this Quarterly Report on Form 10-Q and the documents that we incorporate by reference in and have filed as exhibits to this Quarterly Report on Form 10-Q, completely and with the understanding that our actual future results may be materially different from what we expect. Except as required by law, we assume no obligation to update any forward-looking statements publicly, or to update the reasons actual results could differ materially from those anticipated in any forward-looking statements, even if new information becomes available in the future.

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Overview

Business Overview

We are a biopharmaceutical company dedicated to the discovery, development and commercialization of small molecules for the treatment of cancer, including treatments for breast and prostate cancer, and other serious medical conditions, including conditions where the building of muscle mass may be important. Our current strategy is focused on the further development of selective androgen receptor modulators, or SARMs, a class of drugs that we believe has 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 unmet medical needs in muscle-related diseases may benefit from increasing muscle mass, such as stress urinary incontinence, or SUI, and Duchenne muscular dystrophy, or DMD. In 2015, we 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 we believe has the potential to provide compounds that can degrade multiple forms of androgen receptor, or AR, by inhibiting tumor growth in patients with progressive castration-resistant prostate cancer, or CRPC, including those patients who do not respond to or are resistant to current therapies.

Business Highlights

Our lead SARM candidate, enobosarm (GTx-024), has to date been evaluated in 24 completed or ongoing clinical trials, including in six Phase 2 and two Phase 3 clinical trials, enrolling over 1,700 subjects, of which approximately 1,200 subjects were treated with enobosarm. 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 class of compounds.

In 2016, we initiated a Phase 2 open-label, non-placebo controlled, proof-of-concept clinical trial of enobosarm to treat postmenopausal women with SUI. This is the first clinical trial to evaluate a SARM for the treatment of SUI. In this ongoing Phase 2 proof-of-concept clinical trial, enobosarm 3 mg is being assessed as a potential treatment for post-menopausal women who have demonstrated SUI symptoms for more than six months, with 3 to 15 reported SUI episodes per day averaged over a three-day period, and a positive bladder stress test. The primary endpoint for the clinical trial is the number of SUI episodes per day on the 3-day voiding diary at 12 weeks, compared to base line. In the second quarter of 2017, we announced positive preliminary data from this clinical trial and that an abstract addressing the preliminary data had been accepted for podium presentation at the annual meeting of the International Continence Society (ICS) to be held in Florence, Italy in September 2017. The clinical findings reviewed in the abstract were from the first seven patients enrolled in the trial following their completion of treatment:

- Each of the women treated with enobosarm showed a clinically significant reduction (50 percent or greater) in incontinence episodes per day:
  - mean stress leaks decreased by 80.9 percent from baseline over 12 weeks;
  - stress leaks decreased from a mean of 5.7 leaks/day at baseline, to 1.1 leaks/day at Week 12; and
  - all patients saw at least a 65 percent reduction in number of stress leaks
- Reductions in incontinence episodes were sustained well beyond the stopping of study drug at Week 12:
  - patients demonstrated continued reduction in incontinence episodes for up to five months.
- Women reported improved quality of life in the Patient Global Impression of Improvement, or PGI-I, and Female Sexual Function Index, or FSFI:
  - at Week 12, all seven patients showed improved PGI-I scores and 5 of 7 patients showed improvement in FSFI.
- Reported adverse events are minimal with none above Grade I.

Additional data from this ongoing trial will be reviewed during the podium presentation at the ICS conference in September 2017. Based on the emerging data from our Phase 2 open-label, non-placebo controlled, proof-of-concept clinical trial, we plan to initiate a placebo-controlled Phase 2 clinical trial of enobosarm to treat postmenopausal women with SUI in the second half of 2017. However, our ability to complete this planned Phase 2 clinical trial is subject to our ability to obtain additional funding.

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In 2015, we commenced enrollment in a Phase 2 clinical trial designed to evaluate the efficacy and safety of a 9 mg and 18 mg dose of enobosarm in patients whose advanced breast cancer is both estrogen receptor, or ER, positive and AR positive. We announced in November 2016 that enobosarm achieved the pre-specified primary efficacy endpoint in the 9 mg dose cohort and we anticipate reporting top-line clinical results from this trial in the third quarter of 2017.

We also commenced enrollment in 2015 in a Phase 2 proof-of-concept clinical trial designed to evaluate the efficacy and safety of an 18 mg dose of enobosarm in patients with advanced AR positive triple-negative breast cancer, or TNBC. This clinical trial was conducted utilizing a Simon's two-stage trial design whereby if at least 2 of the first 21 patients achieved clinical benefit, the trial was designed to enroll the second stage, which would result in enrolling 41 evaluable patients in the clinical trial. During the third quarter of 2017, we completed our review of the data from the first stage of the clinical trial. While our review of the data did not raise any safety concerns, it did confirm that there was insufficient efficacy demonstrated among the treated patients to proceed into the second stage of recruitment. One patient has continued on treatment in this clinical trial with stable disease for10 months but no other stage 1 patient achieved an acceptable clinical benefit response following 16 weeks of treatment.

We have also evaluated several SARM compounds in preclinical models of DMD where a SARM's ability to increase muscle mass may prove beneficial to patients suffering from DMD, which is a rare disease characterized by progressive muscle degeneration and weakness.

With respect to SARDs, we believe this class of assets has the potential to treat prostate cancer, as well as other diseases such as benign prostatic hyperplasia and Kennedy's disease. We envision initially developing SARDs as a potentially novel treatment for men with CRPC, including those who do not respond or are resistant to currently approved therapies. Our evaluation of the SARD program is at an early stage. We have ongoing preclinical studies to select the most appropriate compound to move into a first-in-human clinical trial. Our ability to pursue the continued development of our SARD program is contingent upon our ability to obtain additional funding.

We are actively seeking additional funds through potential collaborations, partnering or other strategic arrangements, through additional public or private equity offerings or additional debt financings, or a combination of the foregoing, to provide us the necessary resources for the development of our preclinical and clinical product candidates. We have discussions ongoing with several potential collaboration partners who have expressed potential interest in one or more of our development programs.

Financial Highlights

Our net loss for the six months ended June 30, 2017 was $12.7 million. We expect to incur significant operating losses for the foreseeable future as we continue our preclinical and clinical development activities. 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 product revenue for the foreseeable future. We do not expect that any of our product candidates, including enobosarm, will receive any regulatory approvals for the foreseeable future, and it is possible that none of our product candidates will ever receive any regulatory approvals.

At June 30, 2017, we had cash, cash equivalents and short-term investments of $11.4 million compared to $21.9 million at December 31, 2016. On October 14, 2016, we completed a registered direct offering of our common stock, in which we sold 1.7 million shares of our common stock for net proceeds to us of approximately $13.7 million. On August 10, 2017, we entered into a $15.0 million loan agreement, or the Loan Agreement, with J.R. Hyde, III and The Pyramid Peak Foundation, or the Lenders, as described in more detail under "Liquidity and Capital Resources" below.

Based on our current business plan and assumptions, we estimate that our current cash, cash equivalents and short-term investments, together with interest thereon and the advances available to us under our Loan Agreement with the Lenders, will be sufficient to meet our projected operating requirements only into the second quarter of 2018. Accordingly, we need to, and are actively seeking to, raise substantial additional capital in the near term in order to fund our operations through and beyond the second quarter of 2018 and to continue as a going concern thereafter, and to fund any debt repayment and service obligations. In addition, we have based our cash sufficiency

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estimates on our current business plan and our assumptions that may prove to be wrong. We could utilize our available capital resources sooner than we currently expect, and we could need additional funding to sustain our operations even sooner than currently anticipated. We believe, based on our current estimates of clinical trial expenditures, that our existing capital resources will be adequate to enable us to complete our ongoing Phase 2 clinical trial of enobosarm in patients with ER positive and AR positive advanced breast cancer and our ongoing Phase 2 open-label, non-placebo controlled, proof-of-concept clinical trial of enobosarm in postmenopausal women with SUI. However, our existing capital resources will not be sufficient to allow us to complete our planned placebo-controlled Phase 2 clinical trial of enobosarm to treat postmenopausal women with SUI or to otherwise continue developing enobosarm for any other indication, which we may be unable to do in a timely manner or at all. Also, our clinical trials may continue to encounter technical, enrollment or other difficulties that could increase our development costs beyond our current estimates or delay our development timelines, and we could otherwise exhaust our available financial resources sooner than we expect. In any event, we need to raise substantial additional capital in order to:

- complete our planned placebo-controlled Phase 2 clinical trial of enobosarm to treat postmenopausal women with SUI;
- undertake any further development of enobosarm in patients with ER positive and AR positive advanced breast cancer beyond our ongoing Phase 2 clinical trial;
- undertake any additional preclinical or clinical development activities related to the development of SARMs as a potential treatment for DMD;
- initiate and complete human clinical studies of our SARD program; and
- fund our operations and any debt repayment and service obligations, and to continue as a going concern.

In addition, the accompanying financial statements have been prepared assuming that we will continue as a going concern. Accordingly, the accompanying financial statements do not include any adjustments or charges that might be necessary should we be unable to continue as a going concern, such as charges related to impairment of our assets, the recoverability and classification of assets or the amounts and classification of liabilities or other similar adjustments. However, because we estimate that our current cash, cash equivalents and short-term investments, together with interest thereon and the advances available to us under our Loan Agreement with the Lenders, will be sufficient to meet our projected operating requirements only into the second quarter of 2018, there is substantial doubt raised about our ability to continue as a going concern. If we are unable to raise additional funds in the near term to fund our operations through and beyond the second quarter of 2018 and to continue as a going concern thereafter, and to fund any debt repayment and service obligations, we could be required to, among other things, make further reductions in our workforce, reevaluate our plans to complete our planned placebo-controlled Phase 2 clinical trial of enobosarm to treat postmenopausal women with SUI, discontinue further development of enobosarm and/or SARDs, liquidate all or a portion of our assets, and/or seek protection under the provisions of the U.S. bankruptcy code, all of which would have a material adverse effect on our business and stock price.

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 product revenue for the foreseeable future. Apart from advances available to us under the Loan Agreement with the Lenders, we do not have any commitments for future external funding. Accordingly, we are actively seeking additional funds through potential collaborations, partnering or other strategic arrangements, through additional public or private equity offerings or additional 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 may be adversely impacted by the uncertainty regarding the prospects of our development of enobosarm for the treatment of patients with ER positive and AR positive advanced breast cancer and/or SUI and our ability to advance the development of enobosarm 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, recent and potential future management turnover, and continued volatility and instability in the global financial markets. As a result of these and other factors, we cannot be certain that additional funding will be available on acceptable terms, or at all.

