GTX INC /DE/
Form 10-K
March 18, 2019

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

FORM 10-K

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

For the fiscal year ended December 31, 2018

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)

Securities registered pursuant to Section 12(b) of the Act:

Title of Each Class	Name of Each Exchange on Which Registered
Common Stock, par value $0.001 per share	The Nasdaq Stock Market, LLC

Securities registered pursuant to Section 12(g) of the Act: None

Indicate by check mark if the registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act. Yes o No ý

Indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or Section 15(d) of the Act. Yes o No ý

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 ý No o

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). Yes ý No o

Indicate by check mark if disclosure of delinquent filers pursuant to Item 405 of Regulation S-K is not contained herein, and will not be contained, to the best of registrant's knowledge, in definitive proxy or information statements incorporated by reference in Part III of this Form 10-K or any amendment to this Form 10-K. ý

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 ý
Non-accelerated filer o	Smaller reporting company ý
	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 Act). Yes o No ý

The aggregate market value of common stock held by non-affiliates of the registrant based on the closing sales price of the registrant's common stock on June 30, 2018 as reported on The Nasdaq Capital Market was $189,830,468.

There were 24,051,844 shares of registrant's common stock issued and outstanding as of March 12, 2019.

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DOCUMENTS INCORPORATED BY REFERENCE

Certain information required by Part III, Items 10-14 of this Form 10-K is incorporated by reference to the Registrant's definitive Proxy Statement for the 2019 Annual Meeting of Stockholders to be filed with the Securities and Exchange Commission pursuant to Regulation 14A not later than 120 days after the end of the fiscal year covered by this Form 10-K, provided that if such Proxy Statement is not filed within such period, such information will be included in an amendment to this Form 10-K to be filed within such 120-day period.

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SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

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

our ability to satisfy the required conditions and otherwise complete our planned merger, or the Merger, with Oncternal Therapeutics, Inc., or Oncternal, pursuant to the Agreement and Plan of Merger and Reorganization, dated March 6, 2019, or the Merger Agreement, by and among GTx, Grizzly Merger Sub, Inc., a wholly-owned subsidiary of GTx, and Oncternal, on a timely basis or at all;

the expected benefits and potential value created by the proposed Merger for our stockholders, including the ownership percentage of our stockholders in the combined organization immediately following the consummation of the proposed Merger and the potential value of the contingent value rights to be received by our stockholders in connection with the proposed Merger if it is completed;

the implementation of our business strategies, including our ability to preserve or realize any significant value from our selective androgen receptor degrader, or SARD, program and our selective androgen receptor modulators, or SARMs;

our expectations regarding the near-term development of our SARD program, including our ability to advance a SARD compound into a first-in-human clinical trial;

the therapeutic and commercial potential of our SARD program;

our ability to establish and maintain potential new collaborative, partnering or other strategic arrangements for the development of our SARD program;

our ability to establish and maintain potential new collaborative, partnering or other strategic arrangements for our SARM assets, including a sale or other divestiture of our SARM assets;

our ability to raise additional capital, whether through potential new collaborative, partnering or other strategic arrangements or otherwise;

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

our projected operating and financial performance; and

our estimates regarding the sufficiency of our cash resources, expenses, including those related to the consummation of the proposed Merger, capital requirements and needs for additional financing, and our ability to obtain additional financing and to continue as a going concern if the Merger is not completed.

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In some cases, you can identify forward-looking statements by terms such as "anticipates," "believes," "could," "estimates," "expects," "intends," "may," "plans," "potential," "predicts," "projects," "should," "will," "would," and similar expressions intended to identify forward-looking statements. Forward-looking statements reflect our current views with respect to future events, are based on assumptions, and are subject to risks, uncertainties and other important factors. We discuss many of these risks in this Annual Report on Form 10-K in greater detail in the section entitled "Risk Factors" under Part I, 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 Annual Report on Form 10-K. You should read this Annual Report on Form 10-K and the documents that we incorporate by reference in and have filed as exhibits to this Annual Report on Form 10-K, 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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PART I

ITEM 1. BUSINESS

Overview

We are a biopharmaceutical company dedicated to the discovery, development and commercialization of medicines to treat serious and/or significant unmet medical conditions. Under an exclusive worldwide license agreement with the University of Tennessee Research Foundation, or UTRF, we are developing UTRF's proprietary selective androgen receptor degrader, or SARD, technology, which we believe has the potential to provide compounds that can degrade or antagonize multiple forms of androgen receptor, or AR, thereby potentially 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 androgen targeted therapies. We are in the process of completing ongoing mechanistic preclinical studies in order to select the most appropriate SARD compounds to move forward into the additional preclinical studies required to submit an investigational new drug application, or IND, and potentially advance one of our SARD compounds into a first-in-human clinical trial.

We had been developing selective androgen receptor modulators, or SARMs. Our SARM product candidate, enobosarm (GTx-024), was most recently evaluated in post-menopausal women with stress urinary incontinence, or SUI. During the third quarter of 2018, we announced that our randomized, placebo-controlled Phase 2 clinical trial, or the ASTRID trial, evaluating the change in the mean number of daily SUI episodes following 12 weeks of enobosarm treatment failed to achieve statistical significance on the primary endpoint of the proportion of patients with a greater than 50% reduction in incontinence episodes per day compared to placebo. We have completed the ASTRID trial, including our review of the full data sets from the clinical trial, and have determined that there is not a sufficient path forward to warrant additional clinical development of enobosarm to treat SUI. We have therefore discontinued further development of enobosarm to treat SUI, including discontinuing the related durability and open-label safety extension studies we initiated before we received topline data from the ASTRID trial. We have also discontinued any further development of our SARM program generally.

Following the announcement of the ASTRID trial results, our board of directors commenced a process of evaluating strategic alternatives to maximize stockholder value. To assist with this process, our board of directors engaged a financial advisory firm to help explore our available strategic alternatives, including possible mergers and business combinations, a sale of part or all of our assets, and collaboration and licensing arrangements. On March 6, 2019, we and Oncternal announced the signing of the Merger Agreement. Upon the terms and subject to the satisfaction of the conditions described in the Merger Agreement, including approval of the transaction by our stockholders and Oncternal's stockholders, a wholly-owned subsidiary of GTx will be merged with and into Oncternal, with Oncternal surviving the Merger as a wholly-owned subsidiary of GTx.

The proposed Merger is structured as a stock-for-stock transaction whereby all of Oncternal's outstanding shares of common stock and securities convertible into or exercisable for Oncternal's common stock will be converted into the right to receive GTx common stock and securities convertible into or exercisable for GTx common stock. Under the exchange ratio formula in the Merger Agreement, the former Oncternal stockholders immediately before the Merger are expected to own approximately 75% of the outstanding capital stock of GTx, and the stockholders of GTx immediately before the Merger are expected to own approximately 25% of the outstanding capital stock of GTx, subject to certain assumptions. The exchange ratio formula excludes Oncternal's outstanding stock options and warrants and GTx's outstanding stock options and warrants. To the extent Oncternal's outstanding stock options or warrants are exercised in the future, it will result in further dilution to

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GTx's stockholders. Under certain circumstances as set forth in the Merger Agreement, the ownership percentages may be adjusted upward or downward based on cash levels of the respective companies at the closing of the Merger. We anticipate that the Merger will close in the second quarter of 2019. Following the closing of the Merger, James Breitmeyer will serve as GTx's Chief Executive Officer, Richard Vincent will serve as GTx's Chief Financial Officer, and Lauren Otsuki will serve as GTx's Chief Operating Officer. Additionally, following the closing of the Merger, our board of directors will consist of nine directors, including two current GTx board members. This transaction, which has been approved by our board of directors and the board of directors of Oncternal, is subject to the satisfaction or waiver of certain conditions, including the required approvals by the parties' stockholders (including stockholder approval from one of Oncternal's significant stockholders, Shanghai Pharmaceutical (USA) Inc., which holds all of the outstanding shares of one series of Oncternal's preferred stock that must approve the transactions contemplated by the Merger Agreement) and other customary closing conditions. Certain affiliates of ours who hold approximately 45% of our common stock as of date of the Merger Agreement have agreed to vote in favor of the Merger and certain affiliates of Oncternal, excluding Shanghai Pharmaceutical (USA) Inc., who hold approximately 42% of the outstanding capital stock of Oncternal as of date of the Merger Agreement have agreed to vote in favor of the Merger. However, Oncternal currently expects that it will receive stockholder approval from Shanghai Pharmaceutical (USA) Inc. approximately two months after the date of the Merger Agreement based on the internal approval process required for such approval at Shanghai Pharmaceutical (USA) Inc.

In addition, at the effective time of the Merger, GTx and certain other parties will enter into a Contingent Value Rights Agreement, or the CVR Agreement. Pursuant to the CVR Agreement, for each share of GTx common stock held, GTx stockholders of record as of immediately prior to the effective time of the Merger will receive one contingent value right, or CVR, entitling such holders to receive in the aggregate 50% of any net proceeds received during the 15-year period after the closing of the Merger from the grant, sale or transfer of rights to our SARD or SARM technology that occurs during the 10-year period after the closing of the Merger (or in the 11th year if based on a term sheet approved during the initial 10-year period) and, if applicable, to receive royalties on the sale of any SARD products by the combined company during the 15-year period after the closing of the Merger. Under the CVR Agreement, Oncternal (as successor in interest to GTx) agreed to use commercially reasonable efforts to develop SARD products and to divest our SARM technology, subject to certain limitations. The CVRs will not be transferable, except in certain limited circumstances, will not be certificated or evidenced by any instrument and will not be registered with the SEC or listed for trading on any exchange.

Although we have entered into the Merger Agreement and intend to consummate the proposed Merger, there is no assurance that we will be able to successfully consummate the proposed Merger on a timely basis, or at all. If, for any reason, the proposed Merger is not completed, we will reconsider our strategic alternatives and could pursue one or more of the following courses of action:

Continue development of our SARD program. As set forth above, we are in the process of completing ongoing mechanistic preclinical studies in order to select the most appropriate SARD compounds to move forward into the additional preclinical studies required to submit an IND and potentially advance one of our SARD compounds into a first-in-human clinical trial. Accordingly, if, for any reason, the proposed Merger is not consummated, we may determine to move forward with our planned IND-enabling studies of our SARD compounds. However, while we believe that our existing capital resources will be adequate to enable us to conduct and complete planned IND-enabling preclinical studies of our SARD compounds, we will require significant additional financial resources in order to initiate and complete initial human clinical trials of a SARD compound and to otherwise further the development of our SARD program.

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As a result, we may also resume our efforts to seek additional funds through potential collaborative, partnering or other strategic arrangements to provide us with the necessary resources for the development of our SARD program.

Pursue potential collaborative, partnering or other strategic arrangements for our SARM assets, including a sale or other divestiture of our SARM assets. We have discontinued further development of our SARM program, including enobosarm, and do not currently have any plans to resume development of our SARM program. We continue our efforts to seek potential collaborative, partnering or other strategic arrangements for our SARM assets, including a sale or other divestiture of our SARM assets.

Pursue another strategic transaction like the proposed Merger. Our board of directors may elect to pursue an alternative strategy, one of which may be a strategic transaction similar to the proposed Merger.

Dissolve and liquidate our assets. If, for any reason, the proposed Merger is not consummated and we are unable to identify and complete an alternative strategic transaction like the Merger or potential collaborative, partnering or other strategic arrangements for our SARM assets, or to continue to operate our business due to our inability to raise additional funding for the development of our SARD program or otherwise, we may be required to dissolve and liquidate our assets. In such case, we would be required to pay all of our debts and contractual obligations, and to set aside certain reserves for potential future claims, and there can be no assurances as to the amount or timing of available cash left to distribute to our stockholders after paying our debts and other obligations and setting aside funds for reserves.

Our SARD Program

SARDs for the Potential Treatment of Castration Resistant Prostate Cancer

Scientific Overview. SARDs are a novel class of drugs. The AR is a major driver of prostate tumor cell proliferation, and blocking its activity is a therapeutic target. Despite the use of therapies designed to inhibit the AR pathway in men with advanced prostate cancer, a significant number of men have tumors that do not respond to such therapeutic approaches and/or become resistant to them. This lack of response may be due to the presence of forms of the AR (splice variants and mutated) for which these therapies are not effective. SARDs are designed to not only bind to androgen receptors, but also induce androgen receptor degradation and ultimately inhibit tumor cell growth. Selective AR degradation which targets the N-terminus may be an effective therapeutic strategy where a variant or mutated AR can be degraded by the SARD. This ability to circumvent common drug resistance in prostate cancer patients may provide an important tool for effective new treatments.

We believe SARDs have 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. Although current therapies have improved overall survival in men with CRPC, approximately one-third of the CRPC patients do not respond to these therapies, due in part to the presence of splice variants, including AR-V7, as well as mutations in the androgen receptor. Splice variants of the androgen receptor have been identified in which the ligand binding domain, the binding site for androgens and necessary for the action of many of the current therapies, is lost. In addition, most patients who initially respond to available treatments eventually progress due to the emergence of resistance to these therapies. It is believed that CRPC growth remains highly dependent on androgen receptor activity, although the mechanisms which underlie this resistance are not fully understood. We

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believe a therapeutic agent that would safely degrade multiple forms of the androgen receptor, including those without the ligand binding domain, would be uniquely positioned to address this patient population.

Potential Market. In the United States alone, we believe there are approximately 80,000 men who have developed resistance to luteinizing hormone-releasing hormone, or LHRH, therapies and therefore have CRPC but who have not received chemotherapy. We believe there are approximately 36,000 men diagnosed each year with metastatic hormone sensitive prostate cancer. Zytiga® and XTANDI® are currently the only drugs approved for the treatment of metastatic CRPC in patients who have not yet received chemotherapy, although several other drugs are in clinical development for this indication. We believe new hormonal therapies in development, if approved, will be used prior to chemotherapy as physicians and patients look for treatment options capable of delaying cancer progression and possibly prolonging survival prior to chemotherapy.

Preclinical Development. We are in the process of completing ongoing mechanistic preclinical studies in order to select the most appropriate SARD compounds to move forward into the additional preclinical studies required to submit an IND and potentially advance one of our SARD compounds into a first-in-human clinical trial. However, while we believe that our existing capital resources will be adequate to enable us to conduct and complete planned IND-enabling preclinical studies of our SARD compounds, we will require significant additional financial resources in order to initiate and complete initial human clinical trials of a SARD compound and to otherwise further the development of our SARD program. Accordingly, if, for any reason, the proposed Merger is not consummated, we may resume our efforts to seek additional funds through potential collaborative, partnering or other strategic arrangements to provide us with the necessary resources for the development of our SARD program.

SARMs

Evaluation of Enobosarm for the Treatment of Postmenopausal Women with SUI. In the third quarter of 2017, we initiated the ASTRID trial at over 60 clinical trial centers in the United States to evaluate the change in the mean number of daily SUI episodes following 12 weeks of enobosarm treatment. The ASTRID trial evaluated the safety and efficacy of enobosarm (1 mg and 3 mg) compared with placebo in post-menopausal women who have demonstrated SUI symptoms for more than six months, with an average of 3 to 15 reported SUI episodes per day over a three-day period, and a positive bladder stress test. The primary endpoint for the ASTRID trial was the percentage of patients with at least a 50 percent reduction in mean leaks per day at week 12, compared to baseline. During the third quarter of 2018, we announced that the ASTRID trial failed to achieve statistical significance on the primary endpoint of the proportion of patients with a greater than 50% reduction in incontinence episodes per day compared to placebo. The percentage of patients with a greater than 50% reduction after 12 weeks of enobosarm treatment was 58.9% for 3 mg, 57.7% for 1 mg and 52.7% for placebo. Enobosarm was generally safe and well tolerated, and reported adverse events were minimal and similar across all treatment groups. We have completed the ASTRID trial, including our review of the full data sets from the clinical trial, and have determined that there is not a sufficient path forward to warrant additional clinical development of enobosarm to treat SUI. We have therefore discontinued further development of enobosarm to treat SUI, including discontinuing the related durability and open-label safety extension studies we initiated before we received topline data from the ASTRID trial.

Evaluation of Enobosarm for the Treatment of Breast Cancer. We have previously evaluated enobosarm 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 with 9 patients achieving a clinical benefit response, or CBR,

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defined as a complete response, partial response, or stable disease, among the first 22 evaluable patients in that cohort. In November 2017, we announced that in the 9 mg cohort, a total of 14 patients achieved a CBR following 24 weeks of treatment. We also announced in November of 2017 that the 18 mg cohort achieved the pre-specified primary efficacy endpoint as 12 patients achieved a CBR at 24 weeks. Although both the 9 mg and 18 mg cohorts met the primary efficacy endpoint in the Phase 2 clinical trial, after evaluating the breast cancer environment where the treatment paradigms are shifting to immunotherapies and/or combination therapies, we decided in the third quarter of 2017 that the time and cost of conducting the necessary clinical trials for potential approval in this indication does not warrant further development of enobosarm in this indication. In 2015, we also commenced enrollment 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 were insufficient patients achieving clinical benefit from enobosarm treatment to continue this clinical trial and we closed the clinical trial down.

Discontinuation of SARM Development Efforts. Following our review of the full data sets from the ASTRID trial, we discontinued further development of enobosarm to treat SUI and otherwise discontinued any further development of our SARM program. We continue our efforts to seek potential collaborative, partnering or other strategic arrangements for our SARM assets, including a sale or other divestiture of our SARM assets. If the Merger is completed, any net proceeds derived from the disposition or licensing of our SARM assets following completion of the Merger will be made available to our stockholders in accordance with the CVR Agreement. We have for many years actively pursued, but have been unable to successfully enter into, potential collaborative, partnering or other strategic arrangements for our SARM assets. If we are unable to ultimately enter into any such arrangements for our SARM assets, we will not receive any return on our investment in enobosarm and our other SARMs.

Licenses and Collaborative Relationships

We have in the past established and, if the proposed Merger is not completed, we may continue to pursue, in-licenses and partnering, and collaborative or other strategic relationships with academic institutions and with other pharmaceutical and biotechnology companies.

In March 2015, we and UTRF entered into a license agreement, or the SARD License Agreement, pursuant to which we were granted exclusive worldwide rights in all existing SARD technologies owned or controlled by UTRF, including all improvements thereto. Under the SARD License Agreement, we are 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. We are 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. Unless terminated earlier, the term of the SARD License Agreement will continue, on a country-by-country basis, until the expiration of the last valid claim of any licensed patent in the particular country in which a licensed patent is granted. UTRF may terminate the SARD License Agreement for our uncured breach or upon our bankruptcy.

In July 2007, we and UTRF also previously entered into a consolidated, amended and restated license agreement, or the SARM License Agreement, to consolidate and replace our two previously

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existing SARM license agreements with UTRF and to modify and expand certain rights and obligations of each of the parties under both license agreements. Pursuant to the SARM License Agreement, we were granted exclusive worldwide rights in all existing SARM technologies owned or controlled by UTRF, including enobosarm, and certain improvements thereto, and exclusive rights to certain 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. Unless terminated earlier, the term of the SARM License Agreement will continue, on a country-by-country basis, for the longer of 20 years or until the expiration of the last valid claim of any licensed patent in the particular country in which a licensed product is being sold. UTRF may terminate the SARM License Agreement for our uncured breach or upon our bankruptcy.

Under the SARM License Agreement, we paid UTRF a one-time, upfront fee of $290,000 as consideration for entering into the SARM License Agreement. We are also 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. We also agreed to pay all expenses to file, prosecute and maintain the patents relating to the licensed SARM technologies, and are obligated to use commercially reasonable efforts to develop and commercialize products based on the licensed SARM technologies. While we currently have ceased development efforts for SARMs, we continue to seek potential collaborative, partnering or other strategic arrangements for our SARM assets, including a sale or other divestiture of our SARM assets. In December 2008, we and UTRF amended the SARM License Agreement, or the SARM License Amendment, to, among other things, clarify the treatment of certain payments that we may receive from our current and future sublicensees for purposes of determining sublicense fees payable to UTRF, including the treatment of payments made to us in exchange for the sale of our securities in connection with sublicensing arrangements. In consideration for the execution of the SARM License Amendment, we paid UTRF $494,000.

Manufacturing

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 any product candidates.

There are no complicated chemistries or unusual equipment required in the manufacturing process for either SARMs or SARDs. We rely and expect to continue to rely on third-party vendors for drug substance and drug product manufacturing, including drug substance for SARDs used in our current and potential future preclinical studies.

Competition

The biotechnology and biopharmaceutical industries are characterized by rapidly advancing technologies, intense competition and a strong emphasis on proprietary products. We face competition from many different sources, including commercial pharmaceutical and biotechnology enterprises, academic institutions, government agencies and private and public research institutions.

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. Our commercial opportunities will be reduced or eliminated if our competitors

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develop and commercialize similar products that are safer, more effective, have fewer side effects or are less expensive than any products that we and/or our collaborators may develop.

SARDs for the Potential Treatment of CRPC

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 or antagonize multiple forms of the AR thereby inhibiting tumor growth in patients with CRPC, including those patients who do not respond or are resistant to current therapies. Drugs in development having potentially similar approaches to removing the AR by degradation include Arvinas Inc.'s ARV-110, which is a chimera with an AR binding moiety on one end and an E3 ligase recruiting element on the other that has recently entered Phase 1 development for the treatment of advanced prostate cancer, and Androscience Corporation's androgen receptor degrader enhancer, ASC-J9, which is currently in development for acne and alopecia with the potential for development as a treatment for prostate cancer. Additionally, Essa Pharma Inc. recently completed a Phase 1 study with EPI-506, an AR antagonist that targets the N-terminal domain of the AR, and has plans to develop a second generation agent. 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 recently initiated a clinical trial with CCS1477 in prostate cancer. Oric Pharmaceuticals is targeting the glucocorticoid receptor as a means to impact men that have CRPC, and has a lead candidate ORIC-101 in preclinical testing. 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. XTANDI® received FDA approval in July 2018 for the treatment of men with non-metastatic CRPC. Zytiga®, sold by Johnson & Johnson, has been approved for the treatment of metastatic CRPC and metastatic high-risk castration-sensitive prostate cancer. Johnson & Johnson also received FDA approval for a second generation anti-androgen ERLEADA (apalutamide) for the treatment of men with non-metastatic castrate-resistant prostate cancer. Bayer HealthCare and Orion Corporation recently announced that the primary endpoint of increased metastatic free survival was met in a Phase 3 study of darolutamide (ODM-201) in men with CRPC without metastases and with a rising PSA. Another target in prostate cancer that is being pursued by several companies is bromodomain inhibition. Zenith Epigenetics, Gilead Sciences Inc., CellCentric, Incyte Corporation and GlaxoSmithKline are among the companies that are evaluating BET inhibitors in Phase 1-2 trials.

SARMs

With respect to SARMs, there are other SARM product candidates in development that may compete with enobosarm and any future SARM product candidates, if approved for commercial sale. For example, Viking Therapeutic's VK5211 recently reported positive results from a Phase 2 study for patients recovering from non-elective hip fracture surgery. Radius Health Inc.'s RAD140 is currently being evaluated in a Phase 1 study in postmenopausal women with hormone-receptor positive locally advanced or metastatic breast cancer. GlaxoSmithKline is conducting a Phase 1 study to assess the effect of GSK2881078 on physical strength and function after 13 weeks of treatment in patients with chronic obstructive pulmonary disease, or COPD, and muscle weakness. OPKO Health's OPK88004 is enrolling in a dose ranging study to improve symptoms of benign prostatic hyperplasia (BPH) by

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reducing prostate size and, on the basis of data from a previous trial in 350 men, increase muscle mass and bone strength and decrease body fat.

Intellectual Property

We will be able to protect our technology from unauthorized use by third parties only to the extent it is covered by valid and enforceable patents or is effectively maintained as trade secrets. Patents and other proprietary rights are an essential element of our business.

For our SARD compounds and methods of use thereof, we have filed certain patent applications in the United States, Canada, Mexico, Australia, Japan, China, and other countries in Asia and before the European Patent Office and are the exclusive licensee of worldwide rights for the SARD technology under a license agreement with UTRF executed in 2015. Thus far we have six issued patents and one is allowed, all in the United States. The patents and patent applications (if are issued) will expire between 2036 and 2039.

For enobosarm and our other SARM compounds, we have an exclusive license from UTRF under its issued patents and pending patent applications in the United States, Canada, Australia, Japan, China and other countries in Asia, before the European Patent Office designating Germany, Great Britain, Spain, France, Italy, and other European Union countries, as well as in certain other countries outside those regions, covering the composition of matter of the active pharmaceutical ingredient for pharmaceutical products, pharmaceutical compositions and methods of synthesizing the active pharmaceutical ingredients. We have also exclusively licensed from UTRF issued and pending patent applications in the United States, Canada, Australia, Japan, China and other countries in Asia, before the European Patent Office designating Germany, Great Britain, Spain, France, Italy and other European Union countries, as well as in certain other countries outside those regions, related to methods for treating muscle wasting disorders, including Duchenne Muscular Dystrophy, or DMD, and cancer cachexia, and for treating conditions such as SUI and fecal incontinence, as well as sarcopenia, and increasing muscle performance, muscle size and muscle strength and increasing the strength of or mass of a bone and for treating bone related disorders, including bone frailty and osteoporosis. Issued patents for enobosarm composition of matter that we licensed from UTRF and issued in the United States expire in 2024. Issued patents for composition of matter for our other SARM compounds in the United States will expire from 2021-2029, depending on the specific SARM compound. The issued patents outside of the United States for enobosarm expire in 2025, and with respect to other SARM compounds, expire in 2023 and 2027, depending on the specific SARM compound. We have pending patent applications directed to composition of matter and methods of use for our other SARM compounds that, if issued, would expire in the United States and in countries outside the United States in 2027. We have issued patents in the United States, and issued patents and pending applications in countries outside the United States for enobosarm and certain other SARM compounds as a feed composition for animals. The patents in the United States will expire in 2025. Issued patents outside the United States, and patent applications, if issued, which are pending outside the United States, will expire in 2027 or 2031 depending on the country. Patent applications which are pending in the United States and outside the United States using SARMs for SUI and pelvic floor disorders will expire in 2035, if the patents are issued. Our issued patent in the United States using enobosarm for DMD will expire in 2021. Our issued patent in the United States using other SARMs for DMD will expire in 2024. Patent applications, if issued, which are pending in the United States, using other SARMs for DMD will expire in 2024 or 2027 depending on the SARM.

We have our own issued patents and pending patent applications in the United States, Canada, Australia, Europe, Japan, China and other countries in Asia, as well as in certain other countries outside those regions, related to solid forms of enobosarm. Issued patents covering solid forms of

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enobosarm in the United States will expire in 2029. Issued patents and pending patent applications, if issued, in countries outside of the United States will expire in 2028. We have our own pending patent applications and issued patents in the United States and in Europe, Canada, Australia, Japan, China and other countries in Asia related to methods of treating breast cancer using our SARM compounds. Such patents and patent applications, if issued, would expire in 2033 in the United States and outside of the United States. We have issued patents in the United States directed to androgen receptor positive breast cancer in general, various categories of estrogen receptor and androgen receptor positive breast cancer, as well as triple negative breast cancer.

We cannot be certain that any of our pending patent applications, or those of UTRF, will result in issued patents. In addition, because the patent positions of biopharmaceutical companies are highly uncertain and involve complex legal and factual questions, the patents we own and license, or any further patents we may own or license, may not prevent other companies from developing similar or therapeutically equivalent products. Patents also will not protect our product candidates if competitors devise ways of making or using these product candidates without legally infringing our patents. In recent years, several companies have been extremely aggressive in challenging patents covering pharmaceutical products, and the challenges have often been successful. We cannot be assured that our patents will not be challenged by third parties or that we will be successful in any defense we undertake. Failure to successfully defend a patent challenge could materially and adversely affect our business.

In addition, changes in patent laws, rules or regulations or in their interpretations in the United States and other countries by the courts may materially diminish the value of our intellectual property or narrow the scope of our patent protection, which could have a material adverse effect on our business and financial condition.

We also rely on trade secrets, technical know-how and continuing innovation to develop and maintain our competitive position. We seek to protect our proprietary information by requiring our employees, consultants, contractors, outside scientific collaborators and other advisors to execute non-disclosure and confidentiality agreements and our employees to execute assignment of invention agreements to us on commencement of their employment. Agreements with our employees also prevent them from bringing any proprietary rights of third parties to us. We also require confidentiality or material transfer agreements from third parties that receive our confidential data or materials.

Government Regulation

New Drug Development and Approval Process

Numerous governmental authorities in the United States and other countries extensively regulate the testing, clinical development, manufacturing and marketing of pharmaceutical products and ongoing research and development activities. In the United States, the FDA rigorously reviews pharmaceutical products under the Federal Food, Drug, and Cosmetic Act and applicable regulations. Non-compliance with FDA regulations can result in administrative and judicial sanctions, including warning or untitled letters, clinical holds, fines, recall or seizure of products, injunctions, total or partial suspension of production, refusal of the government to approve marketing applications or allow entry into supply contracts, refusal to permit import or export of products, civil penalties, criminal prosecution and other actions affecting a company and its products. The FDA also has the authority to revoke previously granted marketing authorizations.

To secure FDA approval, an applicant must submit extensive preclinical and clinical data, as well as information about product manufacturing processes and facilities and other supporting information

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to the FDA for each indication to establish a product candidate's safety and efficacy. The development and approval process takes many years, requires the expenditure of substantial resources and may be subject to delays or limitations of approval or rejection of an applicant's new drug application, or NDA. Even if the FDA approves a product, the approval is subject to post-marketing surveillance, adverse drug experience and other recordkeeping and reporting obligations, and may involve ongoing requirements for post-marketing studies. The FDA also has authority to place conditions on any approvals that could restrict the commercial applications, advertising, promotion or distribution of these products. Product approvals may be withdrawn if compliance with regulatory standards is not maintained or if problems occur following initial marketing.

Preclinical and Clinical Testing

Preclinical studies involve laboratory evaluation of product characteristics and animal studies to assess the biological activity and safety of the product. In some cases, long-term preclinical studies are conducted while clinical studies are ongoing. The FDA, under its Good Laboratory Practices regulations, regulates preclinical studies. Violations of these regulations can, in some cases, lead to invalidation of the studies, requiring these studies to be replicated. When the preclinical testing is considered adequate by the sponsor to demonstrate the safety and scientific rationale for initial human studies, the results of the preclinical tests, together with manufacturing information and analytical data, are submitted to the FDA as part of an IND submission. The IND becomes effective, if not rejected by the FDA, within 30 days after the FDA receives the IND. The FDA may, either during the 30-day period after filing of an IND or at any future time, impose a clinical hold on proposed or ongoing clinical trials on various grounds, including that the study subjects are or would be exposed to an unreasonable and significant health risk. If the FDA imposes a clinical hold, clinical trials cannot commence or recommence without FDA authorization and then only under terms authorized by the FDA.

Clinical trials involve the administration of the investigational product candidates to humans under the supervision of a qualified principal investigator. Clinical trials must be conducted in accordance with Good Clinical Practices under protocols submitted to the FDA as part of the IND. In addition, each clinical trial must be approved and conducted under the auspices of an Investigational Review Board, or IRB, and with patient informed consent. The IRB typically considers, among other things, ethical factors and the safety of human subjects.

Clinical trials are conducted in three sequential phases, but the phases may overlap. Phase 1 clinical trials usually involve healthy human subjects. The goal of a Phase I clinical trial is to establish initial data about the safety, tolerability and pharmacokinetic properties of the product candidates in humans. In Phase 2 clinical trials, controlled studies are conducted on an expanded population of patients with the targeted disease. The primary purpose of these tests is to evaluate the initial effectiveness of the drug candidate on the intended target and to determine if there are any side effects or other risks associated with the drug and to determine the optimal dose of the drug from the safety and efficacy profile developed from the clinical study. Phase 3 trials involve even larger patient populations, often with several hundred or even several thousand patients, depending on the use for which the drug is being studied. Phase 3 trials are intended to establish the overall risk-benefit ratio of the drug and provide, if appropriate, an adequate basis for product labeling. During all clinical trials, physicians monitor the patients to determine effectiveness and to observe and report any reactions or other safety risks that may result from use of the drug candidate.

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Product Formulation and Manufacture

Concurrent with clinical trials and preclinical studies, companies must develop information about the chemistry and physical characteristics of the drug and finalize a process for manufacturing the product. In addition, manufacturers, including contract manufacturers, are required to comply with current applicable FDA Good Manufacturing Practice, or cGMP, regulations. The cGMP regulations include requirements relating to quality control and quality assurance, as well as the corresponding maintenance of records and documentation. The manufacturing process must be capable of consistently producing quality batches of the product and the manufacturer must develop methods for testing the quality, purity and potency of the final drugs. Additionally, appropriate packaging must be selected and tested and chemistry stability studies must be conducted to demonstrate that the product does not undergo unacceptable deterioration over its shelf-life.

Compliance with cGMP regulations also is a condition of new drug application approval. The FDA must approve manufacturing facilities before they can be used in the commercial manufacture of drug products. In addition, manufacturing establishments are subject to pre-approval inspections and unannounced periodic inspections.

New Drug Application Process

After the completion of the clinical trial phases of development, if the sponsor concludes that there is substantial evidence that the drug candidate is safe and effective for its intended use, the sponsor may submit a NDA to the FDA. The application must contain all of the information on the drug candidate gathered to that date, including data from the clinical trials, and be accompanied by a user fee.

Under the Prescription Drug User Fee Act, or PDUFA, submission of a NDA with clinical data requires payment of a fee, with some exceptions. In return, the FDA assigns a goal of six or ten months from filing of the application to return of a first "complete response," in which the FDA may approve the product or request additional information. There can be no assurance that an application will be approved within the performance goal timeframe established under PDUFA. The FDA initially determines whether a NDA as submitted is acceptable for filing. The FDA may refuse to file an application, in which case the FDA retains one-half of the user fees. If the submission is accepted for filing, the FDA begins an in-depth review of the application. As part of this review, the FDA may refer the application to an appropriate advisory committee, typically a panel of clinicians, for review, evaluation and a recommendation. The FDA is not bound by the recommendation of an advisory committee.