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Research and Development

Since our inception in 1997, we have been focused on drug discovery and development programs. Research and development expenses include, but are not limited to, our expenses for personnel and supplies associated with our research activities, screening and identification of product candidates, formulation and synthesis activities, manufacturing, preclinical studies, toxicology studies, clinical trials, regulatory and medical affairs activities, quality assurance activities and license fees.

We expect that our research and development expenses for fiscal year 2017 will increase as compared to fiscal year 2016 primarily due to our ongoing Phase 2 clinical trials of enobosarm in ER positive and AR positive advanced breast cancer and for the treatment of postmenopausal women with SUI, preclinical development of the SARD program, and preparatory activities related to and the initiation of our planned placebo-controlled Phase 2 clinical trial of enobosarm to treat postmenopausal women with SUI in the second half of 2017.

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

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Product Development Programs

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

Product Candidate/ Proposed Indication	Program	Development Phase	Status
Enobosarm Treatment of postmenopausal women with SUI (1 mg and 3 mg)	SARM	Phase 2	Currently enrolling a Phase 2 open-label, non-placebo controlled, proof-of-concept clinical trial evaluating enobosarm (3 mg) in postmenopausal women with SUI with data expected in the third quarter of 2017. Plan to initiate a placebo-controlled Phase 2 clinical trial of enobosarm (1 mg and 3 mg) to treat postmenopausal women with SUI in the second half of 2017.
Enobosarm Treatment of women with ER positive/AR positive advanced breast cancer (9 mg and 18 mg)	SARM	Phase 2	Completed enrollment of a Phase 2 open-label clinical trial evaluating enobosarm in patients whose advanced breast cancer is both ER positive and AR positive. Previously announced clinical benefit was achieved in the 9 mg dose cohort in the ongoing clinical trial. Top-line clinical results for the trial expected in the third quarter of 2017.
Enobosarm Treatment of women with advanced AR positive TNBC (18 mg)	SARM	Phase 2	Currently closing down the Phase 2 open-label proof-of-concept clinical trial evaluating enobosarm in patients with advanced AR positive TNBC as there were insufficient patients achieving clinical benefit from enobosarm treatment to continue the trial.
SARMs Treatment of DMD	SARM	Preclinical	Preclinical research being evaluated by potential collaboration partners for the treatment of DMD.
SARDs Treatment of castration resistant prostate cancer	SARD	Preclinical	Preclinical studies are ongoing to select the most appropriate compound to move into a first-in-human clinical trial.

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.

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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 condensed 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 filed with the Securities and Exchange Commission on March 24, 2017, we believe that the following accounting policies are most critical to aid you in fully understanding and evaluating our reported financial results.

Research and Development Expenses

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

Share-Based Compensation

We have stock option and equity incentive plans that provide for the purchase 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 is based upon 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.

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.

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The following table summarizes share-based compensation expense included within the condensed statements of operations for the three and six months ended June 30, 2017 and 2016:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2017	2016	2017	2016
	(in thousands)		(in thousands)	
Research and development expenses	$ 181	$ 369	$ 414	$ 663
General and administrative expenses	520	445	991	895
Total share-based compensation	$ 701	$ 814	$ 1,405	$ 1,558

Share-based compensation expense recorded in the condensed statement of operations as general and administrative expense for the three months ended June 30, 2017 and 2016 included share-based compensation expense related to deferred compensation arrangements for our non-employee directors of $42,000 and $29,000, respectively. Share-based compensation expense recorded in the condensed statement of operations as general and administrative expense for the six months ended June 30, 2017 and 2016 included share-based compensation expense related to deferred compensation arrangements for our non-employee directors of $83,000 and $59,000, respectively. At June 30, 2017, the total compensation cost related to non-vested stock options not yet recognized was approximately $6.3 million with a weighted average expense recognition period of 3.93 years. At June 30, 2017, the total compensation cost related to non-vested RSUs not yet recognized was approximately $1.1 million with a weighted average expense recognition period of less than one year.

Results of Operations

Three and Six Months Ended June 30, 2017 and 2016

Research and Development Expenses

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

Proposed Candidate / Proposed Indication	Program	Three Months Ended June 30, 2017	Three Months Ended June 30, 2016	Six Months Ended June 30, 2017	Six Months Ended June 30, 2016
		(in thousands)			
Enobosarm					
Treatment of postmenopausal women with SUI (1 mg and 3 mg)	**SARM**	$ 1,494	$ 265	$ 2,283	$ 538
Enobosarm					
Treatment of women with ER positive and AR	**SARM**	1,548	1,846	3,357	3,341

positive advanced breast cancer (9 mg and 18 mg)					
Enobosarm					
Treatment of women with advanced AR positive TNBC (18 mg)	**SARM**	742	1,381	1,627	2,672
Other research and development		664	566	1,374	1,478
Total research and development expenses		$ 4,448	$ 4,058	$ 8,641	$ 8,029

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Research and development expenses increased to $4.4 million for the three months ended June 30, 2017 from $4.1 million for the three months ended June 30, 2016. Research and development expenses increased to $8.6 million for the six months ended June 30, 2017 from $8.0 million for the six months ended June 30, 2016.

Research and development expenses for enobosarm for the treatment of postmenopausal women with SUI increased for the three and six months ended June 30, 2017 from the prior year comparable periods related to expenses of the Phase 2 open-label, non-placebo controlled, proof-of-concept clinical trial of enobosarm to treat postmenopausal women with SUI that initiated enrollment in the first quarter of 2016 and expenses related to preparatory activities for the planned placebo-controlled Phase 2 clinical trial of enobosarm to treat postmenopausal women with SUI, which is planned to begin in the second half of 2017.

Research and development expenses for enobosarm for the treatment of women with ER positive and AR positive advanced breast cancer decreased slightly for the three months ended June 30, 2017 from the prior year comparable period and remained relatively consistent for the six months ended June 30, 2017 from the prior year comparable period due primarily to the timing and nature of activities related to conducting the ongoing Phase 2 clinical trial evaluating enobosarm 9 mg and enobosarm 18 mg in this indication. The clinical trial commenced enrollment during the third quarter of 2015 and completed enrollment in the first quarter of 2017. The prior year period consisted primarily of expenses related to the ongoing Phase 2 clinical trial for the treatment of women with ER positive and AR positive advanced breast cancer and expenses related to the previous Phase 2 proof-of-concept clinical trial evaluating enobosarm 9 mg in women who have previously responded to hormonal therapy for the treatment of their metastatic breast cancer.

Research and development expenses for enobosarm for the treatment of women with AR positive TNBC decreased for both the three and six months ended June 30, 2017 from the prior year comparable periods due to the timing and nature of activities related to conducting the first stage of the ongoing Phase 2 clinical trial, which commenced enrollment during the fourth quarter of 2015. During the third quarter of 2017, we determined that there were insufficient patients achieving clinical benefit from enobosarm treatment to continue this clinical trial and we are in the process of closing down the clinical trial.

“Other research and development” expenses for the three months ended June 30, 2017 increased from the prior year period primarily related to preclinical development of our SARD program partially offset by a decrease in expenses related to our completed Phase 2 clinical trial to evaluate GTx-758 as secondary hormonal therapy in men with metastatic CRPC that was completed in the prior year. During 2016, we determined to discontinue further development of GTx-758. “Other research and development” expenses for the six months ended June 30, 2017 decreased due to the completion of our GTx-758 Phase 2 clinical trial. This decrease was slightly offset by increased spending on preclinical development of our SARD program.

General and Administrative Expenses

General and administrative expenses of $2.0 million and $4.1 million for the three and six months ended June 30, 2017 remained consistent with the prior year comparable periods.

Gain on Change in Fair Value of Warrant Liability

Prior to March 25, 2016, we recognized a warrant liability due to certain provisions of the warrants issued as part of our November 2014 private placement of common stock and warrants. The warrants were required to be accounted for as a liability at fair value and the fair value was required to be revalued at each balance sheet date until the earlier of the exercise of the warrants, the modification to remove the provision that could require cash settlement of the warrants upon certain change of control transactions or the expiration of such provision on December 31, 2016. The resulting non-cash gain or loss on the fair value revaluation at each balance sheet date was recorded as non-operating income in our condensed statement of operations. Effective March 25, 2016, each of the warrants was amended by agreement of the warrant holders to remove the provision that could require cash settlement upon certain change of control transactions. These warrants were no longer accounted for as a liability at March 31, 2016. We recorded a non-cash reclassification of the warrant fair value to stockholders’ equity based on the warrants’ fair value as of the March 25, 2016 modification date, with no further adjustments to the fair value of these warrants being required. The final revaluation of the warrants’ fair value as of March 25, 2016 resulted in a non-cash gain of $8.2 million that was recorded as the change in fair value of warrant liability in our condensed statement of operations for the six months ended June 30, 2017.

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Liquidity and Capital Resources

At June 30, 2017, we had cash, cash equivalents and short-term investments of $11.4 million compared to $21.9 million at December 31, 2016. Net cash used in operating activities was $10.4 million and $9.3 million for the six months ended June 30, 2017 and 2016, respectively, and resulted primarily from

funding our operations.

Net cash provided by investing activities was $6.8 million for the six months ended June 30, 2017 and resulted primarily from the maturities of short-term investments of $18.0 million offset by the purchase of short-term investments of $11.2 million. Net cash provided by investing activities was $5.0 million for the six months ended June 30, 2016 and resulted from the maturities of short-term investments of $22.2 million offset by the purchase of short-term investments of $17.2 million.

Net cash used in financing activities for the six months ended June 30, 2017 was $156,000 for tax payments related to shares withheld for vested restricted stock units. Net cash used in financing activities for the six months ended June 30, 2016 was $210,000 for tax payments related to shares withheld for vested restricted stock units.

On August 10, 2017, we entered into a loan agreement with J.R. Hyde, III and The Pyramid Peak Foundation (the "Loan Agreement") to borrow up to a total of $15.0 million (the "Loans"). Each of Mr. Hyde and The Pyramid Peak Foundation (the "Lenders") was reported as a greater than 5% holder of shares of our common stock in our 2017 Proxy Statement filed with the Securities and Exchange Commission on March 31, 2017. Additionally, Mr. Hyde serves on our board of directors.