If the FDA evaluations of the NDA and the manufacturing facilities are favorable, the FDA may issue an approval letter authorizing commercial marketing of the drug candidate for specified indications. The FDA could also issue a "complete response" letter at the end of the review period. A "complete response" letter will be issued to let a company know that the review period for a drug is complete and that the application is not yet ready for approval. The letter will describe specific deficiencies and, when possible, will outline recommended actions the applicant might take to get the application ready for approval, including calling for additional clinical trial data.

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Marketing Approval and Post-Marketing Obligations

If the FDA approves an application, the drug becomes available for physicians to prescribe. Periodic reports must be submitted to the FDA, including descriptions of any adverse reactions reported. The FDA may require post-marketing studies, also known as Phase IV studies, as a condition of approval. In addition to studies required by the FDA after approval, trials and studies are often conducted to explore new indications for the drug. The purpose of these trials and studies and related publications is to develop data to support additional indications for the drug, which must be approved by the FDA, and to increase its acceptance in the medical community. In addition, some post-marketing studies are done at the request of the FDA to develop additional information regarding the safety of a product.

The FDA may 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 marketplace. REMS could add training requirements for healthcare professionals, safety communications 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. Whether a REMS would be imposed on a product and any resulting financial impact is uncertain at this time.

Any products manufactured or distributed pursuant to FDA approvals are subject to continuing regulation by the FDA, including record keeping requirements, reporting of adverse experiences with the drug, drug sampling and distribution requirements, notifying the FDA and gaining its approval of certain manufacturing or labeling changes, complying with certain electronic records and signature requirements, and complying with FDA promotion and advertising requirements. Drug manufacturers and their subcontractors are required to register their establishments and are subject to periodic unannounced inspections for compliance with cGMP requirements. Also, newly discovered or developed safety or effectiveness data may require changes to a product's approved labeling, including the addition of new warnings and contraindications, or even in some instances revocation or withdrawal of the product's approval.

Approval Outside of the United States

In order to market any product outside of the United States, we must comply with numerous and varying regulatory requirements of other countries regarding safety and efficacy and governing, among other things, clinical trials and commercial sales and distribution of our products, which broadly reflect the issues addressed by the FDA above. Approval procedures vary among countries and can involve additional product testing and additional administrative review periods. The time required to obtain approval in other countries might differ from and be longer than that required to obtain FDA approval. Marketing approval in one country does not ensure marketing approval in another, but a failure or delay in obtaining marketing approval in one country may negatively impact the regulatory process in other countries.

As in the United States, the marketing approval process in Europe and in other countries is a lengthy, challenging and inherently uncertain process. If we fail to comply with applicable foreign regulatory requirements, we may be subject to fines, suspension or withdrawal of marketing approvals, product recalls, seizure of products, operating restrictions and criminal prosecution. Generally the development and approval procedures are harmonized throughout the European Union: however, there is limited harmonization in relation to national pricing and reimbursement practices.

Under European Union regulatory systems, a company may not market a medicinal product without marketing authorization. There are three procedures for submitting a MAA in the EU: (1) the

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mutual recognition procedure (MRP); (2) the decentralized procedure (DCP) and (3) the centralized procedure (CP). The submission strategy for a given product will depend on the nature of the product, the target indication(s), the history of the product, and the marketing plan. The centralized procedure is compulsory for medicinal products which are produced by biotechnology processes, advanced therapy medicinal products and orphan drugs. Besides the products falling under the mandatory scope, the centralized procedure is also open for other innovative products that are new active substances or other medicinal products that constitute a significant therapeutic, scientific or technical innovation.

The centralized procedure leads to approval of the product in all 27 EU member states and in Norway, Iceland and Liechtenstein. Submission of one MAA thus leads to one assessment process and one authorization that allows access to all applicable markets within the entire EU. The process of the centralized procedure is triggered when the applicant sends the letter announcing the intent to submit a MAA (letter of intent). The letter of intent also initiates the assignment of the Rapporteur and Co-Rapporteur, who are the two appointed members of the Committee for Human Medicinal Products, or CHMP, representing two EU member states. However, in light of the United Kingdom's vote in 2016 to leave the European Union, the so-called Brexit vote, there may be changes forthcoming in the scope of the centralized approval procedure as the terms of that exit are negotiated between the UK and the European Union.

When using the MRP or DCP, the applicant must select which and how many EU member states in which to seek approval. In the case of an MRP, the applicant must initially receive national approval in one EU member state. This will be the so-called reference member state (RMS) for the MRP. Then, the applicant seeks approval for the product in other EU member states, the so-called concerned member states (CMS) in a second step: the mutual recognition process. For the DCP, the applicant will approach all chosen member states at the same time. To do so, the applicant will identify the RMS that will assess the submitted MAA and provide the other selected member states with the conclusions and results of the assessment.

When the application for marketing authorization is made, the competent authority responsible for granting a marketing authorization must verify whether the application complies with the relevant requirements, including compliance with the agreed pediatric investigational plan, or PIP. Assuming it does, the marketing authorization may be granted and the relevant results are included in the summary of product characteristics (SmPC) for the product, along with a statement indicating compliance with the agreed PIP. It is not necessary for the product actually to be indicated for use in the pediatric population (for example, if the results show that that would not be appropriate).

Drug Price Competition and Patent Term Restoration Act of 1984

Under the Drug Price Competition and Patent Term Restoration Act of 1984, known as the Hatch-Waxman Act, a portion of a product's patent term that was lost during clinical development and application review by the FDA may be restored. The Hatch-Waxman Act also provides for a statutory protection, known as exclusivity, against the FDA's acceptance or approval of certain competitor applications. The Hatch-Waxman Act also provides the legal basis for the approval of abbreviated new drug applications, or ANDAs.

Patent term extension can compensate for time lost during product development and the regulatory review process by returning up to five years of patent life for a patent that covers a new product or its use. This period is generally one-half the time between the effective date of an IND and the submission date of a NDA, plus the time between the submission date of a NDA and the approval of that application. Patent term extensions, however, are subject to a maximum extension of five years, and the patent term extension cannot extend the remaining term of a patent beyond a total of 14 years.

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The application for patent term extension is subject to approval by the United States Patent and Trademark Office in conjunction with the FDA. It generally takes at least six months to obtain approval of the application for patent term extension.

The Hatch-Waxman Act also provides for a period of statutory protection for new drugs that receive NDA approval from the FDA. If a new drug receives NDA approval as a new chemical entity, meaning that the FDA has not previously approved any other new drug containing the same active entity, then the Hatch-Waxman Act prohibits an ANDA or a NDA submitted pursuant to section 505(b)(2) of the Federal Food, Drug, and Cosmetics Act, where the applicant does not own or have a legal right of reference to all of the data required for approval to be submitted by another company for a generic version of such drug (505(b)(2) NDA), with some exceptions, for a period of five years from the date of approval of the NDA. The statutory protection provided pursuant to the Hatch-Waxman Act will not prevent the filing or approval of a full NDA, as opposed to an ANDA or 505(b)(2) NDA, for any drug, including, for example, a drug with the same active ingredient, dosage form, route of administration, strength and conditions of use. In order to obtain a NDA, however, a competitor would be required to conduct its own clinical trials, and any use of the drug for which marketing approval is sought could not violate another NDA holder's patent claims.

If NDA approval is received for a new drug containing an active ingredient that was previously approved by the FDA but the NDA is for a drug that includes an innovation over the previously approved drug, for example, a NDA approval for a new indication or formulation of the drug with the same active ingredient, and if such NDA approval was dependent upon the submission to the FDA of new clinical investigations, other than bioavailability studies, then the Hatch-Waxman Act prohibits the FDA from making effective the approval of an ANDA or 505(b)(2) NDA for a generic version of such drug for a period of three years from the date of the NDA approval. This three year exclusivity, however, only covers the innovation associated with the NDA to which it attaches. Thus, the three year exclusivity does not prohibit the FDA, with limited exceptions, from approving ANDAs or 505(b)(2) NDAs for drugs containing the same active ingredient but without the new innovation.

While the Hatch-Waxman Act provides certain patent restoration and exclusivity protections to innovator drug manufacturers, it also permits the FDA to approve ANDAs for generic versions of their drugs assuming the approval would not violate another NDA holder's patent claims. The ANDA process permits competitor companies to obtain marketing approval for a drug with the same active ingredient for the same uses but does not require the conduct and submission of clinical studies demonstrating safety and effectiveness for that product. Instead of safety and effectiveness data, an ANDA applicant needs only to submit data demonstrating that its product is bioequivalent to the innovator product as well as relevant chemistry, manufacturing and product data. The Hatch-Waxman Act also instituted a third type of drug application that requires the same information as a NDA, including full reports of clinical and preclinical studies, except that some of the information from the reports required for marketing approval comes from studies which the applicant does not own or have a legal right of reference. This type of application, a 505(b)(2) NDA, permits a manufacturer to obtain marketing approval for a drug without needing to conduct or obtain a right of reference for all of the required studies.

If a competitor submits an ANDA or 505(b)(2) NDA for a compound or use of any compound covered by another NDA holder's patent claims, the Hatch-Waxman Act requires, in some circumstances, the applicant to notify the patent owner and the holder of the approved NDA of the factual and legal basis of the applicant's opinion that the patent is not valid or will not be infringed. Upon receipt of this notice, the patent owner and the NDA holder have 45 days to bring a patent infringement suit in federal district court and obtain a 30-month stay against the company seeking to reference the NDA. The NDA holder could still file a patent suit after the 45 days, but if they miss the

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45-day deadline, they would not have the benefit of the 30-month stay. Alternatively, after this 45-day period, the applicant may file a declaratory judgment action, seeking a determination that the patent is invalid or will not be infringed. Depending on the circumstances, however, the applicant may not be able to demonstrate a controversy sufficient to confer jurisdiction on the court. The discovery, trial and appeals process in such suits can take several years. If such a suit is commenced, the Hatch-Waxman Act provides a 30-month stay on the approval of the competitor's ANDA or 505(b)(2) NDA. If the litigation is resolved in favor of the competitor or the challenged patent expires during the 30-month period, unless otherwise extended by court order, the stay is lifted and the FDA may approve the application. Under regulations issued by the FDA, and essentially codified under the Medicare prescription drug legislation, the patent owner and the NDA holder have the opportunity to trigger only a single 30-month stay per ANDA or 505(b)(2) NDA. Once the applicant of the ANDA or 505(b)(2) NDA has notified the patent owner and the NDA holder of the infringement, the applicant cannot be subjected to another 30-month stay, even if the applicant becomes aware of additional patents that may be infringed by its product.

Pharmaceutical Pricing and Reimbursement

We currently have no marketed products. In both domestic and foreign markets, sales of any products for which we receive regulatory approval for commercial sale will depend in part on the availability of reimbursement from third-party payors. Third-party payors include government authorities or programs, managed care providers, private health insurers and other organizations. These third-party payors are increasingly challenging the price and examining the cost-effectiveness of medical products and services. In addition, significant uncertainty exists as to the reimbursement status of newly approved healthcare products. We may need to conduct expensive pharmacoeconomic studies in order to demonstrate the cost-effectiveness of our products. Our product candidates may not be considered cost-effective. Adequate third-party reimbursement may not be available to enable us to maintain price levels sufficient to realize an appropriate return on our investment in product development. Third-party payors may also control access to, or manage utilization of, our products with various utilization management techniques, such as requiring prior authorization for coverage of our products.

Within the United States, if we obtain appropriate approval in the future to market any of our oral drug product candidates, those products could potentially be covered by various government health benefit programs as well as purchased by government agencies. The participation in such programs or the sale of products to such agencies is subject to regulation. The marketability of any products for which we receive regulatory approval for commercial sale may suffer if the government and third-party payors fail to provide adequate coverage and reimbursement.

Medicaid is a joint federal and state program that is administered by the states for low income and disabled beneficiaries. Under the Medicaid Drug Rebate Program, participating manufacturers are required to pay a rebate for each unit of product reimbursed by the state Medicaid programs. The amount of the rebate for each product is set by law and may be subject to an additional discount if certain pricing increases more than inflation.

Medicare is a federal program that is administered by the federal government that covers individuals age 65 and over as well as those with certain disabilities. Oral drugs may be covered under Medicare Part D. Medicare Part D provides coverage to enrolled Medicare patients for self-administered drugs (*i.e.*, drugs that do not need to be injected or otherwise administered by a physician). Medicare Part D is administered by private prescription drug plans approved by the U.S. government and each drug plan establishes its own Medicare Part D formulary for prescription drug coverage and pricing, which the drug plan may modify from time-to-time. The prescription drug plans negotiate pricing with manufacturers and may condition formulary placement on the availability of

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manufacturer discounts. Since 2011, manufacturers with marketed brand name drugs have been required to provide a 50% discount the negotiated price for on brand name prescription drugs utilized by Medicare Part D beneficiaries when those beneficiaries reach the coverage gap in their drug benefits, and, beginning in 2019, that discount increased to 70%.

Drug products are subject to discounted pricing when purchased by federal agencies via the Federal Supply Schedule (FSS). FSS participation is required for a drug product to be covered and reimbursed by certain federal agencies and for coverage under Medicaid, Medicare Part B and the Public Health Service (PHS) pharmaceutical pricing program. FSS pricing is negotiated periodically with the Department of Veterans Affairs. FSS pricing is intended not to exceed the price that a manufacturer charges its most-favored non-federal customer for its product. In addition, prices for drugs purchased by the Veterans Administration, Department of Defense (including drugs purchased by military personnel and dependents through the TRICARE retail pharmacy program), Coast Guard, and PHS are subject to a cap on pricing (known as the "federal ceiling price") and may be subject to an additional discount if pricing increases more than the rate of inflation.

To maintain coverage of drugs under the Medicaid Drug Rebate Program, manufacturers are required to extend discounts to certain purchasers under the PHS pharmaceutical pricing program. Purchasers eligible for discounts include hospitals that serve a disproportionate share of financially needy patients, community health clinics and other entities that receive health services grants from the PHS.

The United States and state governments continue to propose and pass legislation designed to reform delivery of, or payment for, health care, which include initiatives to reduce the cost of healthcare. For example, in March 2010, the United States Congress enacted the Patient Protection and Affordable Care Act and the Health Care and Education Reconciliation Act ("Healthcare Reform Act") which includes changes to the coverage and reimbursement of drug products under government health care programs. Under the Trump administration, there have been ongoing efforts to modify or repeal all or certain provisions of the Healthcare Reform Act. For example, tax reform legislation was enacted at the end of 2017 that eliminates the tax penalty for individuals who do not maintain sufficient health insurance coverage beginning in 2019 (the so-called "individual mandate"). In a May 2018 report, the Congressional Budget Office estimated that, compared to 2018, the number of uninsured will increase by 3 million in 2019 and 6 million in 2028, in part due to the elimination of the individual mandate. The Healthcare Reform Act has also been subject to judicial challenge. In December 2018, a federal district court judge, in a challenge brought by a number of state attorneys general, found the Healthcare Reform Act unconstitutional in its entirety because, once Congress repealed the individual mandate provision, there was no longer a basis to rely on Congressional taxing authority to support enactment of the law. Pending appeals, which could take some time, the Healthcare Reform Act is still operational in all respects.

There have also been other reform initiatives under the Trump Administration, including initiatives focused on drug pricing. For example, in May of 2018, President Trump and the Secretary of the Department of Health and Human Services released a "blueprint" to lower prescription drug prices and out-of-pocket costs. Certain proposals in the blueprint, and related drug pricing measures proposed since the blueprint, could cause significant operational and reimbursement changes for the pharmaceutical industry. As another example, in November of 2018, CMS issued an advance notice of proposed rulemaking that proposed revisions to Medicare Part D to support health plans' negotiation of lower drug prices with manufacturers and reduce health plan members' out-of-pocket costs. The HHS Office of Inspector General also issued a proposed rule in February of 2019 that would revise the federal anti-kickback statute to limit protection for discounts offered by pharmaceutical manufacturers to pharmacy benefit managers ("PBMs"), Medicare Part D plans, and Medicaid managed care plans

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that are not reflected in the price charged to the patient at the pharmacy counter and to provide protection only for certain types of service fees paid by pharmaceutical manufacturers to PBMs.

Recently, there has been considerable public and government scrutiny in the U.S. of pharmaceutical pricing and proposals to address the perceived high cost of pharmaceuticals. There have also been several recent state legislative efforts to address drug costs, which generally have focused on increasing transparency around drug costs or limiting drug prices or price increases. Adoption of new legislation at the federal or state level could affect demand for, or pricing of, our product candidates if approved for sale.

We cannot predict the ultimate content, timing or effect of any changes to the Healthcare Reform Act or other federal and state reform efforts. There is no assurance that federal or state health care reform will not adversely affect our future business and financial results.

Although we currently have no products approved for commercial sale, we marketed FARESTON® through September 30, 2012 and the product was covered under various government health benefit programs as well as purchased by federal agencies. We could be subject to liability under federal laws regulating our participation in such programs or the sale of our product to such agencies if we failed to comply with applicable requirements, including reporting prices for our products or offering products for sale at certain prices.

Regulations Pertaining to Sales and Marketing

Although we currently have no products approved for commercial sale, we may be subject to various federal and state laws pertaining to health care "fraud and abuse," including anti-kickback laws and false claims laws for activities related to our previous sales of FARESTON®, which we sold to a third party in 2012, or to future sales of any of our product candidates that may in the future receive regulatory and marketing approval. Anti-kickback laws generally prohibit a prescription drug manufacturer from soliciting, offering, receiving, or paying any remuneration to generate business, including the purchase or prescription of a particular drug. Although the specific provisions of these laws vary, their scope is generally broad and there may not be regulations, guidance or court decisions that apply the laws to particular industry practices. There is therefore a possibility that our practices might be challenged under such anti-kickback laws. False claims laws prohibit anyone from knowingly and willingly presenting, or causing to be presented, any claims for payment for reimbursed drugs or services to third party payors (including Medicare and Medicaid) that are false or fraudulent. Violations of fraud and abuse laws may be punishable by criminal or civil sanctions, including fines and civil monetary penalties, and/or exclusion from federal health care programs (including Medicare and Medicaid).

Laws and regulations have been enacted by the federal government and various states to regulate the sales and marketing practices of pharmaceutical manufacturers with marketed products. The laws and regulations generally limit financial interactions between manufacturers and health care providers and/or require disclosure to the government and public of such interactions. Many of these laws and regulations contain ambiguous requirements or require administrative guidance for implementation. Given the lack of clarity in laws and their implementation, our prior activities (when we marketed FARESTON®) or any future activities (if we obtain approval and/or reimbursement from federal healthcare programs for our product candidates) could be subject to the penalty provisions of the pertinent laws and regulations.

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Employees

As of December 31, 2018, we had 21 employees, 6 of whom were M.D.s, Pharm.D.s and/or Ph.D.s. None of our employees are subject to a collective bargaining agreement. We believe that we have good relations with our employees.

Available Information

We were originally incorporated under the name Genotherapeutics, Inc. in Tennessee in September 1997. We changed our name to GTx, Inc. in 2001, and we reincorporated in Delaware in 2003. Our principal executive office is located at 175 Toyota Plaza, 7th Floor, Memphis, TN 38103, and our telephone number is (901) 523-9700.

We file electronically with the U.S. Securities and Exchange Commission, or SEC, our annual reports on Form 10-K, quarterly reports on Form 10-Q, current reports on Form 8-K and amendments to those reports filed or furnished pursuant to Section 13(a) or 15(d) of the Securities Exchange Act of 1934. We make available on our Web site at www.gtxinc.com, free of charge, copies of these reports as soon as reasonably practicable after we electronically file such material with, or furnish it to, the SEC. The information provided on our Web site is not part of this report, and is therefore not incorporated by reference unless such information is otherwise specifically referenced elsewhere in this report.

Executive Officers of the Registrant

The following table sets forth information about our executive officers as of March 12, 2019:

Name	Age	Position(s)
Executive Officers		
Marc S. Hanover	56	Chief Executive Officer
Robert J. Wills, Ph.D	65	Executive Chairman
Henry P. Doggrell	70	Vice President, Chief Legal Officer and Secretary
Jason T. Shackelford	43	Vice President, Finance and Accounting, and Principal Financial and Accounting Officer

Marc S. Hanover*,* a co-founder of GTx, served as our President and Chief Operating Officer from our inception in September 1997 until his appointment as our permanent Chief Executive Officer in February 2015, and served as our acting Principal Financial Officer from December 31, 2013 until his appointment as our interim Chief Executive Officer on April 3, 2014. He also previously served as a member of our Board of Directors from September 1997 to August 2011. Prior to joining GTx, Mr. Hanover was a founder of Equity Partners International, Inc., a private equity firm in Memphis, Tennessee, and participated as a founder and investor in three healthcare companies. From 1985 to 1997, Mr. Hanover was a Senior Vice President and a member of the Executive Management Committee of National Bank of Commerce in Memphis, Tennessee. Mr. Hanover holds a B.S. in Biology from the University of Memphis and an MBA in Finance from the University of Memphis.

Robert J. Wills, Ph.D., joined GTx as Executive Chairman of the Board of Directors and as the Chairman of the Board's Scientific and Development Committee on March 2, 2015. Prior to joining GTx, Dr. Wills served as Vice President, Alliance Manager for Johnson & Johnson (J&J) and was responsible for managing strategic alliances for J&J's Pharmaceutical Group worldwide since 2002. Prior to this, Dr. Wills spent 22 years in pharmaceutical drug development, 12 of which were at J&J and 10 of which were at Hoffmann-La Roche Inc. Before assuming his alliance management role at

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J&J, Dr. Wills served as Senior Vice President Global Development at J&J where he was responsible for its late stage development pipeline and was a member of several internal commercial and research and development operating boards. Since 2015, Dr. Wills has served as the chairman of the board of Cymabay Therapeutics Inc. (Nasdaq: CBAY). Dr. Wills holds a B.S. in Biochemistry and a M.S. in Pharmaceutics from the University of Wisconsin and a Ph.D. in Pharmaceutics from the University of Texas.

Henry P. Doggrell currently serves as our Vice President, Chief Legal Officer and Secretary, after joining GTx in October 2001 as General Counsel and Secretary. From April 1998 to August 2001, Mr. Doggrell was Senior Vice President, Corporate Affairs at Buckeye Technologies, Inc., a specialty cellulose company, where he was responsible for matters including corporate finance, investor relations, mergers and acquisitions, intellectual property and licensing and strategic development. From 1996 to 1998, Mr. Doggrell served as General Counsel and Secretary of Buckeye Technologies. Prior to joining Buckeye Technologies, Mr. Doggrell was a partner of the Baker, Donelson, Bearman, Caldwell and Berkowitz law firm from 1988 to 1996, where he served as a member of the law firm management committee and Chair of the firm's Corporate Securities department. Mr. Doggrell holds a B.S. in Commerce from the University of Virginia and a JD from Vanderbilt University.

Jason T. Shackelford currently serves as our Vice President, Finance and Accounting, after joining GTx in July 2007 as Director, Accounting and Corporate Controller, and has served as our principal accounting officer since December 31, 2013 and as our principal financial and accounting officer since April 3, 2014. Prior to joining GTx, Mr. Shackelford was a Senior Audit Manager at KPMG LLP. Mr. Shackelford is a Certified Public Accountant and holds a Bachelor of Business Administration and Master of Accountancy from the University of Mississippi.

ITEM 1A. RISK FACTORS

We have identified the following additional risks and uncertainties that may have a material adverse effect on our business, financial condition or results of operations. Investors should carefully consider the risks described below before making an investment decision. Our business faces significant risks and the risks described below may not be the only risks we face. Additional risks not presently known to us or that we currently believe are immaterial may also significantly impair our business operations. If any of these risks occur, our business, results of operations or financial condition could suffer, the market price of our common stock could decline and you could lose all or part of your investment in our common stock.

Risks Related to the Proposed Merger

The exchange ratio set forth in the Merger Agreement is not adjustable based on the market price of our common stock, so the merger consideration at the closing of the Merger may have a greater or lesser value than at the time the Merger Agreement was signed.

The Merger Agreement has set the exchange ratio for the Oncternal capital stock, and the exchange ratio is based on the outstanding capital stock of Oncternal and the outstanding common stock of GTx, in each case immediately prior to the closing of the Merger. Applying the exchange ratio formula in the Merger Agreement, the former Oncternal stockholders immediately before the Merger are expected to own approximately 75% of the outstanding capital stock of GTx immediately following the Merger, and the stockholders of GTx immediately before the Merger are expected to own approximately 25% of the outstanding capital stock of GTx immediately following the Merger, subject to certain assumptions. Under certain circumstances further described in the Merger Agreement, however, these ownership percentages may be adjusted upward or downward based on cash levels of

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the respective companies at the closing of the Merger, and as a result, either our stockholders or the Oncternal stockholders could own less of the combined company than expected.

Any changes in the market price of our common stock before the completion of the Merger will not affect the number of shares of our common stock issuable to Oncternal's stockholders pursuant to the Merger Agreement. Therefore, if before the completion of the Merger the market price of our common stock declines from the market price on the date of the Merger Agreement, then Oncternal's stockholders could receive merger consideration with substantially lower value than the value of such merger consideration on the date of the Merger Agreement. Similarly, if before the completion of the Merger the market price of our common stock increases from the market price of our common stock on the date of the Merger Agreement, then Oncternal's stockholders could receive merger consideration with substantially greater value than the value of such merger consideration on the date of the Merger Agreement. The Merger Agreement does not include a price-based termination right. Because the exchange ratio does not adjust as a result of changes in the market price of our common stock, for each one percentage point change in the market price of our common stock, there is a corresponding one percentage point rise or decline, respectively, in the value of the total merger consideration payable to Oncternal's stockholders pursuant to the Merger Agreement.

Failure to complete the proposed Merger may result in GTx and Oncternal paying a termination fee to the other party and could significantly harm the market price of our common stock and negatively affect the future business and operations of each company.

If the proposed Merger is not completed and the Merger Agreement is terminated under certain circumstances, we or Oncternal may be required to pay the other party a termination fee of up to $2.0 million. Even if a termination fee is not payable in connection with a termination of the Merger Agreement, each of GTx and Oncternal will have incurred significant fees and expenses, which must be paid whether or not the Merger is completed. Further, if the proposed Merger is not completed, it could significantly harm the market price of our common stock.

In addition, if the Merger Agreement is terminated and the board of directors of GTx or Oncternal determines to seek another business combination, there can be no assurance that either we or Oncternal will be able to find a partner and close an alternative transaction on terms that are as favorable or more favorable than the terms set forth in the Merger Agreement.

The proposed Merger is subject to approval of the Merger Agreement by our stockholders and the Oncternal stockholders. Failure to obtain these approvals would prevent the closing of the Merger.

Before the proposed Merger can be completed, the stockholders of each of GTx and Oncternal must approve the Merger Agreement. Additionally, the Merger Agreement must be approved by multiple classes of Oncternal preferred stockholders, one class of which is held by a sole stockholder, Shanghai Pharmaceutical (USA) Inc., which has not executed a voting agreement and has not otherwise agreed to vote in favor of the Merger Agreement. Although Oncternal expects to receive stockholder approval from Shanghai Pharmaceutical (USA) Inc. approximately two months after the date of the Merger Agreement, there can be no assurance that all of the necessary stockholder approvals will be obtained. Failure to obtain the required stockholder approvals, including as a result of Shanghai Pharmaceutical (USA) Inc. refusing to approve the transactions contemplated by the Merger Agreement, may result in a material delay in, or the abandonment of, the Merger. Any delay in completing the proposed Merger may materially adversely affect the timing and benefits that are expected to be achieved from the proposed Merger.

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The Merger may be completed even though certain events occur prior to the closing that materially and adversely affect GTx or Oncternal.

The Merger Agreement provides that either GTx or Oncternal can refuse to complete the proposed Merger if there is a material adverse change affecting the other party between March 6, 2019, the date of the Merger Agreement, and the closing of the Merger. However, certain types of changes do not permit either party to refuse to complete the proposed Merger, even if such change could be said to have a material adverse effect on GTx or Oncternal, including:

- general business, economic or political conditions or conditions generally affecting the industries in which Oncternal or GTx, as applicable, operates;
- any natural disaster or any acts of war, armed hostilities or terrorism;
- any changes in financial, banking or securities markets;
- with respect to GTx, any change in the stock price or trading volume of GTx excluding any underlying effect that may have caused such change;
- with respect to GTx, failure to meet internal or analysts' expectations or projects or the results of operations;
- any clinical trial programs or studies, including any adverse data, event or outcome arising out of or related to any such programs or studies;
- any change in accounting requirements or principles or any change in applicable laws, rules, or regulations or the interpretation thereof;
- any effect resulting from the announcement or pendency of the proposed Merger or any related transactions; and
- the taking of any action, or the failure to take any action, by either GTx or Oncternal required to comply with the terms of the Merger Agreement.

If adverse changes occur and GTx and Oncternal still complete the Merger, the market price of the combined organization's common stock may suffer. This in turn may reduce the value of the Merger to the stockholders of GTx, Oncternal or both.

Some GTx and Oncternal officers and directors have interests in the proposed Merger that are different from the respective stockholders of GTx and Oncternal and that may influence them to support or approve the Merger without regard to the interests of the respective stockholders of GTx and Oncternal.

Certain officers and directors of GTx and Oncternal participate in arrangements that provide them with interests in the proposed Merger that are different from the interests of the respective stockholders of GTx and Oncternal, including, among others, the continued service as an officer or director of the combined organization, severance benefits, the acceleration of stock option vesting, continued indemnification and the potential ability to sell an increased number of shares of common stock of the combined organization in accordance with Rule 144 under the Securities Act of 1933, as amended.

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For example, we have entered into certain employment and severance benefits agreements with certain of our executive officers that may result in the receipt by such executive officers of cash severance payments and other benefits in the event of a covered termination of employment of each executive officer's employment. The closing of the Merger will also result in the acceleration of vesting of options to purchase shares of our common stock held by our executive officers and directors, whether or not there is a covered termination of such officer's employment. In addition, and for example, certain of Oncternal's directors and executive officers have options, subject to vesting, to purchase shares of Oncternal's common stock which, at the closing of the Merger, shall be converted into and become options to purchase shares of our common stock, certain of Oncternal's directors and executive officers are expected to become directors and executive officers of GTx upon the closing of the Merger, and all of Oncternal's directors and executive officers are entitled to certain indemnification and liability insurance coverage pursuant to the terms of the Merger Agreement. These interests, among others, may influence the officers and directors of GTx and Oncternal to support or approve the proposed Merger.

The market price of our common stock following the Merger may decline as a result of the Merger.

The market price of our common stock may decline as a result of the Merger for a number of reasons including if:

- investors react negatively to the prospects of the combined organization's product candidates, business and financial condition following the Merger;
- the effect of the Merger on the combined organization's business and prospects is not consistent with the expectations of financial or industry analysts; or
- the combined organization does not achieve the perceived benefits of the Merger as rapidly or to the extent anticipated by financial or industry analysts.

GTx and Oncternal securityholders will have a reduced ownership and voting interest in, and will exercise less influence over the management of, the combined organization following the closing of the Merger as compared to their current ownership and voting interest in the respective companies.

After the completion of the Merger, the current securityholders of GTx and Oncternal will own a smaller percentage of the combined organization than their ownership in their respective companies prior to the Merger. Immediately after the Merger, it is currently estimated that Oncternal securityholders will own approximately 75% of the common stock of the combined organization, and GTx securityholders, whose shares of GTx common stock will remain outstanding after the Merger, will own approximately 25% of the common stock of the combined organization. These estimates are based on the anticipated exchange ratio and are subject to adjustment as provided in the Merger Agreement. See also the risk factor above titled, "*The exchange ratio is not adjustable based on the market price of GTx common stock, so the merger consideration at the closing may have a greater or lesser value than at the time the Merger Agreement was signed.*"

In addition, the nine member board of directors of the company will initially include seven individuals with prior affiliations with Oncternal and two individuals with prior affiliations with GTx. Consequently, securityholders of GTx and Oncternal will be able to exercise less influence over the management and policies of the combined organization following the closing of the Merger than they currently exercise over the management and policies of their respective companies.

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GTx and Oncternal stockholders may not realize a benefit from the Merger commensurate with the ownership dilution they will experience in connection with the Merger.

If the combined organization is unable to realize the strategic and financial benefits currently anticipated from the proposed Merger, GTx's and Oncternal's stockholders will have experienced substantial dilution of their ownership interests in their respective companies without receiving the expected commensurate benefit, or only receiving part of the commensurate benefit to the extent the combined organization is able to realize only part of the expected strategic and financial benefits currently anticipated from the proposed Merger.

The combined company will need to raise additional capital by issuing securities or debt or through licensing or other strategic arrangements, which may cause dilution to the combined company's stockholders or restrict the combined company's operations or impact its proprietary rights.

The combined company may be required to raise additional funds sooner than currently planned. In this regard, while the exchange ratio may be impacted by cash levels of the respective companies at the closing of the Merger, the Merger Agreement does not condition the completion of the Merger upon either company holding a minimum amount of cash at the effective time of the Merger. If either or both of GTx or Oncternal hold less cash at the time of the closing Merger than the parties currently expect, the combined company will need to raise additional capital sooner than expected. Additional financing may not be available to the combined company when it needs it or may not be available on favorable terms. To the extent that the combined company raises additional capital by issuing equity securities, such an issuance may cause significant dilution to the combined company's stockholders' ownership and the terms of any new equity securities may have preferences over the combined company's common stock. Any debt financing the combined company enters into may involve covenants that restrict its operations. These restrictive covenants may include limitations on additional borrowing and specific restrictions on the use of the combined company's assets, as well as prohibitions on its ability to create liens, pay dividends, redeem its stock or make investments. In addition, if the combined company raises additional funds through licensing, partnering or other strategic arrangements, it may be necessary to relinquish rights to some of the combined company's technologies or product candidates and proprietary rights, or grant licenses on terms that are not favorable to the combined company.