Under the Loan Agreement, we can borrow in one or more advances up to a total of $15.0 million. Advances can be prepaid in whole or in part without penalty or premium and may be reborrowed. Interest will accrue monthly on each outstanding advance at a fixed rate of 8.00% and will be payable, together with all principal amounts outstanding, on the earlier of (a) May 10, 2018 (the "Maturity Date") or (b) the date on which we consummate an equity financing resulting in gross proceeds of at least $15,000,000 (a "Qualified Financing"). Events of default under the Loan Agreement include (a) the failure to pay principal or interest when due, (b) the commencement of a voluntary or involuntary bankruptcy or insolvency action and (c) the breach of any representation or warranty contained in the Loan Agreement by us. If an event of default occurs, all unpaid principal and interest will become immediately due and payable. Any borrowings under the Loan Agreement will be unsecured and the Loan Agreement contains no financial covenants. The Loan Agreement contains a covenant by the Lenders to participate, at our option, in any Qualified Financing in amounts up to their respective principal and interest amounts outstanding.

Based on our current business plan and assumptions, we estimate that our current cash, cash equivalents and short-term investments, together with interest thereon and the advances available to us under our Loan Agreement, will be sufficient to meet our projected operating requirements only into the second quarter of 2018. Accordingly, we need to, and are actively seeking to, raise substantial additional capital in the near term in order to fund our operations through and beyond the second quarter of 2018 and to continue as a going concern thereafter, and to fund any debt repayment and service obligations. In addition, we have based our cash sufficiency estimates on our current business plan and our assumptions that may prove to be wrong. We could utilize our available capital resources sooner than we currently expect, and we could need additional funding to sustain our operations even sooner than currently anticipated. We believe, based on our current estimates of clinical trial expenditures, that our existing capital resources will be adequate to enable us to complete our ongoing Phase 2 clinical trial of enobosarm in patients with ER positive and AR positive advanced breast cancer and our ongoing Phase 2 open-label, non-placebo controlled, proof-of-concept clinical trial of enobosarm in postmenopausal women with SUI. However, our existing capital resources will not be sufficient to allow us to complete our planned placebo-controlled Phase 2 clinical trial of enobosarm to treat postmenopausal women with SUI or to otherwise continue developing enobosarm for any other indication, which we may be unable to do in a timely manner or at all. Also, our clinical trials may continue to encounter technical, enrollment or other difficulties that could increase our development costs beyond our current estimates or delay our development timelines, and we could otherwise exhaust our available financial resources sooner than we expect. In any event, we need to raise substantial additional capital in order to:

- complete our planned placebo-controlled Phase 2 clinical trial of enobosarm to treat postmenopausal women with SUI;

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- undertake any further development of enobosarm in patients with ER positive and AR positive advanced breast cancer beyond our ongoing Phase 2 clinical trial;
- undertake any additional preclinical or clinical development activities related to the development of SARMs as a potential treatment for DMD;
- initiate and complete human clinical studies of our SARD program; and
- fund our operations and any debt repayment and service obligations, and to continue as a going concern.

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 enobosarm;
- 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 product revenue for the foreseeable future. Apart from the advances remaining available to us under the Loan Agreement with the Lenders, we do not have any commitments for future external funding. Accordingly, we are actively seeking additional funds through potential collaborations, partnering or other strategic arrangements, through additional public or private equity offerings or additional debt financings, or a combination of the foregoing.

In addition, the accompanying financial statements have been prepared assuming that we will continue as a going concern. Accordingly, the accompanying financial statements do not include any adjustments or charges that might be necessary should we be unable to continue as a going concern, such as charges related to impairment of our assets, the recoverability and classification of assets or the amounts and classification of liabilities or other similar adjustments. However, because we estimate that our current cash, cash equivalents and short-term investments, together with interest thereon and the advances available to us under our Loan Agreement with the Lenders, will be sufficient to meet our projected operating requirements only into the second quarter of 2018, there is substantial doubt raised about our ability to continue as a going concern. If we are unable to raise additional funds in the near term to fund our operations through and beyond the second quarter of 2018 and to continue as a going concern thereafter, and to fund any debt repayment and service obligations, we could be required to, among other things, make further reductions in our workforce, reevaluate our plans to complete our planned placebo-controlled Phase 2 clinical trial of enobosarm to treat postmenopausal women with SUI, discontinue further development of enobosarm and/or SARDs, liquidate all or a portion of our assets, and/or seek protection under the provisions of the U.S. bankruptcy code, all of which would have a material adverse effect on our business and stock price.

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To the extent that we raise additional funds through potential collaborations, 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 SARMs 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, completed a subsequent private placement in November 2014 that represented additional dilution, and we again raised additional funds by issuing shares of common stock in a registered direct offering in October 2016. Our stockholders may experience additional, perhaps substantial, dilution should we again raise additional funds by issuing equity securities. Any additional debt or equity financing 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 may be adversely impacted by the uncertainty regarding the prospects of our development of enobosarm for the treatment of patients with ER positive and AR positive advanced breast cancer and/or SUI and our ability to advance the development of enobosarm 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, recent and potential future management turnover, and continued volatility and instability in the global financial markets. As a result of these and other factors, we cannot be certain that additional funding will be available on acceptable terms, or at all.

Contractual Obligations

Our future minimum contractual obligations were reported in our Annual Report on Form 10-K as filed with the Securities and Exchange Commission on March 24, 2017. There were no material changes during the six months ended June 30, 2017 from the contractual obligations previously disclosed in our Annual Report on Form 10-K filed with the Securities and Exchange Commission on March 24, 2017. As noted above, the Company entered into the Loan Agreement (as described in Part II, Item 5 of this Quarterly Report) on August 10, 2017.

ITEM 3. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK

During the six months ended June 30, 2017, 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 filed with the Securities and Exchange Commission on March 24, 2017.

ITEM 4. CONTROLS AND PROCEDURES

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

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

There were no changes in our internal control over financial reporting during the second quarter of 2017 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

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PART II: OTHER INFORMATION

ITEM 1A. RISK FACTORS

We have identified the following 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 24, 2017.

Risks Related to Our Financial Condition and Need for Additional Financing

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

As of June 30, 2017, we had an accumulated deficit of $543.9 million. Our net loss for the six months ended June 30, 2017 was $12.7 million and we expect to incur significant operating losses for the foreseeable future as we continue our preclinical and 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 candidate, enobosarm (GTx-024), will require significant additional clinical development, financial resources and personnel in order to obtain necessary regulatory approvals for this product candidate and to develop it and our other SARMs into commercially viable products. A substantial portion of our efforts and expenditures were previously 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. Our current strategy is focused on the further development of enobosarm for the treatment of postmenopausal women with stress urinary incontinence, or SUI, and for the treatment of patients with estrogen receptor, or ER, positive and androgen receptor, or AR, positive advanced breast cancer. However, the development of enobosarm for the treatment of patients in these indications is at an early stage, is subject to the substantial risk of failure inherent in the development of early-stage product candidates, and will require significant additional financial resources and personnel in order for such development to continue. With regard to our remaining programs, our preclinical evaluation of our selective androgen receptor degrader, or SARD, technology, and our preclinical evaluation of SARMs as a potential treatment of Duchenne muscular dystrophy, or DMD, will in each case require significant additional financial resources and personnel to continue our development of these programs. 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 any 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 are unable to raise substantial additional capital in the near term to fund our operations through and beyond the second quarter of 2018 and to continue as a going concern thereafter, if we and/or any potential collaborators are unable to develop and commercialize SARMs or SARD technology, if development is further

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delayed or is eliminated, or if sales revenue from any SARM or SARD products upon receiving marketing approval, if ever, is insufficient, we may never become profitable and we will not be successful.

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

At June 30, 2017, we had cash, cash equivalents and short-term investments of $11.4 million. Based on our current business plan and assumptions, we estimate that our current cash, cash equivalents and short-term investments, together with interest thereon and the advances available to us under our loan arrangement, or the Loan Agreement, entered into on August 10, 2017 with J.R. Hyde III and The Pyramid Peak Foundation, or the Lenders, will be sufficient to meet our projected operating requirements only into the second quarter of 2018. Accordingly, we need to, and are actively seeking to, raise substantial additional capital in the near term in order to fund our operations through and beyond the second quarter of 2018 and to continue as a going concern thereafter, and to fund any debt repayment and service obligations. In addition, we have based our cash sufficiency estimates on our current business plan and our assumptions that may prove to be wrong. We could utilize our available capital resources sooner than we currently expect, and we could need additional funding to sustain our operations even sooner than currently anticipated. We believe, based on our current estimates of clinical trial expenditures, that our existing capital resources will be adequate to enable us to complete our ongoing Phase 2 clinical trial of enobosarm in patients with ER positive and AR positive advanced breast cancer and our ongoing Phase 2 open-label, non-placebo controlled, proof-of-concept clinical trial of enobosarm in postmenopausal women with SUI. However, our existing capital resources will not be sufficient to allow us to complete our planned placebo-controlled Phase 2 clinical trial of enobosarm to treat postmenopausal women with SUI or to otherwise continue developing enobosarm for any other indication, which we may be unable to do in a timely manner or at all. Also, our clinical trials may continue to encounter technical, enrollment or other difficulties that could increase our development costs beyond our current estimates or delay our development timelines, and we could otherwise exhaust our available financial resources sooner than we expect. In any event, we need to raise substantial additional capital in order to:

- complete our planned placebo-controlled Phase 2 clinical trial of enobosarm to treat postmenopausal women with SUI;
- undertake any further development of enobosarm in patients with ER positive and AR positive advanced breast cancer beyond our ongoing Phase 2 clinical trial;
- undertake any additional preclinical or clinical development activities related to the development of SARMs as a potential treatment for DMD;
- initiate and complete human clinical studies of our SARD program; and
- fund our operations and any debt repayment and service obligations, and to continue as a going concern.

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 enobosarm;
- 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 product revenue for the foreseeable future. Apart from the advances available to us under the Loan Agreement with the Lenders, we do not have any commitments for future external funding. Accordingly, we are actively seeking additional funds through potential collaborations, partnering or other strategic arrangements, through additional public or private equity offerings or additional debt financings, or a combination of the foregoing.