During the pendency of the proposed Merger, GTx and Oncternal may not be able to enter into a business combination with another party at a favorable price because of restrictions in the Merger Agreement, which could adversely affect their respective businesses.

Covenants in the Merger Agreement impede the ability of GTx and Oncternal to make acquisitions, subject to certain exceptions relating to fiduciary duties, as set forth below, or to complete other transactions that are not in the ordinary course of business pending completion of the proposed Merger. As a result, if the Merger is not completed, the parties may be at a disadvantage to their competitors during such period. In addition, while the Merger Agreement is in effect, each party is generally prohibited from soliciting, initiating, encouraging or entering into certain extraordinary transactions, such as a merger, sale of assets, or other business combination outside the ordinary course of business with any third party, subject to certain exceptions relating to fiduciary duties. Any such transactions could be favorable to such party's stockholders.

Certain provisions of the Merger Agreement may discourage third parties from submitting alternative takeover proposals, including proposals that may be superior to the arrangements contemplated by the Merger Agreement.

The terms of the Merger Agreement prohibit each of GTx and Oncternal from soliciting alternative takeover proposals or cooperating with persons making unsolicited takeover proposals,

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except in limited circumstances when such party's board of directors determines in good faith that an unsolicited alternative takeover proposal is or is reasonably likely to lead to a superior takeover proposal and that failure to cooperate with the proponent of the proposal would be reasonably likely to be inconsistent with the applicable board's fiduciary duties.

Because the lack of a public market for Oncternal's capital stock makes it difficult to evaluate the value of Oncternal's capital stock, the stockholders of Oncternal may receive shares of our common stock in the Merger that have a value that is less than, or greater than, the fair market value of Oncternal's capital stock.

The outstanding capital stock of Oncternal is privately held and is not traded in any public market. The lack of a public market makes it extremely difficult to determine the fair market value of Oncternal. Because the percentage of our common stock to be issued to Oncternal's stockholders was determined based on negotiations between the parties, it is possible that the value of our common stock to be received by Oncternal's stockholders will be less than the fair market value of Oncternal, or GTx may pay more than the aggregate fair market value for Oncternal.

If the conditions to the Merger are not met, the Merger will not occur.

Even if the Merger is approved by the stockholders of GTx and Oncternal, specified conditions must be satisfied or waived to complete the Merger. We cannot assure you that all of the conditions will be satisfied or waived. If the conditions are not satisfied or waived, the Merger will not occur or will be delayed, and GTx and Oncternal each may lose some or all of the intended benefits of the proposed Merger.

Litigation relating to the proposed Merger could require GTx or Oncternal to incur significant costs and suffer management distraction, and could delay or enjoin the proposed Merger.

GTx and Oncternal could be subject to demands or litigation related to the proposed Merger, whether or not the Merger is consummated. Such actions may create uncertainty relating to the Merger, or delay or enjoin the Merger, and responding to such demands.

Risks Related to Our Financial Condition and Our Need for Additional Financing, and Additional Risks Related to the Merger

There is no assurance that the proposed Merger will be completed in a timely manner or at all. If the proposed Merger is not consummated, our business could suffer materially and our stock price could decline.

The closing of the proposed Merger is subject to the satisfaction or waiver of a number of closing conditions, as described above, including the required approvals by GTx and Oncternal stockholders (including stockholder approval from one of Oncternal's significant stockholders, Shanghai Pharmaceutical (USA) Inc., which holds all of the outstanding shares of one series of Oncternal's preferred stock that must approve the transactions contemplated by the Merger Agreement) and other customary closing conditions. See the risk factors above titled, "*The proposed Merger is subject to approval of the Merger Agreement by our stockholders and the Oncternal stockholders. Failure to obtain these approvals would prevent the closing of the Merger*" and "*If the conditions to the Merger are not met, the Merger will not occur.*" If the conditions are not satisfied or waived, including as a result of Shanghai Pharmaceutical (USA) Inc. refusing to approve the transactions contemplated by the Merger Agreement, the proposed Merger may be materially delayed or abandoned. If the proposed Merger is not consummated, our ongoing business may be adversely affected and, without realizing any of the

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benefits of having consummated the proposed Merger, we will be subject to a number of risks, including the following:

- we have incurred and expect to continue to incur significant expenses related to the proposed Merger even if the Merger is not consummated;
- we could be obligated to pay Oncternal a termination fee of up to $2.0 million under certain circumstances set forth in the Merger Agreement;
- the market price of our common stock may decline to the extent that the current market price reflects a market assumption that the proposed Merger will be completed; and
- matters relating to the proposed Merger have required and will continue to require substantial commitments of time and resources by our remaining management and employees, which could otherwise have been devoted to other opportunities that may have been beneficial to us.

We also could be subject to litigation related to any failure to consummate the proposed Merger or to perform our obligations under the Merger Agreement. If the proposed Merger is not consummated, these risks may materialize and may adversely affect our business, financial condition and the market price of our common stock.

If the proposed Merger is not completed, we may be unsuccessful in completing an alternative transaction on terms that are as favorable as the terms of the proposed Merger with Oncternal, or at all, and we may otherwise be unable to continue to operate our business. Our board of directors may decide to pursue a dissolution and liquidation of GTx. In such an event, the amount of cash available for distribution to our stockholders will depend heavily on the timing of such liquidation as well as the amount of cash that will need to be reserved for commitments and contingent liabilities.

Our assets currently consist primarily of cash, cash equivalents and short-term investments, our SARD and SARM assets, the remaining value, if any, of our deferred tax assets, our listing on The Nasdaq Capital Market and the Merger Agreement with Oncternal. While we have entered into the Merger Agreement with Oncternal, the closing of the proposed Merger may be delayed or may not occur at all and there can be no assurance that the proposed Merger will deliver the anticipated benefits we expect or enhance stockholder value. If we are unable to consummate the proposed Merger, our board of directors may elect to pursue an alternative strategy, one of which may be a strategic transaction similar to the proposed Merger. Attempting to complete an alternative transaction like the proposed Merger will be costly and time consuming, and we can make no assurances that such an alternative transaction would occur at all. Alternatively, our board of directors may elect to continue our operations to advance appropriate SARD compounds into the additional preclinical studies required to submit an IND and potentially advance one of our SARD compounds into a first-in-human clinical trial, which would require that we obtain additional funding, and to resume our efforts to seek potential collaborative, partnering or other strategic arrangements for our SARM assets, including a sale or other divestiture of our SARM assets, or our board of directors could instead decide to pursue a dissolution and liquidation of our company. In such an event, the amount of cash available for distribution to our stockholders will depend heavily on the timing of such decision, as with the passage of time the amount of cash available for distribution will be reduced as we continue to fund our operations. In addition, if our board of directors were to approve and recommend, and our stockholders were to approve, a dissolution and liquidation of our company, we would be required under Delaware corporate law to pay our outstanding obligations, as well as to make reasonable provision for contingent and unknown obligations, prior to making any distributions in liquidation to our stockholders. Our commitments and contingent liabilities may include severance obligations,

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regulatory and preclinical obligations, and fees and expenses related to the proposed Merger. As a result of this requirement, a portion of our assets may need to be reserved pending the resolution of such obligations. In addition, we may be subject to litigation or other claims related to a dissolution and liquidation. If a dissolution and liquidation were pursued, our board of directors, in consultation with its advisors, would need to evaluate these matters and make a determination about a reasonable amount to reserve. Accordingly, holders of our common stock could lose all or a significant portion of their investment in the event of a liquidation, dissolution or winding up of the company.

The issuance of shares of our common stock to Oncternal stockholders in the proposed Merger will substantially dilute the voting power of our current stockholders.

If the proposed Merger is completed, each outstanding share of Oncternal common stock will be converted into the right to receive a number of shares of our common stock equal to the exchange ratio determined pursuant to the Merger Agreement. Immediately following the Merger, the former Oncternal stockholders immediately before the Merger are expected to own approximately 75% of our outstanding capital stock, and our stockholders immediately before the Merger are expected to own approximately 25% of our outstanding capital stock, subject to certain assumptions. Accordingly, the issuance of shares of our common stock to Oncternal stockholders in the Merger will reduce significantly the relative voting power of each share of GTx common stock held by our current stockholders. Consequently, our stockholders as a group will have significantly less influence over the management and policies of the combined company after the Merger than prior to the Merger. These estimates are based on the anticipated exchange ratio and are subject to adjustment as provided in the Merger Agreement. See also the risk factor above titled, "*The exchange ratio is not adjustable based on the market price of GTx common stock, so the merger consideration at the closing may have a greater or lesser value than at the time the Merger Agreement was signed.*"

GTx stockholders may not receive any payment on the CVRs and the CVRs may otherwise expire valueless.

If the proposed Merger is completed, we and certain other parties will enter into the CVR Agreement pursuant to which, for each share of GTx common stock held, GTx stockholders of record as of immediately prior to the effective time of the Merger will receive one CVR entitling such holders to receive in the aggregate 50% of any net proceeds received during the 15-year period after the closing of the Merger from the grant, sale or transfer of rights to our SARD or SARM technology that occurs during the 10-year period after the closing of the Merger (or in the 11th year if based on a term sheet approved during the initial 10-year period) and, if applicable, to receive royalties on the sale of any SARD products by the combined company during the 15-year period after the closing of the Merger. The CVRs will not be transferable, will not have any voting or dividend rights, and interest will not accrue on any amounts potentially payable on the CVRs. Accordingly, the right of any GTx stockholder to receive any future payment on or derive any value form the CVRs will be contingent solely upon the achievement of the foregoing events within the time periods specified in the CVR Agreement and if these events are not achieved for any reason within the time periods specified in the CVR Agreement, no payments will be made under the CVRs, and the CVRs will expire valueless. In addition, Oncternal (as successor in interest to GTx) has agreed only to use commercially reasonable efforts to develop SARD products and to divest our SARM technology, subject to certain limitations, which allows for the consideration of a variety of factors in determining the efforts that the combined company is required to use to develop SARD products and to divest our SARM technology, and it does not require the combined company to take all possible actions to continue efforts to develop SARD products and to divest our SARM technology. Accordingly, under certain circumstances the combined company may not be required to continue efforts to develop SARD products and to divest our SARM technology, or may allocate resources to other projects, which would have an adverse effect on the value, if any, of the CVRs. Furthermore, the CVRs will be unsecured obligations of the

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combined company and all payments under the CVRs, all other obligations under the CVR Agreement and the CVRs and any rights or claims relating thereto will be subordinated in right of payment to the prior payment in full of all current or future senior obligations of the combined company. Finally, the U.S. federal income tax treatment of the CVRs is unclear. There is no legal authority directly addressing the U.S. federal income tax treatment of the receipt of, and payments on, the CVRs, and there can be no assurance that the Internal Revenue Service, would not assert, or that a court would not sustain, a position that could result in adverse U.S. federal income tax consequences to holders of the CVRs.

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

As of December 31, 2018, we had an accumulated deficit of $600.1 million. Our net loss for the year ended December 31, 2018 was $38.4 million and we expect to incur significant operating losses for the foreseeable future depending on the extent of our preclinical and any clinical development activities and, if any such development activities are successful, potentially seeking regulatory approval of any potential future product candidates. These losses, among other things, have had and will continue to have an adverse effect on our stockholders' equity and working capital.

A substantial portion of our recent efforts and expenditures have been devoted to, and our prospects were substantially dependent upon, the development of enobosarm for the treatment of postmenopausal women with SUI. However, in September 2018, we announced that our placebo-controlled Phase 2 clinical trial of enobosarm to evaluate the change in frequency of daily SUI episodes following 12 weeks of treatment, or the ASTRID trial, failed to achieve statistical significance on the primary endpoint of the proportion of patients with a greater than 50% reduction in incontinence episodes per day compared to placebo. The failure of the ASTRID trial to achieve its primary endpoint has significantly depressed our stock price and has severely harmed our ability to raise additional capital and to secure potential collaborative, partnering or other strategic arrangements for our SARM assets, and consequently, our prospects to continue as a going concern have been severely diminished. Following our review of the full data sets from the ASTRID trial, we determined to discontinue further development of enobosarm to treat SUI and to otherwise discontinue any further development of our SARM program generally. We continue our efforts to seek potential collaborative, partnering or other strategic arrangements for our SARM assets, including a sale or other divestiture of our SARM assets. We have for many years actively pursued, but have been unable to successfully enter into, potential collaborative, partnering or other strategic arrangements for our SARM assets. If we are unable to ultimately enter into any such arrangements for our SARM assets, we will not receive any return on our investment in enobosarm and our other SARMs.

As a result of our decision to discontinue our SARM development efforts, our development activities are focused solely on completing ongoing mechanistic preclinical studies in order to select the most appropriate SARD compounds to move forward into the additional preclinical studies required to submit an IND and potentially advance one of our SARD compounds into a first-in-human clinical trial. However, while we believe that our existing capital resources will be adequate to enable us to conduct and complete planned IND-enabling preclinical studies of our SARD compounds, we will require significant additional financial resources in order to initiate and complete initial human clinical trials of a SARD compound and to otherwise further the development of our SARD program. Accordingly, if, for any reason, the proposed Merger is not consummated, we may resume our efforts to seek additional funds through potential collaborative, partnering or other strategic arrangements to provide us with the necessary resources for the development of our SARD program. In addition, our preclinical evaluation of our SARD technology is at very early stage and is subject to the substantial risk and probability of failure inherent in the development of early-stage programs.

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Because of the numerous risks and uncertainties associated with developing and commercializing small molecule drugs, we are unable to predict the extent of any future losses or when we will become profitable, if at all. We have 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 the proposed Merger is not completed and we are unable to raise sufficient additional funds for the development of our SARD program, whether through potential collaborative, partnering or other strategic arrangements or otherwise, or if we otherwise determine to discontinue the development of our SARD program, we will likely determine to cease operations. Even if we are able to raise additional funds to permit the continued development of our SARD program, if we and/or any potential collaborators are unable to develop and commercialize our SARDs or SARM technology, if development is further delayed or is eliminated, or if sales revenue from any SARD or partnered SARM products upon receiving marketing approval, if ever, is insufficient, we may never become profitable and we will not be successful.

If we do not successfully complete the proposed Merger, we will need to raise substantial additional capital and may be unable to raise the capital necessary to permit the continued development of our SARD program, which would force us to delay, reduce or eliminate our SARD program and would likely cause us to cease operations.

At December 31, 2018, we had cash, cash equivalents and short-term investments of $28.5 million. If the proposed Merger is not completed, based on our current business plan and spending assumptions as a standalone company, we estimate that our current cash, cash equivalents and short-term investments, together with interest thereon, will be sufficient to meet our projected operating requirements for at least the next 12 months. 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 sooner than currently anticipated.

While we believe that our existing capital resources will be adequate to enable us to conduct and complete planned IND-enabling preclinical studies of SARD compounds, we will require significant additional financial resources in order to initiate and complete initial human clinical trials of a SARD compound and to otherwise further the development of our SARD program. If we are unable to raise sufficient additional funds for the development of our SARD program, whether through potential collaborative, partnering or other strategic arrangements or otherwise, or if we otherwise determine to discontinue the development of our SARD program, we will likely determine to cease operations.

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

- our ability to successfully complete the Merger;
- the scope, rate of progress and cost of our preclinical and potential future clinical development programs;
- the terms and timing of any potential collaborative, partnering and other strategic arrangements that we may establish;

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- the amount and timing of any licensing fees, milestone payments and royalty payments from potential collaborators, if any;
- potential future clinical trial results;
- the cost and timing of regulatory filings and/or approvals to commercialize any potential future 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 have no ongoing collaborations for the development and commercialization of any product candidates and no source of revenue, nor do we expect to generate product revenue for the foreseeable future. We do not have any commitments for future external funding. In addition, although we have entered into an At-the-Market Equity Offering[SM] Sales Agreement with Stifel, Nicolaus & Company, Incorporated, or the ATM Sales Agreement, under which approximately $25.0 million of shares of our common stock remained available for sale at December 31, 2018, it is unlikely we could raise sufficient funds under the ATM Sales Agreement to permit us to initiate and complete initial human clinical trials of a SARD compound, and given our currently-depressed stock price, the ATM Sales Agreement is not otherwise expected to be a practical source of liquidity for us at this time. Further, given our currently-depressed stock price, we are significantly limited in our ability to sell shares of common stock under the ATM Sales Agreement since the issuance and sale of our common stock under the ATM Sales Agreement, if it occurs, would be effected under a registration statement on Form S-3 that we filed with the Securities and Exchange Commission, and in accordance with the rules governing those registration statements, we generally can only sell shares of our common stock under that registration statement in an amount not to exceed one-third of our public float, which limitation for all practical purposes precludes our ability to obtain any meaningful funding through the ATM Sales Agreement at this time.

Until we can generate a sufficient amount of product revenue, which we may never do, we will need to finance future cash needs through potential collaborative, partnering or other strategic arrangements, as well as through public or private equity offerings or debt financings or a combination of the foregoing. If we are unable to raise additional funds, we will need to continue to reduce our expenditures in order to preserve our cash. Further cost-cutting measures that we may take may not be sufficient to enable us to meet our cash requirements, and they may negatively affect our business and our ability to derive any value from our SARD program. In any event, in order to further the development of our SARD program, we will need to raise substantial additional capital. Our failure to do so would likely result in our determining to cease operations.

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 and intellectual property rights thereof, or grant licenses on terms that are not favorable to us, any of which could result in our stockholders having little or no continuing interest in our SARD program and/or SARM assets 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 substantially dilutive private placements of our common stock and warrants in March 2014, November 2014 and September 2017, in addition to a registered direct offering of our

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common stock that we completed in October 2016 and the sale of our common stock pursuant to the ATM Sales Agreement. Our stockholders will 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 have been severely harmed by the failure of the ASTRID trial to meet its primary endpoint and the resulting significant uncertainty regarding our prospects to continue as a going concern. If we are unable to complete the proposed Merger, 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, uncertainties with respect to the prospects for our early-stage SARD program, the sufficiency of our capital resources, potential future management turnover, and volatility and instability in the global financial markets. As a result of these and other factors, there is no guarantee that sufficient additional funding will be available to us on acceptable terms, or at all.

We are substantially dependent on our remaining employees to facilitate the consummation of the proposed Merger.

We have substantially reduced our workforce since November 2018 and as of March 12, 2018, we had only 13 full-time employees. Our ability to successfully complete the proposed Merger depends in large part on our ability to retain our remaining personnel. Despite our efforts to retain these employees, one or more may terminate their employment with us on short notice. The loss of the services of any of these employees could potentially harm our ability to consummate the proposed Merger, to run our day-to-day business operations, as well as to fulfill our reporting obligations as a public company.

The pendency of the proposed Merger could have an adverse effect on the trading price of our common stock and our business, financial condition and prospects.

While there have been no significant adverse effects to date, the pendency of the proposed Merger could disrupt our business in many ways, including:

- the attention of our remaining management and employees may be directed toward the completion of the proposed Merger and related matters and may be diverted from our day-to-day business operations; and
- third parties may seek to terminate or renegotiate their relationships with us as a result of the proposed Merger, whether pursuant to the terms of their existing agreements with us otherwise.

Should they occur, any of these matters could adversely affect the trading price of our common stock or harm our business, financial condition and prospects.

Risks Related to Our Development Activities

We were substantially dependent on the success of enobosarm, and the recent failure of the ASTRID trial to meet its primary endpoint has severely diminished enobosarm's prospects and our prospects to continue as a going concern. As we are now focused solely on our SARD program, our failure to obtain funding for and to advance the development of our SARD program would likely require us to cease operations.

A substantial portion of our recent efforts and expenditures have been devoted to, and our prospects were substantially dependent upon, the development of enobosarm for the treatment of postmenopausal women with SUI. However, in September 2018, we announced that the ASTRID trial failed to achieve statistical significance on the primary endpoint of the proportion of patients with a

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greater than 50% reduction in incontinence episodes per day compared to placebo. The failure of the ASTRID trial to achieve its primary endpoint has significantly depressed our stock price and has severely harmed our ability to raise additional capital and to secure potential collaborative, partnering or other strategic arrangements for our SARM assets, and consequently, our prospects to continue as a going concern have been severely diminished. Following our review of the full data sets from the ASTRID trial, we determined to discontinue further development of enobosarm to treat SUI and to otherwise discontinue any further development of our SARM program generally. We continue our efforts to seek potential collaborative, partnering or other strategic arrangements for our SARM assets, including a sale or other divestiture of our SARM assets. We have for many years actively pursued, but have been unable to successfully enter into, potential collaborative, partnering or other strategic arrangements for our SARM assets. If we are unable to ultimately enter into any such arrangements for our SARM assets, we will not receive any return on our investment in enobosarm and our other SARMs.

As a result of our decision to discontinue our SARM development efforts, our development activities are focused solely on completing ongoing mechanistic preclinical studies in order to select the most appropriate SARD compounds to move forward into the additional preclinical studies required to submit an IND and potentially advance one of our SARD compounds into a first-in-human clinical trial. However, while we believe that our existing capital resources will be adequate to enable us to conduct and complete planned IND-enabling preclinical studies of our SARD compounds, we will require significant additional financial resources in order to initiate and complete initial human clinical trials of a SARD compound and to otherwise further the development of our SARD program. In addition, our preclinical evaluation of our SARD technology is at very early stage and is subject to the substantial risk and probability of failure inherent in the development of early-stage programs.

In any event, if the proposed Merger is not completed and we are unable to raise sufficient additional funds for the development of our SARD program, whether through potential collaborative, partnering or other strategic arrangements or otherwise, or if we otherwise determine to discontinue the development of our SARD program, we will likely determine to cease operations.

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

Significant additional clinical development, financial resources and personnel would be required to obtain necessary regulatory approvals for any potential future 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 top-line or 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 top-line, interim or other preliminary data from our clinical trials, which data is based on a preliminary analysis of then-available efficacy and safety data, and the results and related findings and conclusions are subject to change following a more comprehensive review of the data related to the particular study or trial. We also make assumptions, estimations, calculations and conclusions as part of our analyses of data, and we may not have received or had the opportunity to fully and carefully evaluate all data from the applicable trial. As a result, the top-line results that we report may differ from future results of the same studies, or different conclusions or considerations may qualify such results, once additional data have been received and fully evaluated. Similarly, interim or other 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. Top-line, interim and other

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preliminary data also remain subject to audit and verification procedures that may result in the final data being materially different from such top-line, interim or other preliminary data we previously published. As a result, top-line, 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, in September 2018, we announced that the ASTRID trial failed to achieve statistical significance on the primary endpoint of the proportion of patients with a greater than 50% reduction in incontinence episodes per day compared to placebo. The failure of the ASTRID trial to achieve its primary endpoint has significantly depressed our stock price and has severely harmed our ability to raise additional capital and to secure potential collaborative, partnering or other strategic arrangements for our SARM assets, and consequently, our prospects to continue as a going concern have been severely diminished. Likewise, 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. Additionally, in the third quarter of 2017, we decided not to pursue additional clinical development of enobosarm to treat women with ER positive, AR positive advanced breast cancer after evaluating the breast cancer environment where the treatment paradigms are shifting to immunotherapies and/or combination therapies, along with the time and cost of conducting the necessary clinical trials for potential approval, even though we announced that our Phase 2 clinical trial of enobosarm in this indication achieved its primary endpoint in both the 9 mg and 18 mg cohorts of the clinical trial. Following our review of the full data sets from the ASTRID trial, we determined to discontinue further development of enobosarm to treat SUI and to otherwise discontinue any further development of our SARM program generally. We continue our efforts to seek potential collaborative, partnering or other strategic arrangements for our SARM assets, including a sale or other divestiture of our SARM assets. We have for many years actively pursued, but have been unable to successfully enter into, potential collaborative, partnering or other strategic arrangements for our SARM assets. If we are unable to ultimately enter into any such arrangements for our SARM assets, we will not receive any return on our investment in enobosarm and our other SARMs.

In the first quarter of 2015, we entered into an exclusive worldwide license agreement with UTRF to develop its proprietary SARD technology and we are currently focused solely on the further development of our SARD program. Our preclinical evaluation of our SARD technology is at an early stage and is subject to the substantial risk and probability of failure inherent in the development of early-stage programs. While we believe that our existing capital resources will be adequate to enable us to conduct and complete planned IND-enabling preclinical studies of our SARD compounds, we will require significant additional financial resources in order to initiate and complete initial human clinical trials of a SARD compound and to otherwise further the development of our SARD program. If our research and preclinical development of our SARD program is unsuccessful, is discontinued and/or we are not able to obtain sufficient funding to advance the development of our SARD program, we will likely cease operations.

Significant delays in preclinical and clinical testing could materially impact our product development costs. We do not know whether our planned preclinical and potential future 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

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and the clinical trial process that could delay or prevent our or our potential collaborators' ability to commercialize any 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 or any potential collaborators may experience substantial delays in obtaining these authorizations;
- we or any potential collaborators may be delayed in reaching, or may fail to reach, agreement on acceptable terms with prospective clinical research organizations, or CROs, and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;
- 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 preclinical development or clinical trials;
- patient 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;
- our product candidates may not have the desired effects or may include undesirable side effects; and
- changes in regulatory requirements, policies and guidelines.

If any of these events were to occur in the future and, as a result, we or any potential collaborators have significant delays in or termination of potential future 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 any potential future product candidates are in development or after our products are approved and on the market, we or any potential collaborators may be required to perform lengthy additional clinical trials, may be required to cease further development of such product candidates, may be denied regulatory approval of such products, may be forced to change the labeling of such products or may be required to withdraw any such products from the market, any of which would hinder or preclude our ability to generate revenues.

In 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

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adverse cardiovascular events. Mild transient elevations in liver enzymes that were within normal limits were observed in our Phase 2 proof-of-concept clinical trial of enobosarm to treat postmenopausal women with SUI, except for one patient with levels greater than 1.5 times the upper limit of normal which returned to normal following her 12-week treatment period. Reductions in total cholesterol, low-density lipoproteins, or LDL, HDL and triglycerides were also observed. Results of the placebo-controlled ASTRID study in postmenopausal women with SUI indicated that enobosarm was generally safe and well tolerated, and reported adverse events were generally mild to moderate in intensity and similar across all treatment groups. Mild transient elevations in hepatic enzymes and changes in lipid profile were dose dependent, and consistent with results seen in previous trials. 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, the 3 mg dose that was the subject of the ASTRID trial and higher enobosarm doses that may potentially be tested by potential future collaborators in later stage longer duration trials, if any, 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.

If the incidence of serious or other adverse events related to enobosarm or any other SARD or SARM 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 potential future 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 adoption and 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 the proposed Merger is not completed and we do not establish collaborative, partnering or other strategic arrangements for our SARD program and SARM assets or otherwise raise substantial additional capital, we will likely determine to cease operations.

Our current strategy is dependent on our ability to secure potential collaborative, partnering or other strategic arrangements with other pharmaceutical and biotechnology companies to assist us in furthering development and potential commercialization of any SARD and SARM product candidates,

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and to otherwise obtain funding for such activities. For example, we are currently focused solely on the further development of our SARD program and while we believe that our existing capital resources will be adequate to enable us to conduct and complete planned IND-enabling preclinical studies of SARD compounds, we will require significant additional financial resources in order to initiate and complete initial human clinical trials and to otherwise further the development of our SARD program. Accordingly, if, for any reason, the proposed Merger is not consummated, we may resume our efforts to seek additional funds through potential collaborative, partnering or other strategic arrangements to provide us with the necessary resources for the development of our SARD program. We face significant competition in seeking such arrangements, and such arrangements are complex and time consuming to negotiate and document. In any event, we may not be successful in entering into new collaborative, partnering or other strategic arrangements with third parties for the further development of our SARD program (or our SARD assets) on acceptable terms, or at all. In this regard, we have for many years actively pursued, but have been unable to successfully enter into, potential collaborative, partnering or other strategic arrangements for our SARM assets and we likewise have not been successful to date in entering into potential collaborative, partnering or other strategic arrangements for our SARD program. In addition, we are unable to predict when, if ever, we will enter into any potential collaborative, partnering or other such strategic arrangements because of the numerous risks and uncertainties associated with establishing such arrangements, and we have otherwise been unsuccessful, for many years, in our efforts to establish such arrangements. In any event, if the proposed Merger is not completed and we are unable to raise sufficient additional funds for the development of our SARD program, whether through potential collaborative, partnering or other strategic arrangements or otherwise, or if we otherwise determine to discontinue the development of our SARD program, we will likely determine to cease operations. In addition, because we have discontinued our SARM development efforts, if we are unable to ultimately enter into any potential collaborative, partnering or other such strategic arrangements for our SARM assets, we will not receive any return on our investment in enobosarm and our other SARMs.

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, if the proposed Merger is not completed, we intend to continue to seek to establish, partnering, collaborative and similar strategic arrangements with third parties to develop and commercialize any potential 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 any product candidate. We may not be able to locate third-party collaborators to develop and market any product candidates, and we lack the necessary financial resources to develop any 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;

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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 clinical and commercial drug supplies in sufficient quantities, in the required timeframe, at an acceptable cost, and with appropriate quality control, clinical development and commercialization of any potential future 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 any product candidates. Our current and anticipated future dependence upon others for the manufacture of our product candidates may adversely affect our future profit margins, if any, and our ability to develop product candidates and commercialize any product candidates on a timely and competitive basis.

We rely and expect to continue to rely on third-party vendors for drug substance and drug product manufacturing, including drug substance for SARDs used in our current and potential future preclinical studies. If the contract manufacturers that we are currently utilizing to meet our supply needs for SARD compounds or any potential future SARD product candidates prove incapable or unwilling to continue to meet our supply needs, we could experience a delay in conducting any additional preclinical or clinical trials of SARD compounds or any potential future 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 our 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 any potential future product candidates.

Use of third-party manufacturers may increase the risk that we will not have adequate drug supplies for preclinical, clinical and commercial use.

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.

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If we are not able to obtain adequate drug supplies, including SARD compounds, it will be more difficult for us to develop any product candidates and compete effectively. Our potential future 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 any potential future 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 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 any potential future 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 enobosarm and other SARM compounds, and to SARD compounds and, for both, to methods of use thereof, 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 enobosarm and other SARM compounds and/or SARD compounds and the intellectual property covered by those agreements to market, distribute and sell licensed products, which may prevent us from continuing our business and would likely 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, if any, will depend in part on obtaining and maintaining patent and trade secret protection for any product candidates that we may develop, as well as the methods for treating

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patients in the product indications using these product candidates. We will be able to protect any potential future 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 any potential future product candidates and/or 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

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

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

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

Risks Related to Regulatory Approval

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.

The activities associated with the development and commercialization of drug candidates are subject to comprehensive regulation by the FDA, other regulatory agencies in the United States and by comparable authorities in other countries, including the European Medicines Agency, or 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 product candidate in any jurisdiction, and we do not expect to obtain FDA, EMA or any other regulatory approvals to market any potential future 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 Strategy, or REMS, on a product if the FDA believes there is a reason to monitor the safety of the

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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. Failure to obtain approval in one jurisdiction may negatively impact our ability to obtain approval elsewhere.

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, including Phase 4 clinical studies. For example, in October 2009, we received a Complete Response Letter from the FDA regarding our new drug application, or NDA, for toremifene 80 mg to reduce fractures in men with prostate cancer on androgen deprivation therapy 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 marketing authorization application, or MAA, to the EMA seeking marketing approval of enobosarm 3 mg in the European Union, or EU, for the prevention and treatment of muscle wasting in patients with advanced NSCLC, based on input from the Medicines and Healthcare Products Regulatory Agency, or MHRA, we determined that the data from the POWER trials was 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, we were unable to file with the FDA a NDA for enobosarm 3 mg for the prevention and treatment of muscle wasting in patients with advanced NSCLC.

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 product candidates for the foreseeable future, if at all. The inability to obtain approval from the FDA, the EMA and other foreign regulatory authorities for our product candidates would prevent us or any potential collaborators from commercializing these product candidates in the United States, the EU, or other countries. See the section entitled "Business Government Regulation" under Part 1, Item 1 of this Annual Report on Form 10-K for additional information regarding risks associated with marketing approval, as well as risks related to potential post-approval requirements.

Risks Related to Commercialization

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

Any products that we and/or any potential collaborators may develop may not gain market acceptance for its stated indication among physicians, patients, health care payors and the medical

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community despite regulatory approval. 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 become and/or remain a preferred course of treatment;
- the ability to offer our product candidates for sale at competitive prices;
- relative convenience and ease of administration compared to alternative treatment;
- 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 potential future 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 any such product candidates. Either of these options would be expensive and time-consuming. We may be unable to build our own sales and marketing capabilities, and there are risks involved with entering into arrangements with third parties to perform these services, which could delay the commercialization of any of our product candidates if approved for commercial sale. In addition, to the extent that we enter into arrangements with third parties to perform sales, marketing and distribution services, our product revenues are likely to be lower than if we market and sell any products that we develop ourselves.

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

Sales of products developed by us and/or any potential collaborators are dependent on the availability and extent of reimbursement from third-party payors, both governmental and private. Changes in the coverage and/or 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 Healthcare Reform Act, which, among other initiatives, implemented cost containment and other measures that could adversely affect revenues from sales of product candidates, including an increase in the drug rebates that manufacturers must pay under Medicaid for brand name

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prescription drugs and extension of these rebates to Medicaid managed care and a requirement that manufacturers provide a 50% discount on the negotiated price of Medicare Part D brand name drugs utilized by Medicare Part D beneficiaries during the coverage gap (the so-called "donut hole")(which discount has subsequently been increased to 70% in 2019).