In addition, the accompanying financial statements have been prepared assuming that we will continue as a going concern. Accordingly, the accompanying financial statements do not include any adjustments or charges that might be necessary should we be unable to continue as a going concern, such as charges related to impairment of our assets, the recoverability and classification of assets or the amounts and classification of liabilities or other similar adjustments. However, because we estimate that our current cash, cash equivalents and short-term investments, together with interest thereon and the advances available to us under our Loan Agreement with the Lenders, will be sufficient to meet our projected operating requirements only into the second quarter of 2018, there is substantial doubt raised about our ability to continue as a going concern. If we are unable to raise additional funds in the near term to fund our operations through and beyond the second quarter of 2018 and to continue as a going concern thereafter, and to fund any debt repayment and service obligations, we could be required to, among other things, make further reductions in our workforce, reevaluate our plans to complete our planned placebo-controlled Phase 2 clinical trial of enobosarm to treat postmenopausal women with SUI, discontinue further development of enobosarm and/or SARDs, liquidate all or a portion of our assets, and/or seek protection under the provisions of the U.S. bankruptcy code, all of which would have a material adverse effect on our business and stock price.

To the extent that we raise additional funds through potential collaborations, 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 SARMs 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, completed a subsequent private placement in November 2014 that represented additional dilution, and we again raised additional funds by issuing shares of common stock in a registered direct offering in October 2016. Our stockholders may experience additional, perhaps substantial, dilution should we again raise additional funds by issuing equity securities. Any additional debt or equity financing 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 may be adversely impacted by the uncertainty regarding the prospects of our development of enobosarm for the treatment of patients with ER positive and AR positive advanced breast cancer and/or SUI and our ability to advance the development of enobosarm 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, recent and potential future management turnover, and continued volatility and instability in the global financial markets. As a result of these and other factors, we cannot be certain that additional funding will be available on acceptable terms, or at all.

Risks Related to Development of Product Candidates

We are substantially dependent on the success of enobosarm and our failure to 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 postmenopausal women with SUI and for the treatment of patients with ER positive and AR positive advanced breast cancer. We are evaluating enobosarm in these indications in two ongoing Phase 2 clinical trials,

and plan to initiate an additional placebo-controlled Phase 2 clinical trial of enobosarm to treat postmenopausal women with SUI. Even if our ongoing and planned clinical trials are successful, we will still need to conduct costly and time-consuming additional clinical trials of enobosarm in these indications to determine whether enobosarm is an effective treatment for patients with ER positive and AR positive advanced breast cancer or for postmenopausal women with SUI.

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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 ongoing Phase 2 clinical trial of enobosarm in patients with ER positive and AR positive advanced breast cancer and our ongoing and planned Phase 2 clinical trials of enobosarm to treat postmenopausal women with SUI. Furthermore, the positive results from our Phase 2 proof-of-concept clinical trial evaluating enobosarm 9 mg in women whose advanced breast cancer is both ER positive and AR positive does not ensure that our ongoing Phase 2 clinical trial of enobosarm in patients with ER positive and AR positive advanced breast cancer will be successful or that any later trials will be successful. Likewise, the fact that we achieved clinical benefit in the 9 mg cohort for both the first and second stages of our ongoing Phase 2 clinical trial of enobosarm in patients with ER positive and AR positive advanced breast cancer and achieved the first stage milestone in the 18 mg cohort in this trial does not ensure that this trial will be successful. Similarly, the fact that we reported positive preliminary data from our Phase 2 open-label, non-placebo controlled, proof-of-concept clinical trial evaluating enobosarm in postmenopausal women with SUI does not ensure that either this trial or our planned placebo-controlled Phase 2 clinical trial of enobosarm to treat postmenopausal women with SUI will be successful. For example, even though we reported positive results from our Phase 2 proof-of-concept clinical trial evaluating enobosarm 9 mg in women whose advanced breast cancer is both ER positive and AR positive, we determined during the third quarter of 2017 that enobosarm treatment did not sufficiently prove to be an efficacious treatment for patients with TNBC. Accordingly, we are in the process of closing down this clinical trial. 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 ER positive and AR positive advanced breast cancer or for the treatment of postmenopausal women with SUI, or in developing or partnering 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.

Our evaluation of our SARD program is at an early stage and to initiate and complete initial human clinical trials, we will require additional funding. In addition, our evaluation of SARMs as a potential treatment for DMD is at a very early stage, and our ability to meaningfully advance development of SARMs as a potential treatment for DMD is subject to our ability to obtain additional funding, either through financing or by entering into new collaborative arrangements or other strategic transactions with third parties for any such further development.

Accordingly, our current strategy and near-term prospects are substantially dependent on the successful development of enobosarm for the treatment of postmenopausal women with SUI and for the treatment of patients with ER positive and 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. In this regard, from time to time, we have and may in the future publish or report interim or preliminary data from our clinical trials, including the preliminary data we reported in the second quarter of 2017 from our Phase 2 proof-of-concept clinical trial of enobosarm to treat postmenopausal women with SUI. Interim or preliminary data from clinical trials that we may conduct may not be indicative of the final results of the trial and are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and more patient data become available. Interim or preliminary data also remain subject to audit and verification procedures that may result in the final data being materially

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different from the interim or preliminary data. As a result, interim or preliminary data should be viewed with caution until the final data are available.

Typically, the failure rate for development candidates is high. If a product candidate fails at any stage of development, we will not have the anticipated revenues from that product candidate to fund our operations, and we will not receive any return on our investment in that product candidate. For example, during the third quarter of 2017, we determined that there were insufficient patients achieving clinical benefit from enobosarm treatment to continue our Phase 2 proof-of-concept clinical trial evaluating enobosarm in patients with advanced AR positive triple-negative breast cancer, or TNBC. Accordingly, we are in the process of closing down this clinical trial. In addition, 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.

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 SARD program is at an early stage and it is possible that we may determine not to move forward with any meaningful preclinical development of our SARD program. Even if we do determine to move forward with any meaningful preclinical development of our SARD program, to initiate and complete initial human clinical trials, 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 our ongoing or planned clinical trials will need to be modified or will be completed on schedule, if at all. We or any potential collaborators may experience numerous unforeseen and/or adverse events during, or as a result of, preclinical testing and the clinical trial process that could delay or prevent our or our potential collaborators' ability to commercialize our product candidates, including:

- regulators or institutional review boards may not authorize us or any potential collaborators to commence a clinical trial or conduct a clinical trial at a prospective trial site, or we may experience substantial delays in obtaining these authorizations;
- preclinical or clinical trials may produce negative or inconclusive results, which may require us or any potential collaborators to conduct additional preclinical or clinical testing or to abandon projects that we expect to be promising;
- even if preclinical or clinical trial results are positive, the FDA or foreign regulatory authorities could nonetheless require us to conduct unanticipated additional clinical trials;
- registration or enrollment in clinical trials may be slower than we anticipate resulting in significant delays, additional costs and/or study terminations;
- we or any potential collaborators may suspend or terminate clinical trials if the participating patients are being exposed to unacceptable health risks;
- regulators or institutional review boards may suspend or terminate clinical research for various reasons, including noncompliance with regulatory requirements; and
- our product candidates may not have the desired effects or may include undesirable side effects.

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

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

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In our Phase 2 clinical trials for enobosarm for the treatment of muscle wasting in patients with cancer and healthy older males and postmenopausal females, we observed mild elevations of hepatic enzymes, which in certain circumstances may lead to liver failure, in a few patients in both the placebo and enobosarm treated groups. Reductions in high-density lipoproteins, 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 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, a serious adverse event, or SAE, was assessed as possibly related to enobosarm. Although doses up to 30 mg have been evaluated in short duration studies, doses of 9 mg and 18 mg currently being tested in our ongoing 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.

In three Phase 2 clinical trials of GTx-758, we observed venous thromboembolic events (VTEs), 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, and in this trial, there was one reported incidence of a VTE and one reported incidence of a myocardial infarction, or MI, in patients enrolled in the 250 mg arm of the trial, resulting in the discontinuation of both patients from active treatment. We have determined to discontinue further development of GTx-758 and we do not expect to receive any return on our investment from this product candidate.

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

- we or any potential collaborators may be required to conduct additional preclinical or clinical trials, make changes in the labeling of any such approved products, reformulate any such products, or implement changes to or obtain new approvals of our contractors' manufacturing facilities;
- regulatory authorities may be unwilling to approve our product candidates or may withdraw approval of our products;
- we may experience a significant drop in the sales of the affected products;
- our reputation in the marketplace may suffer; and
- we may become the target of lawsuits, including class action suits.

Any of these events could prevent approval or harm sales of the affected product candidates or products, or could substantially increase the costs and expenses of commercializing and marketing any such products.

Risks Related to Our Dependence on Third Parties

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

Our strategy includes selectively partnering or collaborating with leading pharmaceutical and biotechnology companies to assist us in furthering development and potential commercialization of our product candidates and to provide funding for such activities. 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

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curtail the development of a particular product candidate, reduce, delay, or terminate its development or one or more of our other development programs, delay its potential commercialization or reduce the scope of our sales or marketing activities or increase our expenditures and undertake development or commercialization activities at our own expense. For example, we may have to cease further development of our enobosarm program if we are unable to raise sufficient funding for any additional clinical development of enobosarm through new collaborative arrangements or other strategic transactions with third parties or other financing alternatives. In this regard, if we decide to undertake any further development of our SARMs beyond our ongoing and planned clinical trials and preclinical development, we would need to obtain additional funding for such development, either through financing or by entering into new collaborative arrangements or other strategic transactions with third parties for any such further development. Moreover, we will need additional funding in order to complete our planned placebo-controlled Phase 2 clinical trial of enobosarm to treat postmenopausal women with SUI. Likewise, to initiate and complete initial human clinical trials for our SARD program, we will require additional funding. In addition, our evaluation of SARMs as a potential treatment for DMD is at an early stage, and our ability to meaningfully advance development of SARMs as a potential treatment for DMD is subject to our ability to obtain additional funding. There can be no assurances that we will be successful in obtaining additional funding in any event. If we are not able to raise substantial additional capital, either through financing or by entering into new collaborative arrangements or other strategic transactions with third parties for the further development of our product candidates, 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. As of the date of this report, we have no ongoing collaborations for the development and commercialization of our product candidates. We may not be able to locate third-party collaborators to develop and market our product candidates, and we lack the capital and resources necessary to develop our product candidates alone.