The provisions of the Healthcare Reform Act have been subject to judicial and Congressional challenges, as well as efforts by the Trump administration to modify certain requirements of the Healthcare Reform Act by executive branch order. For example, 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. On October 12, 2017, President Trump signed another Executive Order directing certain federal agencies to propose regulations or guidelines to provide small businesses with greater opportunities to form association health plans, expand the availability of short-term, limited duration insurance, and allow employees to make use of certain employer-paid health benefits, called health reimbursement arrangements, to pay for health insurance that does not meet all Healthcare Reform Act requirements. In addition, citing legal guidance from the U.S. Department of Justice, the U.S. Department of Health and Human Services, or HHS, concluded that cost-sharing reduction, or CSR, payments to insurance companies required under the Healthcare Reform Act had not received necessary appropriations from Congress. President Trump subsequently discontinued these payments. The loss of the CSR payments is expected to increase premiums on certain policies issued by qualified health plans under the Healthcare Reform Act. Certain administrative actions have been subject to judicial challenge. In Congress, there have been a number of legislative initiatives to modify, repeal and/or replace portions of the Healthcare Reform Act. Tax reform legislation enacted at the end of 2017 eliminated the tax penalty for individuals who do not maintain sufficient health insurance coverage beginning in 2019. The Bipartisan Budget Act of 2018 contained various provisions that affect coverage and reimbursement of drugs, including an increase in the discount that manufacturers of Medicare Part D brand name drugs must provide to Medicare Part D beneficiaries during the coverage gap from 50% to 70% starting in 2019. Congress may consider other legislation to modify, repeal and/or replace certain elements of the Healthcare Reform Act. In December 2018, a federal district court judge, in a challenge brought by a number of state attorneys general, found the Healthcare Reform Act unconstitutional in its entirety because, once Congress repealed the individual mandate provision, there was no longer a basis to rely on Congressional taxing authority to support enactment of the law. Pending appeals, which could take some time, the Healthcare Reform Act is still operational in all respects. We continue to evaluate the effect that the Healthcare Reform Act and its possible repeal, replacement or modification may have on our business. Such 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.

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

Another development that could affect the pricing of drugs would be if the Secretary of HHS 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, replace and/or modify the Healthcare Reform Act in the United States. Federal and state legislatures within the United States and foreign governments will likely continue to consider other 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 administrative, legislative and enforcement interest in the United States with respect to drug pricing practices. There have been several 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

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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 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 any potential future 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.

We believe SARDs have the potential to provide compounds that can degrade or antagonize multiple forms of the AR thereby inhibiting tumor growth in patients with CRPC, including those patients who do not respond or are resistant to current therapies. Drugs in development having potentially similar approaches to removing the AR by degradation include Arvinas Inc.'s ARV-110, which is a chimera with an AR binding moiety on one end and an E3 ligase recruiting element on the other that has recently entered Phase 1 development for the treatment of advanced prostate cancer, and Androscience Corporation's androgen receptor degrader enhancer, ASC-J9, which is currently in development for acne and alopecia with the potential for development as a treatment for prostate

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cancer. Additionally, Essa Pharma Inc. recently completed a Phase 1 study with EPI-506, an AR antagonist that targets the N-terminal domain of the AR, and has plans to develop a second generation agent. 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 recently initiated a clinical trial with CCS1477 in prostate cancer. Oric Pharmaceuticals is targeting the glucocorticoid receptor as a means to impact men that have CRPC, and has a lead candidate ORIC-101 in preclinical testing. 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. XTANDI® received FDA approval in July 2018 for the treatment of men with non-metastatic CRPC. Zytiga®, sold by Johnson & Johnson, has been approved for the treatment of metastatic CRPC and metastatic high-risk castration-sensitive prostate cancer. Johnson & Johnson also received FDA approval for a second generation anti-androgen ERLEADA (apalutamide) for the treatment of men with non-metastatic castrate-resistant prostate cancer. Bayer HealthCare and Orion Corporation recently announced that the primary endpoint of increased metastatic free survival was met in a Phase 3 study of darolutamide (ODM-201) in men with CRPC without metastases and with a rising PSA. Another target in prostate cancer that is being pursued by several companies is bromodomain inhibition. Zenith Epigenetics, Gilead Sciences Inc., CellCentric, Incyte Corporation and GlaxoSmithKline are among the companies that are evaluating BET inhibitors in Phase 1-2 trials.

With respect to SARMs, there are other SARM product candidates in development that may compete with enobosarm and any future SARM product candidates, if approved for commercial sale. For example, Viking Therapeutic's VK5211 recently reported positive results from a Phase 2 study for patients recovering from non-elective hip fracture surgery. Radius Health Inc.'s RAD140 is currently being evaluated in a Phase 1 study in postmenopausal women with hormone-receptor positive locally advanced or metastatic breast cancer. GlaxoSmithKline is conducting a Phase 1 study to assess the effect of GSK2881078 on physical strength and function after 13 weeks of treatment in patients with chronic obstructive pulmonary disease, or COPD, and muscle weakness. OPKO Health's OPK88004 is enrolling in a dose ranging study to improve symptoms of benign prostatic hyperplasia (BPH) by reducing prostate size and, on the basis of data from a previous trial in 350 men, increase muscle mass and bone strength and decrease body fat.

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.

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Risks Related to Employees, Growth and Other Aspects of Our 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 and could result in significant financial, legal, regulatory, business and reputational harm to us.

Despite the implementation of security measures, our internal computer and information technology 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 potential future clinical trials involving our product candidates, if any, could result in delays in our development and regulatory filing efforts and significantly increase our costs. In addition, while all information technology operations are inherently vulnerable to inadvertent or intentional security breaches, incidents, attacks and exposures, the size, complexity, accessibility and distributed nature of our information technology systems, and the large amounts of sensitive information stored on those systems, make such systems potentially vulnerable to unintentional or malicious, internal and external attacks on our technology environment. Potential vulnerabilities can be exploited from inadvertent or intentional actions of our employees, third-party vendors, business partners, or by malicious third parties. Attacks of this nature are increasing in their frequency, levels of persistence, sophistication and intensity, and are being conducted by sophisticated and organized groups and individuals with a wide range of motives (including, but not limited to, industrial espionage) and expertise, including organized criminal groups, "hacktivists," nation states and others. To the extent that any disruption or security breach or incident were to result in a loss of, or damage to, our data, or inappropriate disclosure of confidential, proprietary or protected health information, we could be subject to significant legal, financial and regulatory exposure and suffer reputational harm, and the development of our product candidates could be delayed. In addition, security breaches and other inappropriate access events can be difficult to detect, and any delay in identifying them may lead to increased harm of the type described above. Moreover, the prevalent use of mobile devices to access confidential information increases the risk of security breaches. While we have implemented security measures to protect our information technology systems and infrastructure, there can be no assurance that such measures will prevent service interruptions or security breaches that could adversely affect our business. 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. In addition, failure to maintain effective internal accounting controls related to security breaches and cybersecurity in general could impact our ability to produce timely and accurate financial statements and subject us to regulatory scrutiny.

If we fail to attract and keep senior management and key scientific personnel, we may be unable to continue our business operations.

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. Significant competition exists for qualified personnel in the biotechnology field, particularly clinical development personnel. We may incur greater costs than anticipated, or may not be successful, in attracting new scientists or management or in retaining or motivating our existing personnel. If we are not able to attract and keep senior management and key scientific personnel, our ability to progress the development of any product candidates and any future growth could be impaired, and our business and the value of your investment

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would be adversely impacted. All of our employees are at-will employees and can terminate their employment at any time.

To conserve our cash resources, we have substantially reduced our workforce since November 2018 and have ceased our SARM development activities and all other operations except for day-to-day business operations, completing ongoing mechanistic SARD preclinical studies and those activities necessary to complete the proposed Merger. As of March 12, 2018, we had only 13 full-time employees. Accordingly, we have been and are continuing operating with a shortage of resources and may not be able to effectively conduct our operations with this limited number of employees. In addition, our ability to successfully complete the proposed Merger depends in large part on our ability to retain our remaining personnel. Despite our efforts to retain these employees, one or more may terminate their employment with us on short notice. The loss of the services of any of these employees could potentially harm our ability to consummate the Merger, to run our day-to-day business operations, as well as to fulfill our reporting obligations as a public company.

If the proposed Merger is not completed and we are able to raise sufficient additional funds necessary to pursue the continued development of our SARD program, we will need to hire a substantial number of additional employees. Any inability to manage future growth could harm our ability to develop and commercialize any potential future product candidates, increase our costs and adversely impact our ability to compete effectively.

As of March 12, 2018, we had only 13 full-time employees. If the proposed Merger is not completed and we are able to raise sufficient additional funds necessary to pursue the continued development of our SARD program, we will need to hire experienced personnel to continue to develop our SARD program and to develop and commercialize any potential future product candidates, and we will need to expand the number of our managerial, operational, financial and other employees to support that growth. Significant competition exists for qualified personnel in the biotechnology field, particularly clinical development personnel.

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

Management transition creates uncertainties and could harm our business.

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

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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 market price for our common stock has varied between a high of $25.60 on September 13, 2018, and a low of $0.74 on December 24, 2018, in the twelve-month period ended December 31, 2018. 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:

our ability to consummate the transactions contemplated by the Merger Agreement, including the proposed Merger;

our ability to execute on our SARD development program, including our ability to conduct and complete IND-enabling studies and potentially advance one of our SARD compounds into a first-in-human clinical trial;

our ability to raise sufficient additional funds necessary for the continued development of our SARD program, whether through potential collaborative, partnering or other strategic arrangements or otherwise;

our ability to realize any value from our SARM assets, particularly in light of our decision to discontinue the development of enobosarm and our SARM program generally;

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

uncertainties created by our potential future management turnover;

our inability to comply with the minimum listing requirements of The Nasdaq Stock Market LLC;

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;

reports of unacceptable incidences of adverse events observed in any future clinical trials of any product candidates that we and/or any potential collaborators may develop;

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

actions taken by regulatory agencies with respect to any potential future product candidates or our potential future clinical trials, if any, including regulatory actions requiring or leading to a delay or stoppage of any clinical trials;

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;

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

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;

if our patents covering our products candidates 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;

competition from third parties with products in the same class of products as any potential future product candidates or products with the same active pharmaceutical ingredients as those product candidates;

any intellectual property infringement lawsuit involving us;

actual or anticipated fluctuations in our results of operations;

changes in financial estimates or recommendations by securities analysts;

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

sales of our common stock and other securities by us;

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 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 changes in tariffs and other restrictions on free trade stemming from the Trump Administration and foreign government 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

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management's attention and resources, which could result in delays of our clinical trials or commercialization efforts.

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 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 5, 2016, we effected one-for-ten reverse stock split of our outstanding common stock, or the Reverse Stock Split, the 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, the closing bid price of our common stock has recently been well below $1.00 per share, and there can be no assurance that we will 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.

In addition, we are required pursuant to the terms of the Merger Agreement to submit to our stockholders a proposal to approve an amendment to our restated certification of incorporate to authorize our board of directors to effect a reverse stock split of all outstanding shares of our common stock. The approval of the reverse stock split by the stockholders is a condition to closing, pursuant to the Merger Agreement. If this reverse stock split proposal is not approved by our stockholders, and if the parties waive this closing condition, the combined company resulting from the proposed Merger will likely not be able to obtain compliance with the minimum bid price requirement for an initial listing on The Nasdaq Capital Market and, as a consequence, Nasdaq will immediately provide the combined company with written notification that our common stock will be delisted.

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

a limited availability of market quotations for our common stock;

a reduced amount of news and analyst coverage for us;

a decreased ability to issue additional securities and a concomitant substantial impairment in our ability to obtain sufficient additional capital to fund our operations and to continue as a going concern;

reduced liquidity for our stockholders;

potential loss of confidence by employees and potential future partners or collaborators; and

loss of institutional investor interest and fewer business development opportunities.

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

Based solely on the most recent Schedules 13G and 13D filed with the SEC and reports filed with the SEC under Section 16 of the Exchange Act, our executive officers, directors and holders of 5% or more of our outstanding common stock, including their affiliated or associated entities, held approximately 53.5% of our outstanding common stock, and our executive officers and directors alone, including their affiliated or associated entities, held approximately 30.0% of our outstanding common stock as well as warrants to purchase up to an additional 3.2 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, the approval of the issuance of shares of our common stock pursuant to the Merger Agreement, and the approval of potential alternative mergers or other business combination transactions. The interests of this group of stockholders may not always coincide with our interests or the interests of other stockholders.

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

We have a significant amount of federal and state net operating loss carryforwards. In this regard, as of December 31, 2018, we had net federal operating loss carryforwards of approximately $472.1 million. The federal operating loss carryforwards originating prior to 2018 will expire from 2019 to 2037 if not utilized, and state operating loss carryforwards of approximately $411.4 million, which expire from 2019 to 2038 if not utilized. Our ability to use our federal and state net operating loss carryforwards to offset potential future taxable income and related income taxes that would otherwise be due is dependent upon our generation of future taxable income before the expiration dates of the net operating loss carryforwards, and we cannot predict with certainty when, or whether, we will generate sufficient taxable income to use all of our net operating loss carryforwards. On December 22, 2017, President Trump signed into law new tax legislation, or the Tax Reform Act. Under the Tax Reform Act, federal net operating losses incurred in 2018 and in future years may be carried forward indefinitely, but the deductibility of such federal net operating losses is limited. It is uncertain if and to what extent various states will conform to the Tax Reform Act. In addition, 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, 2016 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, 2018, though we have not yet conducted an in-depth analysis since the last study. 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 2016 and 2017 equity offerings, our past and potential future issuances of common stock pursuant to the ATM Sales Agreement, other 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. In this regard, the proposed Merger, if consummated, will constitute an ownership change (within the meaning Section 382 of the Internal Revenue Code) which would eliminate or otherwise substantially limit our federal and state net operating loss carryforwards.

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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.

Our amended and restated bylaws provide that the Court of Chancery of the State of Delaware will be the exclusive forum for substantially all disputes between us and our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers or employees.

Our amended and restated bylaws provide that the Court of Chancery of the State of Delaware is the exclusive forum for any derivative action or proceeding brought on behalf of GTx, for any action asserting a claim of breach of a fiduciary duty owed by any current or former director, officer, other employee or stockholder of GTx to GTx or to our stockholders, for any action asserting a claim arising pursuant to any provision of the General Corporation Law of the State of Delaware, or the DGCL, our restated certificate of incorporation or our amended and restated bylaws or as to which the DGCL confers jurisdiction on the Court of Chancery of the State of Delaware, or for any action asserting a claim governed by the internal affairs doctrine. The choice of forum provision may limit a stockholder's ability to bring a claim in a judicial forum that it finds favorable for disputes with us or our directors, officers or other employees, which may discourage such lawsuits against us and our directors, officers and other employees. If a court were to find the choice of forum provision contained in our amended and restated bylaws to be inapplicable or unenforceable in an action, we may incur additional costs associated with resolving such action in other jurisdictions, which could harm our financial condition.

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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 December 31, 2018, the average daily trading volume of our common stock on The Nasdaq Capital Market was only 705,027 shares. As a result, future sales of a substantial number of shares of our common stock in the public market, or the perception that such sales may occur, could adversely affect the then-prevailing market price of our common stock. As of December 31, 2018, we had 24,051,844 shares of common stock outstanding. In addition, as a result of the low trading volume of our common stock, which was exacerbated by the one-for-ten reverse stock split of our outstanding common stock effected on December 5, 2016, or 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 addition, due to the limitations of our market, the volatility in the market price of our common stock and our currently-depressed stock price, stockholders may face difficulties in selling shares at attractive prices when they want to sell.

In September 2017, we completed a private placement of 5.5 million shares of our common stock and warrants to purchase 3.3 million shares of our common stock. 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 the registration rights or securities purchase agreements we entered into in connection with these private placements, we have filed registration statements under the Securities Act registering the resale of an aggregate of approximately 23.8 million shares of common stock that we issued to, or are issuable upon the exercise of warrants that we issued to, the investors in these private placements, which investors include our largest stockholders. 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 785,000 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 private placements, were to sell large blocks of shares in a short period of time, the market price of our common stock could drop substantially.

The comprehensive U.S. tax reform bill passed in 2017 could adversely affect our business and financial condition.

On December 22, 2017, President Trump signed the Tax Reform Act into law, which significantly revises the Internal Revenue Code of 1986, as amended. The Tax Reform Act, among other things, contains significant changes to corporate taxation, including reduction of the corporate tax rate from a top marginal rate of 35% to a flat rate of 21%, limitation of the tax deduction for interest expense to 30% of adjusted earnings (except for certain small businesses), limitation of the deduction for net operating losses to 80% of current year taxable income and elimination of net operating loss carrybacks, one time taxation of offshore earnings at reduced rates regardless of whether they are repatriated, immediate deductions for certain new investments instead of deductions for depreciation expense over time, and modifying or repealing many business deductions and credits (including reducing the business tax credit for certain clinical testing expenses incurred in the testing of certain drugs for rare diseases or conditions). Notwithstanding the reduction in the corporate income tax rate, the overall impact of the new Tax Reform Act is uncertain and our business and financial condition

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could be adversely affected. In addition, it is uncertain if and to what extent various states will conform to the Tax Reform Act. The impact of the Tax Reform Act on holders of our common stock is also uncertain and could be adverse. We urge our stockholders to consult with their legal and tax advisors with respect to this legislation and the potential tax consequences of investing in or holding our common stock.