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

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

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

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

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candidates on a timely and competitive basis.

We rely on third-party vendors for the manufacture of SARM and SARD drug substance. If the contract manufacturers that we are currently utilizing to meet our supply needs for enobosarm or any future SARM or SARD 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 or SARD product candidates. 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 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 our product candidates.

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

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

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

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

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

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

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

Risks Related to Our Intellectual Property

If we lose our licenses from UTRF, we may be unable to continue our business.

We have licensed intellectual property rights and technology from UTRF used in substantially all 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

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and/or SARD technology and intellectual property covered by those agreements to market, distribute and sell licensed products, which may prevent us from continuing our business and may cause us to cease operations altogether.

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.

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 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

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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 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 other toremifene-based 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 input from the MHRA, we determined that the data from the POWER trials was

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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 elected not 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 would not accept a NDA for enobosarm 3 mg for the prevention and treatment of muscle wasting in patients with advanced NSCLC.

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 ER positive and AR positive advanced breast cancer or for the treatment of postmenopausal women with SUI 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 future clinical trials of enobosarm, which could require us to abandon the development of enobosarm.

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

We do not expect to receive regulatory approval for the commercial sale of any of our product candidates that are in development for the foreseeable future, if at all. The inability to obtain approval from the FDA, the EMA and other foreign regulatory authorities for our product candidates would prevent us or any potential collaborators from commercializing these product candidates in the United States, the EU, or other countries. See the section entitled “Business — Government Regulation” under Part 1, Item 1 of our Annual Report on Form 10-K, filed with the SEC on March 24, 2017, for additional information regarding risks associated with marketing approval, as well as risks related to potential post-approval requirements.

Risks Related to Commercialization

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

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

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

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

We have limited experience as a company in the sales, marketing and distribution of pharmaceutical products. In the event one of our product candidates is approved, we will need to establish sales and marketing capabilities or establish and maintain agreements with third parties to market and sell our product candidates. We may be unable to build our own sales and marketing capabilities, and there are risks involved with entering into arrangements with

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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, both governmental and private. 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, or Healthcare Reform Act. The Healthcare Reform Act, among other initiatives, 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.

In January 2017, the United States House of Representatives and Senate passed legislation, the concurrent budget resolution for fiscal year 2017, which initiates actions that would repeal certain aspects of the Healthcare Reform Act. Further, on January 20, 2017, President Trump signed an Executive Order directing federal agencies with authorities and responsibilities under the Healthcare Reform Act, to waive, defer, grant exemptions from, or delay the implementation of any provision of the Healthcare Reform Act that would impose a fiscal or regulatory burden on states, individuals, healthcare providers, health insurers, or manufacturers of pharmaceuticals or medical devices. In May 2017, following the passage of the budget resolution for fiscal year 2017, the U.S. House of Representatives passed legislation known as the American Health Care Act, or the AHCA, which, if enacted, will amend and repeal significant portions of the Healthcare Reform Act. However, the U.S. Senate is unlikely to adopt the AHCA as passed by the U.S. House of Representatives. The U.S. Senate considered but was unable to adopt other legislation to amend and/or replace elements of the Healthcare Reform Act. We continue to evaluate the effect that the Healthcare Reform Act and its possible repeal and replacement has on our business. This legislation and other healthcare reform measures that may be adopted in the future could have a material adverse effect on our industry generally and on our ability to successfully commercialize our product candidates, if approved.

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.

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

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

Among policy makers and payors in the United States and elsewhere, there is significant interest in health care reform, as evidenced by the initial enactment of, as well as the efforts to repeal and replace the Healthcare Reform Act in the United States. Aside from the possible repeal and replacement of the Healthcare Reform Act, 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. For example, there has been increasing legislative and enforcement interest in the United States with respect to specialty drug pricing practices. Specifically, there have been several recent U.S. Congressional inquiries and legislative and administrative initiatives at the federal and state levels intended to, among other things, bring more transparency to drug pricing and modify government program reimbursement for drugs. We cannot predict what health care reform initiatives may be adopted in the future. Further federal, state and foreign legislative and regulatory developments are likely, and we expect ongoing initiatives to increase pressure on drug pricing, which 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.

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

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

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

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

We have product liability insurance that covers our clinical trials and any commercial products up to a $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

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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 are developing enobosarm for the treatment of patients with ER positive and AR positive advanced breast cancer. Radius Health, Inc. has stated that it is testing its SARM compound, RAD140, in breast cancer. To our knowledge, no other SARMs are currently in development for the treatment of ER positive and AR positive advanced breast cancer; however, other companies with SARMs in development for muscle wasting and cachexia could enter into a breast cancer program 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. ARAs in development include XTANDI® (enzalutamide) being developed by Medivation Inc., which was recently acquired by Pfizer Inc., and Astellas Pharma, Inc., VT 464 being developed by Innocrin Pharmaceuticals Inc., 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. In ER positive breast cancer, a number of targeted therapies are being developed to be used in combination with other hormonal agents. These therapies include CDK 4/6 inhibitors (palbociclib being developed by Pfizer and ribociclib being developed by Novartis have been approved by FDA, and Lilly has completed Phase 3 trials of abemaciclib and plans to submit an NDA), PI3K/AKT inhibitors (BKM120 and BYL719 being developed by Novartis, Taselisib being developed by Roche), and mTOR inhibitors (Everolimus being developed by Novartis (FDA approved) AZD2014 (AstraZeneca) and MLN0128 (Takeda) are in Phase II trials). In ER positive breast cancer, new selective estrogen receptor modulators and selective estrogen receptor degraders targeting the estrogen receptor are in development, including GDC-0927 (Roche), RAD 1901 (Radius Pharmaceuticals), and AZD9496 (Astra Zeneca).

We initiated a Phase 2 open-label, non-placebo controlled, proof-of-concept clinical trial of enobosarm to treat postmenopausal women with SUI. There are a variety of treatments that may be used for SUI in women; however, currently, there are no available oral agents approved for the treatment of SUI. Behavioral modification and pelvic floor physical therapy are common initial treatment approaches. Bulking agents, including carbon coated beads (Durasphere® marketed by Coloplast Corp), calcium hydroxlapatite (Coaptite® marketed by BioForm Medical, Inc.) and silicon (Macroplastique® marketed by Cogentix Medical), can be injected into or around the urethra for treating intrinsic sphincter deficiency, a cause of SUI symptoms. Biologic bulking agents including patient-derived adipose stem cells and autologous muscle-derived stem cells (Cook Myosite) are being developed. Recently, an over-the-counter vaginal pessary (Impressa® marketed by Kimberly-Clark) has been approved for the temporary management of urine leakage in women with SUI. Finally, surgical procedures (e.g. sling; bladder neck suspension) have been demonstrated to be effective in some women.

We are also exploring the potential of SARMs to treat DMD. DMD is a rare genetic disorder which currently has no cure and leads to a progressive weakening of all the muscles in the body. A number of drugs are in various stages of development by pharmaceutical companies to meet the unmet medical need in DMD. These drugs may compete for patient enrollment during the clinical trial phase, should we be able to advance the development of SARMs as a potential treatment of DMD, or commercially if approved. The most advanced development is by those companies who are targeting the genetic mutation with exon skipping or codon blocking therapies including eteplirsen by Sarepta Therapeutics Inc. (which recently received FDA approval) and DS-1541b, by

Daiichi Sankyo Co. Marathon Pharmaceuticals LLC recently received FDA approval for a glucocorticoid, deflazacort, which was recently acquired from Marathon by PTC Therapeutics. Santhera Pharmaceuticals has completed a Phase 3 trial with a synthetic analog of coenzyme Q_{10}, idebenone. Eli Lilly and Company completed a Phase 3 trial with tadalafil, a PDE5 inhibitor, although the study did not meet its primary endpoint. Pfizer Inc. is developing its anti-

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myostatin monoclonal antibody, PF-06252616, and is currently in a Phase 2 trial. Bristol Myers Squibb Company is developing BMS 986089, an anti-myostatin adnectin, and currently has a Phase 2 trial ongoing. Italfarmco S.p.A. has a Phase 2 trial ongoing with givinostat, an HDAC inhibitor. Summit Therapeutics PLC has initiated a Phase 2 trial with ezutromid, an utrophin upregulator. Cardero Therapeutics Inc. is planning a Phase 2 trial with epicatechin, a flavanol. In addition, Akashi Therapeutics is developing two compounds for DMD, one of which is a SARM. Tarix Orphan is developing TXA127, an angiotensin 1-7 peptide. Fibrogen is developing FG-3019, a monoclonal antibody which inhibits connective tissue growth factor. Catabasis Pharmaceuticals Inc. is developing CAT-1004, an NF-KB inhibitor. ReveraGen Biopharma Inc. has initiated a Phase 2 trial in DMD with VPB 15, a novel glucocorticoid. Capricor Therapeutics has an ongoing Phase 1/2 trial with CAP 1002, cardiosphere derived cells.

We have entered into an exclusive worldwide license agreement with UTRF to develop its proprietary SARD technology which we believe has the potential to provide compounds that can degrade multiple forms of the AR by inhibiting tumor growth in patients with CRPC, including those patients who do not respond or are resistant to current therapies. Drugs in commercial development having potentially similar approaches to removing the AR by degradation include Arvinas Inc.'s ARV-330, which is a chimera with an AR binding moiety on one end and an E3 ligase recruiting element on the other that is in preclinical development for the treatment of advanced prostate cancer and Androscience Corporation's androgen receptor degrader enhancer, or ARD, which is currently in development for acne and alopecia with the potential for development as a treatment for prostate cancer. Additionally, Essa Pharma Inc. is beginning early studies with EPI-506, an AR antagonist that targets the N-terminal domain of the AR. C4 Therapeutics, Inc. is developing degronimids as means to degrade the AR through the ligand binding domain associated degradation. CellCentric is developing therapies that target the histone methyltransferase enzyme to lower AR levels and Oric Pharmaceuticals is targeting the glucocorticoid receptor as a means to impact men that have CRPC. 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. Pfizer and Astellas Pharma market XTANDI® (enzalutamide), an oral androgen receptor antagonist, for the treatment of metastatic CRPC 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. Similarly, Johnson & Johnson acquired Aragon Pharmaceuticals, Inc., which developed a second generation anti-androgen apalutamide (ARN-509) that is currently being evaluated in Phase 3 studies in men with progressive, advanced prostate cancer. Bayer HealthCare and Orion Corporation are currently performing a Phase 3 study of darolutamide (ODM-201) in men with CRPC without metastases and with a rising PSA examining safety and efficacy by measuring metastatic free survival. In addition to targeting the androgen receptor, therapeutic approaches are being developed to target the progesterone receptor in these patients by Arno Therapeutics Inc.

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.

Over the past few years, we have experienced significant changes in executive leadership, and more could occur. For example, on April 3, 2014, Marc S. Hanover was appointed as our interim Chief Executive Officer and on February 12, 2015, Mr. Hanover was appointed as our permanent Chief Executive Officer. Also, on March 2, 2015, Robert J. Wills was appointed as our Executive Chairman and effective July 13, 2015, Diane C. Young joined us as our Vice President, Chief Medical Officer.

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.

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

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 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, proprietary or protected health 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 26 employees remained as employees of GTx as of June 30, 2017. Accordingly, we have been and are operating with a shortage of resources and may not be able to effectively conduct our operations with this limited number of employees. In addition, we announced past workforce reductions in each of December 2009 and June 2011, and our history of implementing workforce reductions, along with the potential for future workforce reductions, may negatively affect our ability to retain or attract talented employees. Further, to the extent we experience additional management transition, competition for top management is high and it may take many months to find a candidate that meets our requirements. If we are unable to attract and retain qualified management personnel, our business could suffer.

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

If we are able to raise sufficient additional funds necessary to continue as a going concern and to pursue the development of our SARM and SARD programs, we may need to hire experienced personnel to develop and commercialize our product candidates and to otherwise grow our business, and we may need to expand the number of our managerial, operational, financial and other employees to support that growth. Competition exists for qualified personnel in the biotechnology field. As of June 30, 2017, we had only 26 employees.

Future growth, if any, will impose significant added responsibilities on members of management, including the need to identify, recruit, maintain and integrate additional employees. Our future financial performance and our

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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

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 closing sale price for our common stock has varied between a high of $9.50 on November 18, 2016 and a low of $2.92 on June 7, 2017 in the twelve-month period ended June 30, 2017 (such prices as adjusted to give effect to the one-for-ten reverse stock split of our outstanding common stock effected on December 5, 2016, or the Reverse Stock Split). 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:

- new or continued delays in the initiation, enrollment and/or completion of our ongoing, planned and any future clinical trials of enobosarm, or negative, inconclusive or mixed results reported in any of our ongoing and any future clinical trials of enobosarm;
- our ability to raise additional capital to carry through with our preclinical and clinical development plans, including to potentially complete our planned placebo-controlled Phase 2 clinical trial of enobosarm to treat postmenopausal women with SUI, 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 or planned clinical trials of enobosarm;
- 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 commercial success of any product approved by the FDA or its foreign counterparts;
- introductions or announcements of technological innovations or new products by us, our potential collaborators, or our competitors, and the timing of these introductions or announcements;
- market conditions for equity investments in general, or the biotechnology or pharmaceutical industries in particular;
- regulatory developments in the United States and foreign countries;
- changes in the structure or reimbursement policies of health care payment systems;
- any intellectual property infringement lawsuit involving us;
- actual or anticipated fluctuations in our results of operations;

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- changes in financial estimates or recommendations by securities analysts;
- hedging or arbitrage trading activity that may develop regarding our common stock;
- sales of large blocks of our common stock;
- sales of our common stock by our executive officers, directors and significant stockholders;
- the low 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 and pharmaceutical stocks in particular, have experienced significant volatility that has often been unrelated to the operating performance of particular companies. For example, negative publicity regarding drug pricing and price increases by pharmaceutical companies, including as a result of statements on drug pricing by the Trump Administration, has negatively impacted, and may continue to negatively impact, the markets for biotechnology and pharmaceutical stocks. Likewise, as a result of significant changes in U.S. social, political, regulatory and economic conditions or in laws and policies governing foreign trade and health care spending and delivery, including the possible repeal and/or replacement of all or portions of the Healthcare Reform Act or greater restrictions on free trade stemming from Trump Administration policies, the financial markets could experience significant volatility that could also negatively impact the markets for biotechnology and pharmaceutical stocks. These broad market fluctuations may adversely affect the trading price of our common stock.

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

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

As of June 30, 2017, our executive officers, directors and holders of 5% or more of our outstanding common stock, including their affiliated or associated entities, held approximately 76.8% of our outstanding common stock, and our executive officers and directors alone, including their affiliated or associated entities, held approximately 37.5% of our outstanding common stock as well as warrants to purchase up to an additional 2.5 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.

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 Capital Market. The NASDAQ Stock Market LLC, or NASDAQ, has minimum requirements that a company must meet in order to remain listed on The NASDAQ Capital Market. These requirements include maintaining a minimum closing bid price of $1.00 per share, or the Bid Price Requirement, and the closing bid price of our common stock has in the past been well below $1.00 per share. In this regard, on December 23, 2015, we received a letter from the staff, or Staff, of NASDAQ providing notification 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 Capital Market, or the Bid Price Requirement. The notification had no immediate effect on the listing of our common stock. In accordance with NASDAQ listing rules, we were afforded 180 calendar days, or until June 20, 2016, to regain compliance with the Bid Price Requirement. On June 21, 2016, we received a letter from the Staff notifying us that we were eligible for an additional 180 calendar day period, or until December 19, 2016, to regain compliance with the minimum $1.00 Bid Price Requirement. In the letter, the Staff noted that our common stock had not regained compliance with the Bid Price Requirement during the initial 180-day compliance period that ended on June 20, 2016 and that we had submitted written notice of our intention to cure the Bid Price Requirement deficiency by effecting a reverse stock split prior to December 19, 2016, if necessary. On December 5, 2016, we effected the Reverse Stock Split, the

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primary purpose of which was to enable us to regain compliance with the Bid Price Requirement, which compliance was regained on December 20, 2016. However, there can be no assurance that the market price of our common stock will remain in excess of the $1.00 minimum bid price for a sustained period of time. In any event, there can be no assurance that we will continue to meet the Bid Price Requirement, or any other NASDAQ continued listing requirement, in the future. If we fail to meet these requirements, including the Bid Price Requirement and requirements to maintain minimum levels of stockholders' equity or market values of our common stock, NASDAQ may notify us that we have failed to meet the minimum listing requirements and initiate the delisting process. If our common stock is delisted, the liquidity of our common stock would be adversely affected and the market price of our common stock could decrease, and our ability to obtain sufficient additional capital to fund our operations and to continue as a going concern would be substantially impaired.

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

Under Section 382 of the Internal Revenue Code of 1986, as amended, if a corporation undergoes an "ownership change," generally defined as a greater than 50% change (by value) in its equity ownership over a three-year period, the corporation's ability to use its pre-change net operating loss carryforwards and other pre-change tax attributes (such as research tax credits) to offset its post-change taxable income or taxes may be limited. We completed a study through December 31, 2014 to determine whether any Section 382 limitations exist and, as a result of this study and our analysis of subsequent ownership changes, we do not believe that any Section 382 limitations exist through December 31, 2016. 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, our recent registered direct offering of our common stock, future equity offerings and/or 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 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 15% or more 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 15% or more of our outstanding voting stock, unless the merger or combination is approved in a prescribed manner. Finally, these provisions establish advance notice requirements for nominations for election to our Board of Directors or for proposing matters that can be acted upon at stockholder meetings. These provisions would apply even if the offer may be considered beneficial by some stockholders.

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

For the 12-month period ended June 30, 2017, the average daily trading volume of our common stock on The NASDAQ Capital Market was only 71,171 shares (as adjusted to give effect to the Reverse Stock Split). As a result, future sales of a substantial number of shares of our common stock in the public market, or the perception that such

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sales may occur, could adversely affect the then-prevailing market price of our common stock. As of June 30, 2017, we had 16,041,923 shares of common stock outstanding. In addition, as a result of the low trading volume of our common stock, which was exacerbated by the Reverse Stock Split, 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 October 2016, we completed a registered direct offering in which we sold 1.7 million shares of our common stock (as adjusted to give effect to the Reverse Stock Split). In November 2014, we completed a private placement of 6.4 million shares of our common stock and warrants to purchase 6.4 million shares of our common stock (as adjusted to give effect to the Reverse Stock Split). Similarly, in March 2014 we completed a private placement of 1.2 million shares of our common stock and warrants to purchase 1.0 million shares of our common stock (as adjusted to give effect to the Reverse Stock Split). 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 1.2 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 1.0 million shares of common stock underlying the warrants we issued to those investors (which warrants subsequently expired unexercised). Likewise, pursuant to the terms of the securities purchase agreement we entered into in connection with the

November 2014 private placement, we filed registration statements under the Securities Act registering the resale of the 6.4 million shares of common stock we issued to the investors in the November 2014 private placement, which included J.R. Hyde, III, as well as the additional 6.4 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 790,000 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 or in our 2016 registered direct offering of common stock, 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

On August 10, 2017, we entered into a loan agreement with J.R. Hyde, III and The Pyramid Peak Foundation (the "Loan Agreement") to borrow up to a total of $15.0 million (the "Loans"). Each of Mr. Hyde and The Pyramid Peak Foundation (the "Lenders") was reported as a greater than 5% holder of shares of our common stock in our 2017 Proxy Statement filed with the Securities and Exchange Commission on March 31, 2017. Additionally, Mr. Hyde serves on our board of directors.

Under the Loan Agreement, we can borrow in one or more advances up to a total of $15.0 million. Advances can be prepaid in whole or in part without penalty or premium and may be reborrowed. Interest will accrue monthly on each outstanding advance at a fixed rate of 8.00% and will be payable, together with all principal amounts outstanding, on the earlier of (a) May 10, 2018 (the "Maturity Date") or (b) the date on which we consummate an equity financing resulting in gross proceeds of at least $15,000,000 (a "Qualified Financing"). Events of default under the Loan Agreement include (a) the failure to pay principal or interest when due, (b) the commencement of a voluntary or involuntary bankruptcy or insolvency action and (c) the breach of any representation or warranty contained in the Loan Agreement by us. If an event of default occurs, all unpaid principal and interest will become immediately due and payable. Any borrowings under the Loan Agreement will be unsecured and the Loan Agreement contains no financial covenants. The Loan Agreement contains a covenant by the Lenders to participate, at our option, in any Qualified Financing in amounts up to their respective principal and interest amounts outstanding.

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.

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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: August 14, 2017

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

Date: August 14, 2017

By: /s/ Jason T. Shackelford
Jason T. Shackelford, Vice President, Finance and Accounting and Principal Financial and Accounting Officer
(Principal Financial and Accounting Officer)

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EXHIBIT INDEX

Exhibit Number	Exhibit Description	Incorporation By Reference			
		Form	SEC File No.	Exhibit	Filing Date
2.1	Asset Purchase Agreement dated as of September 28, 2012 between the Registrant and Strakan International S.à r.l.	8-K	000-50549	2.1	10/03/2012
3.1	Restated Certificate of Incorporation of GTx, Inc.	S-3	333-127175	4.1	08/04/2005
3.2	Certificate of Amendment of Restated Certificate of Incorporation of GTx, Inc.	8-K	000-50549	3.2	05/06/2011
3.3	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	10-Q	000-50549	3.4	05/11/2015

	Incorporation of GTx, Inc.				
3.5	Certificate of Amendment of Restated Certificate of Incorporation of GTx, Inc.	8-K	000-50549	3.1	12/05/2016
3.6	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, 3.5 and 3.6	—	—	—	—
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 on November 14, 2014 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
4.9	Form of Warrant Amendment Agreement entered into effective as of March 25, 2016 between Registrant and each holder of a Common Stock Warrant originally issued on November 14, 2014	10-Q	000-50549	4.9	5/10/2016
10.1+	Loan Agreement, dated as of August 10, 2017, by and among Registrant, J.R. Hyde, III and The Pyramid Peak Foundation and form of Promissory Note	—	—	—	—
31.1+	Certification of Principal Executive Officer, as required by Rule 13a-14(a) or Rule 15d-14(a)	—	—	—	—
31.2+	Certification of Principal Financial Officer, as required by Rule 13a-14(a) or Rule 15d-14(a)	—	—	—	—
32.1+	Certification of Principal Executive Officer, as required by Rule 13a-14(b) or Rule 15d-14(b) and Section 1350 of Chapter 63 of Title 18 of the United States Code (18 U.S.C. §1350)(1)	—	—	—	—
32.2+	Certification of Principal Financial Officer, as required by Rule 13a-14(b) or Rule 15d-14(b) and Section 1350 of	—	—	—	—

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	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.

LOAN AGREEMENT

This **LOAN AGREEMENT** (this "**Agreement**") is entered into as of August 10, 2017 (the "**Effective Date**") by and among The Pyramid Peak Foundation ("**Pyramid**"), J.R. Hyde, III ("**Hyde**" and, together with Pyramid, the "**Lenders**"), and GTx, Inc., a Delaware corporation ("**Borrower**"). The parties agree as follows:

1. ***Loans***. Lenders will make extensions of credit for Borrower's benefit (collectively, "***Loans***"), and Borrower promises to pay Lenders the amount of all loans and other debts, principal and other amounts borrower owes Lenders now or later (collectively, "***Obligations***") pursuant to the terms and conditions of this agreement and as set forth below**.**

1.1 Availability. So long as no Event of Default (as defined below) has occurred and is continuing, from and after the date hereof and through and including May 10, 2018 (the "**Maturity Date**"), the Lenders shall make available to Borrower for borrowings by Borrower from time to time a principal amount of Fifteen Million Dollars ($15,000,000) less the aggregate principal amount of the Advances outstanding on the date hereof (each, an "**Advance**"). Advances may be repaid and, prior to the Maturity Date (as defined below), reborrowed, subject to the applicable terms and conditions precedent herein. Subject to the terms and conditions set forth herein, each Lender severally agrees to (a) make Advances in amounts not exceeding Seven Million Five Hundred Thousand Dollars ($7,500,000) and (b) fund each Advance in equal (50%) amounts.

1.2 Principal Repayment. The outstanding principal amount of each Advance together with all accrued and unpaid interest thereon shall be due and payable on the Maturity Date.

1.3 Interest Rate. Borrower further promises to pay interest on the outstanding principal amount of each Advance from the date thereof until payment in full, which interest shall be payable at a rate equal 8% per annum. Interest shall be due and payable on the Maturity Date, commencing on the first day of the calendar quarter following the calendar quarter in which an Advance is made, and shall be calculated on the basis of a 365/366-day year for the actual number of days elapsed.

1.4 Place of Payment. All amounts payable hereunder shall be payable in lawful money of the United States of America at the office of the Borrower, 175 Toyota Plaza, 7th Floor, Memphis TN, 38103, unless another place of payment shall be specified in writing by each Lender.

1.5 Advance Requests; Evidence of Debt. Whenever Borrower desires an Advance hereunder, Borrower shall notify each Lender by facsimile with a transmission confirmation or by electronic mail as long as a read receipt is requested and received no later than 4:00 p.m. central time, seven (7) calendar days (or such shorter time as the Lenders may agree in writing) prior to the date on which the Advance is requested to be made. The Lenders hereby request, and Borrower agrees to deliver on the date hereof, a promissory note in the form of Exhibit A hereto as evidence of the Loans hereunder (each a "**Note**" and together, the "**Notes**". At the time of any Advance (or at the time of receipt of any payment of principal), each Lender shall make or cause to be made, an appropriate notation on its respective Note reflecting the amount of such Advance (or the amount of such payment). The outstanding amount of the Note shall be prima facie evidence of the principal amount thereof outstanding, but the failure to record, or any error in so recording, shall not limit or otherwise affect the obligations of Borrower to make payments of principal of or interest on such Note when due.

1.6 Application of Payments; Prepayment. Payment on the Advances shall be applied first to accrued interest, and thereafter to the outstanding principal balance thereof. Advances may be prepaid in whole in in part without penalty or premium. Any amount repaid pursuant to this Section 1.6 may be reborrowed subject to Section 1.5 hereof. Any partial prepayment made pursuant to this Section 1.6 shall be applied to interest first and then to principal, and shall be applied to the oldest outstanding Advance first. At the time of any prepayment of principal hereunder, Borrower shall also pay all accrued and unpaid interest on the amount prepaid through the date of prepayment.

1.7 Default Rate. Upon the occurrence and during the continuance of an Event of Default (as defined below), which Event of Default is not remedied by the Borrower or waived by both Lenders within five (5) days after the receipt by Borrower of notice from any Lender of such Event of Default, overdue and unpaid amounts under this Agreement shall bear interest at a rate per annum equal to seven percent (7.0%) above the rate that is otherwise applicable thereto unless each Lender otherwise elects from time to time in its sole discretion to impose a smaller increase. Fees and expenses which are required to be paid by Borrower pursuant to the this Agreement but are not paid when due shall bear interest until paid at a rate equal to the highest rate applicable to the Obligations. Payment or acceptance of the increased interest rate provided in this Section 1.7 is not a permitted alternative to timely payment and shall not constitute a waiver of any Event of Default or otherwise prejudice or limit any rights or remedies of Lenders.

2. Representations, Warranties and Covenants of Borrower. Borrower represents, warrants and covenants to Lenders as follows, as of the Effective Date and with respect to covenants, for so long as this Agreement is in effect or any Obligations remain outstanding:

2.1 Corporate Existence. Borrower has the requisite power and authority to enter into this Agreement and to consummate the transactions contemplated hereby. The execution and delivery of this Agreement by the Borrower and the consummation by the Borrower of the transactions contemplated hereby have been duly authorized by all necessary corporate action on the part of the Borrower. This Agreement has been duly executed and delivered by the Borrower and constitutes the legal, valid and binding agreement of the Borrower enforceable against the Borrower in accordance with its terms, except as may be limited by (i) applicable bankruptcy, insolvency, reorganization, moratorium and other laws of general application affecting enforcement of creditors' rights generally and (ii) equitable principles of general applicability relating to the availability of specific performance, injunctive relief or other equitable remedies.

2.2 Consents. No consent, approval, authorization, order, license, registration or qualification of or with any Governmental Entity is required for the execution and delivery by the Borrower of this Agreement or the transactions contemplated hereby, except such consents, approvals, authorizations, orders, licenses, registrations or qualifications as have been obtained, or which, if not obtained, would not, individually or in the aggregate, have a material adverse effect on the ability of the Borrower to perform its obligations hereunder or consummate the transactions contemplated hereby on a timely basis. As used in this Agreement, the term "**Governmental Entity**" means any agency, bureau, commission, authority, department, official, political subdivision, tribunal or other instrumentality of any government, whether (i) regulatory, administrative or otherwise (including, without limitation, a self-regulatory organization or stock exchange); (ii) federal, state or local; or (iii) domestic or foreign.

2.3 No Conflicts. The execution and delivery by the Borrower of this Agreement, the performance by the Borrower of its obligations hereunder, and the consummation by the Borrower of the transactions contemplated hereby, will not conflict with or result in a breach or violation of (i) any of the terms or provisions of, or constitute a default (or an event which with notice or lapse of time or both would become a default) under, or give to others any rights of termination, amendment, acceleration or cancellation of, any indenture, mortgage, deed of trust, loan agreement or other agreement or instrument to which the Borrower or any of its subsidiaries is a party or by which the Borrower or any of its subsidiaries is bound or to which any of their property or assets is subject or (ii) any applicable law or statute or any order, rule or regulation of any Governmental Entity having jurisdiction over the Borrower or any of its subsidiaries or any of their respective properties, except for in the case of either clause (i) or (ii) such conflicts, breaches or violations that would not prevent or delay the consummation of the transactions contemplated by this Agreement or that would not be reasonably expected to have a material adverse effect on the Borrower, nor will any such action result in any violation of the provisions of the organizational documents of the Borrower.

3. Term. This Agreement shall continue in effect until the earlier of (a) the Maturity Date or (b) the date of a Qualified Financing (as defined below) resulting in an amount of gross proceeds equal to or greater than Fifteen Million Dollars ($15,000,000) (the "**Termination Date**"). On the Termination Date or on any earlier effective date of termination of this Agreement, the Loans shall terminate, and Borrower shall pay in cash all Obligations in full, whether or not such Obligations are otherwise then due and payable. No termination shall relieve Borrower of any obligation to Lenders, until all of the Obligations have been paid and performed in full.

4. Events of Default. The occurrence of any of the following events shall constitute an "**Event of Default**" hereunder: (i) Borrower fails to pay when due any Loan or other monetary Obligation within five Business Days after the due date (during which time no additional Loans shall be made by Lenders); (ii) Borrower fails to perform any obligation (other than payment of any Loan or other Obligations or covenant hereunder, which, if such default can be reasonably cured, is not cured within ten days after the date due (or a later date, as approved in writing by Lenders); (iii) any representation, or written statement given to Lenders by or on behalf of Borrower, now or in the future, is untrue or misleading in a material respect; (iv) the dissolution, winding up, or insolvency of Borrower or (v) the appointment of a receiver, trustee or custodian, for all or part of the property of, assignment for the benefit of creditors by, or commencement of any proceeding by or against, Borrower under any reorganization, bankruptcy, insolvency, arrangement, readjustment of debt, dissolution or liquidation law or statute of any jurisdiction, now or in the future in effect.

5. Rights and Remedies. If an Event of Default occurs and continues, Lenders may, without notice or demand do any or all of the following: (i) accelerate and declare all of the Loans and other Obligations to be immediately due and payable (but if an Event of Default described in Sections 4(iv) or 4(v) occurs, all Obligations are immediately due and payable without any action by Lenders); (ii) stop advancing money or extending credit for Borrower's benefit under this Agreement; and/or (iii) exercise any other rights and remedies permitted by applicable law. All of Lenders' rights and remedies under this Agreement or any other agreement between Lenders and Borrower are cumulative. Borrower waives demand, notice of default or dishonor, notice of payment and nonpayment, notice of any default, nonpayment at maturity, release, compromise, settlement, extension, or renewal of accounts, documents, instruments, chattel paper, and guarantees held by Lenders on which Borrower is liable.

6. Covenant to Invest. Upon a bona-fide sale of equity interests in the Borrower (a "**Qualified Financing**") resulting in gross proceeds equal to or greater than Fifteen Million Dollars $(15,000,000), each Lender agrees to participate in such Qualified Financing in an amount up to their then outstanding Obligations.

7. General.

7.1 No Waivers; Amendments. The failure of each Lender at any time to require Borrower to comply strictly with any of the provisions of this Agreement shall not waive any Lender's right to later demand and receive strict compliance. Any waiver of a default shall not waive any other default. None of the provisions of this Agreement may be waived except by a specific written waiver signed by the affected Lender(s) and delivered to Borrower. The provisions of this Agreement may not be amended except in a writing signed by Borrower and both Lenders.

7.2 Expenses; Attorneys' Fees. Borrower shall reimburse Lenders for all audit fees and expenses and reasonable costs and expenses (including reasonable attorneys' fees and expenses) for preparing, negotiating, administering, defending and enforcing this Agreement and the other loan documents with Lenders (including appeals or insolvency proceedings) (collectively, "**Expenses**"). If, subject to the foregoing, any Lender or Borrower files any lawsuit against the other predicated on a breach of this Agreement, the prevailing party shall be entitled to recover its costs and reasonable attorneys' fees from the non-prevailing party.

7.3 Binding Effect; Assignment. This Agreement is binding upon and for the benefit of the successors and permitted assignees of each party. Borrower may not assign any rights under this Agreement without both Lenders' prior written consent.

7.4 Notices. All notices by any party required or permitted under this Agreement or any other related agreement must be in writing and be personally delivered or sent by overnight delivery, certified mail (postage prepaid and return receipt requested), or facsimile to the addresses and numbers below.

7.5 Governing Law; Jurisdiction. This Agreement shall be governed by, and construed and enforced in accordance with, the laws of the State of Delaware without regard to principles of conflicts of law.

7.6 Other. If any provision hereof is unenforceable, the remainder of this Agreement shall continue in full force and effect. This Agreement (including schedules hereto) and any other written agreements and, documents executed in connection herewith are the complete agreement between Borrower and Lenders and supersede all prior and contemporaneous negotiations and oral representations and agreements, all of which are merged and integrated herein. This Agreement may be executed in one or more counterparts, all of which when taken together will constitute one agreement.

8. Mutual Waiver of Jury Trial. **BORROWER AND BANK EACH WAIVE THEIR RIGHT TO A JURY TRIAL OF ANY CLAIM OR CAUSE OF ACTION ARISING OUT OF THIS AGREEMENT OR ANY RELATED DOCUMENT OR ANY TRANSACTION CONTEMPLATED HEREBY OR THEREBY, INCLUDING CONTRACT, TORT, BREACH OF DUTY AND ALL OTHER CLAIMS. THIS WAIVER IS A MATERIAL INDUCEMENT FOR BOTH PARTIES TO ENTER INTO THIS AGREEMENT.**

[Signature page follows.]

IN WITNESS WHEREOF, the parties hereto have executed this Agreement as of the date initially set forth above.

BORROWER:

GTX, INC.

By: /s/ Marc S. Hanover
Name: Marc S. Hanover
Title: Chief Executive Officer

Address: 175 Toyota Plaza, 7th Floor
Memphis, TN 38103
Attn: Chief Legal officer and Secretary
Facsimile: 901-271-8670

LENDERS:

The Pyramid Peak Foundation

By: /s/ Andy McCarroll
Name: Andy McCarroll
Title: Secretary

Address: 6410 Poplar Ave. Ste. 710
Memphis, TN 38119

J.R. Hyde, III

Signature: /s/ J.R. Hyde, III

Address: 17 West Pontotoc Ave, Suite 100
Memphis, TN 38103

EXHIBIT A

FORM OF NOTE

FOR VALUE RECEIVED, **GTX, INC.**, **a Delaware corporation ("Borrower")**, promises to pay **[·]**, **("Payee")** or its registered assigns, on or before May 10, 2018, the lesser of (a) **seven million five hundred thousand ($7,500,000.00)** and (b) the unpaid principal amount of all Advances made by Payee to Borrower as Loans under the Loan Agreement referred to below.

Borrower also promises to pay interest on the unpaid principal amount hereof, from the date hereof until paid in full, at the rates set forth in the Loan Agreement, dated as of August 10, 2017 (as may be amended, supplemented or otherwise modified, the **"Loan Agreement"**; the terms defined therein and not otherwise defined herein being used herein as therein defined), by and among **BORROWER**, as Borrower and the Lenders party thereto.

This Note is one of the Notes issued pursuant to and entitled to the benefits of the Loan Agreement, to which reference is hereby made for a more complete statement of the terms and conditions under which the Loans evidenced hereby were made and are to be repaid.

All payments of principal and interest in respect of this Note shall be made in lawful money of the United States of America in same day funds as set forth in Section 1.4 of the Loan Agreement. Borrower and the Lenders shall be entitled to deem and treat Payee as the owner and holder of this Note and the obligations evidenced hereby. Payee hereby agrees, by its acceptance hereof, that before disposing of this Note or any part hereof it will make a notation hereon of all principal payments previously made hereunder and of the date to which interest hereon has been paid; provided, the failure to make a notation of any payment made on this Note shall not limit or otherwise affect the obligations of Borrower hereunder with respect to payments of principal of or interest on this Note.

This Note is subject to prepayment at the option of Borrower as provided in the Loan Agreement.

Upon the occurrence and during the continuance of an Event of Default, the unpaid balance of the principal amount of this Note, together with all accrued and unpaid interest thereon, may become, or may be declared to be, due and payable in the manner, upon the conditions and with the effect provided in the Loan Agreement.

The terms of this Note are subject to amendment only in the manner provided in the Loan Agreement.

No reference herein to the Loan Agreement and no provision of this Note or the Loan Agreement shall alter or impair the obligations of Borrower, which are absolute and unconditional, to pay the principal of and interest on this Note at the place, at the respective times, and in the currency herein prescribed.

Borrower and any endorsers of this Note hereby consent to renewals and extensions of time at or after the maturity hereof, without notice, and hereby waive diligence, presentment, protest, demand notice of every kind and, to the full extent permitted by law, the right to plead any statute of limitations as a defense to any demand hereunder (except as expressly provided in the Loan Agreement).

[Signature page follows.]

THIS NOTE SHALL BE GOVERNED BY, AND CONSTRUED AND INTERPRETED IN ACCORDANCE WITH THE LAW OF THE STATE OF DELAWARE.

GTX, INC.

By: _____
Name: Marc S. Hanover
Title: Chief Executive Officer

PRINCIPAL BORROWINGS SCHEDULE

DATE	AMOUNT OF LOAN MADE THIS DATE	AMOUNT OF PRINCIPAL PAID THIS DATE	OUTSTANDING PRINCIPAL BALANCE THIS DATE	NOTATION MADE BY

EXHIBIT 31.1

PRINCIPAL EXECUTIVE OFFICER CERTIFICATION

I, Marc S. Hanover, certify that:

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

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

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

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

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

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

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

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

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

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

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

Date: August 14, 2017

/s/ Marc S. Hanover
Marc S. Hanover
Chief Executive Officer
(Principal Executive Officer)

EXHIBIT 31.2

PRINCIPAL FINANCIAL OFFICER CERTIFICATION

I, Jason T. Shackelford, certify that:

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

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

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

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

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

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

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

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

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

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

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

Date: August 14, 2017

/s/ Jason T. Shackelford
Jason T. Shackelford
Vice President, Finance and Accounting,
and Principal Financial and Accounting Officer
(Principal Financial and Accounting Officer)

EXHIBIT 32.1

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

In connection with the Quarterly Report of GTx, Inc. (the "Company") on Form 10-Q for the period ended June 30, 2017, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I, Marc S. Hanover, Chief Executive Officer of the Company certify, pursuant to Rule 13a-14(b) or Rule 15d-14(b) and 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that, to the best of my knowledge:

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

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

Date: August 14, 2017

/s/ Marc S. Hanover
Marc S. Hanover
Chief Executive Officer
(Principal Executive Officer)

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

EXHIBIT 32.2

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

In connection with the Quarterly Report of GTx, Inc. (the "Company") on Form 10-Q for the period ended June 30, 2017, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I, Jason T. Shackelford, Principal Financial Officer of the Company certify, pursuant to Rule 13a-14(b) or Rule 15d-14(b) and 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that, to the best of my knowledge:

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

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

Date: August 14, 2017

/s/ Jason T. Shackelford
Jason T. Shackelford
Vice President, Finance and Accounting,
and Principal Financial and Accounting Officer
(Principal Financial and Accounting Officer)

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