ITEM 1B. UNRESOLVED STAFF COMMENTS

None.

ITEM 2. PROPERTIES

We sublease approximately 26,000 square feet of office space located at 175 Toyota Plaza, Memphis, Tennessee, under an operating lease which expires on April 30, 2019. We believe that our facilities are currently adequate to meet our needs.

ITEM 3. LEGAL PROCEEDINGS

We are not currently involved in any material legal proceedings.

ITEM 4. MINE SAFETY DISCLOSURES

Not applicable.

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PART II

ITEM 5. MARKET FOR REGISTRANT'S COMMON EQUITY, RELATED STOCKHOLDER MATTERS AND ISSUER PURCHASES OF EQUITY SECURITIES

Market for Registrant's Common Equity

Our common stock trades on The Nasdaq Capital Market under the trading symbol "GTXI." On March 12, 2019, the closing price of our common stock as reported on The Nasdaq Capital Market was $1.42 per share and there were approximately 69 holders of record of our common stock.

Dividend Policy

We have never declared or paid any cash dividends on our capital stock. We currently intend to retain any future earnings to fund the development and expansion of our business, and therefore we do not anticipate paying cash dividends on our common stock in the foreseeable future. Any future determination to pay dividends will be at the discretion of our Board of Directors.

Recent Sales of Unregistered Equity Securities

Except as previously reported in our Quarterly Reports on Form 10-Q and Current Reports on Form 8-K filed with the Securities and Exchange Commission during the year ended December 31, 2018, there were no unregistered sales of equity securities by us during the year ended December 31, 2018.

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ITEM 6. SELECTED FINANCIAL DATA

You should read the selected financial data below in conjunction with "Management's Discussion and Analysis of Financial Condition and Results of Operations" and the audited financial statements, notes thereto and other financial information included elsewhere in this Annual Report on Form 10-K. The following selected financial data have been derived from our audited historical financial statements, certain of which are included elsewhere in the Annual Report on Form 10-K. Historical results are not indicative of the results to be expected in the future.

	Years Ended December 31,				
	2018	2017	2016	2015	2014
	(in thousands, except per share data)				
Statement of Operations Data:					
Expenses:					
Research and development expenses	$ 29,669	$ 21,467	$ 17,228	$ 13,607	$ 20,870
General and administrative expenses	9,390	9,188	8,705	8,234	9,478
Total expenses	39,059	30,655	25,933	21,841	30,348
Loss from operations	(39,059)	(30,655)	(25,933)	(21,841)	(30,348)
Other income (expense), net	641	216	46	57	(259)
Gain (loss) on change in fair value of warrant liability (a)			8,163	3,081	(8,804)
Loss from operations before income taxes	(38,418)	(30,439)	(17,724)	(18,703)	(39,411)
Income tax benefit					
Net loss	$ (38,418)	$ (30,439)	$ (17,724)	$ (18,703)	$ (39,411)
Net loss per share basic and diluted: (b)					
Net loss per share basic	$ (1.65)	$ (1.75)	$ (1.22)	$ (1.33)	$ (4.82)
Net loss per share diluted	$ (1.65)	$ (1.75)	$ (1.22)	$ (1.47)	$ (4.82)

	As of December 31,				
	2018	2017	2016	2015	2014
	(in thousands)				
Balance Sheet Data:					
Cash, cash equivalents and short-term investments (c)	$ 28,458	$ 43,899	$ 21,869	$ 29,256	$ 49,295
Working capital	25,998	38,102	19,687	1,717	17,359
Total assets	31,321	46,236	24,502	32,031	50,651
Accumulated deficit	(600,055)	(561,637)	(531,198)	(513,474)	(494,771)
Total stockholders' equity	26,111	38,261	19,891	1,859	17,829

(a) The gain (loss) on the change in fair value of warrant liability is related to the private placement of warrants completed in November 2014. See Note 6, *Stockholders' Equity*, for further information.

(b)

Net loss per share basic and diluted disclosures have been adjusted to give effect to the one-for-ten reverse stock split of our outstanding common stock effected on December 5, 2016.

(c)

Cash, cash equivalents and short-term investments for the year ended December 31, 2018 includes the net proceeds of $24.5 million received from the sale of common stock under our At-the-Market Equity Offering℠ Sales Agreement with Stifel, Nicolaus & Company, Incorporated, in May 2017. Cash, cash equivalents and short-term investments for the year ended December 31, 2017 includes the net proceeds of $45.6 million received from the private placement of common stock and warrants completed in September 2017. Cash, cash equivalents and short-term investments for the year ended December 31, 2016 includes the net proceeds of $13.7 million received from the registered direct offering of common stock completed in October 2016. Cash, cash equivalents and short-term investments for the year ended December 31, 2014 includes the net proceeds of $21.1 million and $42.8 million received from the private placements of common stock and warrants completed in March and November 2014, respectively. See Note 6, *Stockholders' Equity*, for further information.

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ITEM 7. MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

The following discussion and analysis should be read in conjunction with our financial statements and related notes included elsewhere in this Annual Report on Form 10-K. This discussion contains forward-looking statements based upon current expectations that involve risks and uncertainties. Our actual results and the timing of selected events could differ materially from those anticipated in these forward-looking statements as a result of several factors, including those set forth under Part I, Item 1A "Risk Factors" and elsewhere in this Annual Report on Form 10-K. See "Special Note Regarding Forward-Looking Statements" in this Annual Report on Form 10-K.

Overview

Business Overview and Highlights

We are a biopharmaceutical company dedicated to the discovery, development and commercialization of medicines to treat serious and/or significant unmet medical conditions. Under an exclusive worldwide license agreement with the University of Tennessee Research Foundation, or UTRF, we are developing UTRF's proprietary selective androgen receptor degrader, or SARD, technology, which we believe has the potential to provide compounds that can degrade or antagonize multiple forms of androgen receptor, or AR, thereby potentially 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 androgen targeted therapies. We are in the process of completing ongoing mechanistic preclinical studies in order to select the most appropriate SARD compounds to move forward into the additional preclinical studies required to submit an investigational new drug application, or IND, and potentially advance one of our SARD compounds into a first-in-human clinical trial.

We had been developing selective androgen receptor modulators, or SARMs. Our SARM product candidate, enobosarm (GTx-024), was most recently evaluated in post-menopausal women with stress urinary incontinence, or SUI. During the third quarter of 2018, we announced that our randomized, placebo-controlled Phase 2 clinical trial, or the ASTRID trial, evaluating the change in the mean number of daily SUI episodes following 12 weeks of enobosarm treatment failed to achieve statistical significance on the primary endpoint of the proportion of patients with a greater than 50% reduction in incontinence episodes per day compared to placebo. We have completed the ASTRID trial, including our review of the full data sets from the clinical trial, and have determined that there is not a sufficient path forward to warrant additional clinical development of enobosarm to treat SUI. We have therefore discontinued further development of enobosarm to treat SUI, including discontinuing the related durability and open-label safety extension studies we initiated before we received topline data from the ASTRID trial. We have also discontinued any further development of our SARM program generally.

Following the announcement of the ASTRID trial results, our board of directors commenced a process of evaluating strategic alternatives to maximize stockholder value. To assist with this process, our board of directors engaged a financial advisory firm to help explore our available strategic alternatives, including possible mergers and business combinations, a sale of part or all of our assets, and collaboration and licensing arrangements. On March 6, 2019, we and Oncternal Therapeutics Inc., or Oncternal, announced the signing of an Agreement and Plan of Merger and Reorganization, or the Merger Agreement. Upon the terms and subject to the satisfaction of the conditions described in the Merger Agreement, including approval of the transaction by our stockholders and Oncternal's stockholders, a wholly-owned subsidiary of GTx will be merged with and into Oncternal, or the Merger, with Oncternal surviving the Merger as a wholly-owned subsidiary of GTx. For more information on the terms of the Merger Agreement and the transactions contemplated thereby, see the section entitled "Business Overview" under Part 1, Item 1 of this Annual Report on Form 10-K and Note 2, *Significant Accounting Policies Subsequent Events*, in the accompanying Notes to Financial Statements.

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Although we have entered into the Merger Agreement and intend to consummate the proposed Merger, there is no assurance that we will be able to successfully consummate the Merger on a timely basis, or at all. If, for any reason, the Merger is not completed, we will reconsider our strategic alternatives and could pursue one or more of the following courses of action:

Continue development of our SARD program. As set forth above, we are in the process of completing ongoing mechanistic preclinical studies in order to select the most appropriate SARD compounds to move forward into the additional preclinical studies required to submit an IND and potentially advance one of our SARD compounds into a first-in-human clinical trial. Accordingly, if, for any reason, the Merger is not consummated, we may determine to move forward with our planned IND-enabling studies of our SARD compounds. However, while we believe that our existing capital resources will be adequate to enable us to conduct and complete planned IND-enabling preclinical studies of our SARD compounds, we will require significant additional financial resources in order to initiate and complete initial human clinical trials of a SARD compound and to otherwise further the development of our SARD program. As a result, we may also resume our efforts to seek additional funds through potential collaborative, partnering or other strategic arrangements to provide us with the necessary resources for the development of our SARD program.

Pursue potential collaborative, partnering or other strategic arrangements for our SARM assets, including a sale or other divestiture of our SARM assets. We have discontinued further development of our SARM program, including enobosarm, and do not currently have any plans to resume development of our SARM program. We continue our efforts to seek potential collaborative, partnering or other strategic arrangements for our SARM assets, including a sale or other divestiture of our SARM assets.

Pursue another strategic transaction like the proposed Merger. Our board of directors may elect to pursue an alternative strategy, one of which may be a strategic transaction similar to the proposed Merger.

Dissolve and liquidate our assets. If, for any reason, the Merger is not consummated and we are unable to identify and complete an alternative strategic transaction like the Merger or potential collaborative, partnering or other strategic arrangements for our SARM assets, or to continue to operate our business due to our inability to raise additional funding for the development of our SARM program or otherwise, we may be required to dissolve and liquidate our assets. In such case, we would be required to pay all of our debts and contractual obligations, and to set aside certain reserves for potential future claims, and there can be no assurances as to the amount or timing of available cash left to distribute to our stockholders after paying our debts and other obligations and setting aside funds for reserves.

Financial Highlights

Our net loss for the year ended December 31, 2018 was $38.4 million. We expect to incur significant operating losses for the foreseeable future depending on the extent of our preclinical and any clinical development activities and, if any such development activities are successful, potentially seeking regulatory approval of any potential future product candidates. We have funded our operations primarily through the sale of equity securities, collaboration and license agreements, and prior to September 2012, product revenue from sales of FARESTON®, the rights to which we sold to a third party in the third quarter of 2012. We do not expect to receive regulatory approval for the commercial sale of any product candidates for the foreseeable future, if at all.

At December 31, 2018, we had cash, cash equivalents and short-term investments of $28.5 million compared to $43.9 million at December 31, 2017. In May 2018, we sold 1.5 million shares of common stock under our At-the-Market Equity Offering℠ Sales Agreement, or the ATM Sales Agreement, with Stifel, Nicolaus & Company, Incorporated, or Stifel, and raised net proceeds of $24.5 million.

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To conserve our cash resources, we have substantially reduced our workforce since November 2018 and have ceased our SARM development activities and all other operations except for day-to-day business operations, completing ongoing mechanistic SARD preclinical studies and those activities necessary to complete the proposed Merger. In the first quarter of 2019, due to the entry into the Merger Agreement with Oncternal, our board of directors committed to reducing our workforce down to a total of eleven full-time employees, who will remain with us until the closing of the transaction to assist with our day-to-day business operations, including continuing our ongoing mechanistic SARD preclinical studies, and those activities necessary to complete the proposed Merger. All employees affected by the workforce reduction will be eligible to receive, among other things, specified severance payments based on the applicable employee's level and years of service with us and the continuation of group health insurance coverage. In addition, the affected employees will also be eligible for full vesting acceleration of their outstanding stock options as well as an extension of the post-termination exercise period for their outstanding stock options. As a result of the workforce reduction and prior termination of three employees earlier in the first quarter of 2019, we estimate that we will incur total severance-related charges for these employees of approximately $1.0 million in the first quarter of 2019 and up to an additional $500,000 contingent upon the closing of the Merger. We do not expect to record a non-cash charge related to the modification of outstanding stock options in connection with the workforce reduction.

If the proposed Merger is not completed, based on our current business plan and spending assumptions as a standalone company, we estimate that our current cash, cash equivalents and short-term investments, together with interest thereon, will be sufficient to meet our projected operating requirements for at least the next 12 months. 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 sooner than currently anticipated.

While we believe that our existing capital resources will be adequate to enable us to conduct and complete planned IND-enabling preclinical studies of SARD compounds, we will require significant additional financial resources in order to initiate and complete initial human clinical trials of a SARD compound and to otherwise further the development of our SARD program. If we are unable to raise sufficient additional funds for the development of our SARD program, whether through potential collaborative, partnering or other strategic arrangements or otherwise, or if we otherwise determine to discontinue the development of our SARD program, we will likely determine to cease operations.

While we have been able to fund our operations to date, we have no ongoing collaborations for the development and commercialization of any product candidates and no source of revenue, nor do we expect to generate product revenue for the foreseeable future. We do not have any commitments for future external funding. In addition, although we have entered into an At-the-Market Equity Offering^{SM} Sales Agreement with Stifel, Nicolaus & Company, Incorporated, or the ATM Sales Agreement, under which approximately $25.0 million of shares of our common stock remained available for sale at December 31, 2018, it is unlikely we could raise sufficient funds under the ATM Sales Agreement to permit us to initiate and complete initial human clinical trials of a SARD compound, and given our currently-depressed stock price, the ATM Sales Agreement is not otherwise expected to be a practical source of liquidity for us at this time. Further, given our currently-depressed stock price, we are significantly limited in our ability to sell shares of common stock under the ATM Sales Agreement since the issuance and sale of our common stock under the ATM Sales Agreement, if it occurs, would be effected under a registration statement on Form S-3 that we filed with the Securities and Exchange Commission, and in accordance with the rules governing those registration statements, we generally can only sell shares of our common stock under that registration statement in an amount not to exceed one-third of our public float, which limitation for all practical purposes precludes our ability to obtain any meaningful funding through the ATM Sales Agreement at this time.

Until we can generate a sufficient amount of product revenue, which we may never do, we will need to finance future cash needs through potential collaborative, partnering or other strategic arrangements, as well as

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through public or private equity offerings or debt financings or a combination of the foregoing. If we are unable to raise additional funds, we will need to continue to reduce our expenditures in order to preserve our cash. Further cost-cutting measures that we may take may not be sufficient to enable us to meet our cash requirements, and they may negatively affect our business and our ability to derive any value from our SARD program. In any event, in order to further the development of our SARD program, we will need to raise substantial additional capital. Our failure to do so would likely result in our determining to cease operations.

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 2019 to be significantly less than fiscal year 2018 primarily due to the completion of the ASTRID trial and termination of the related extension studies and due to the reductions in headcount during the fourth quarter of 2018 and the first quarter of 2019.

There is a substantial risk that any development program may not produce revenue. Moreover, because of uncertainties inherent in drug development, including those factors described in Part I, Item 1A "Risk Factors" of this Annual Report on Form 10-K, we and/or potential future collaborators may not be able to successfully develop and commercialize any of our product candidates.

The successful development and commercialization of our product candidates is highly uncertain. We cannot reasonably estimate or know the nature, timing and estimated costs of the efforts necessary to complete the development and commercialization of, or the period in which material net cash inflows are expected to commence from, any of our product candidates due to the numerous risks and uncertainties associated with developing and commercializing drugs, including the uncertainty of:

- the scope, rate of progress and cost of our preclinical and potential future clinical development programs;
- the terms and timing of any potential collaborative, partnering 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;
- potential future clinical trial results;
- the cost and timing of regulatory filings and/or approvals to commercialize any potential future 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.

Any failure to complete the development of any potential future product candidates in a timely manner could have a material adverse effect on our operations, financial position and liquidity. A discussion of the risks

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and uncertainties associated with completing our development efforts on schedule, or at all, and some consequences of failing to do so, are set forth under Part I, Item 1A "Risk Factors" of this Annual Report on Form 10-K.

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 relations services. We expect our general and administrative expenses for fiscal year 2019 to decrease in comparison to fiscal year 2018 due to the reductions in headcount during the fourth quarter of 2018 and the first quarter of 2019.

Critical Accounting Policies and Significant Judgments and Estimates

Our management's discussion and analysis of our financial condition and results of operations is based on our financial statements, which have been prepared in accordance with accounting principles generally accepted in the United States of America. The preparation of these financial statements requires us to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of the financial statements as well as the reported revenues and expenses during the reporting periods. On an ongoing basis, we evaluate our estimates and judgments related to revenue recognition, 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 at the end of this Annual Report on Form 10-K, 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 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 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.

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The determination of the fair value of stock options on the date of grant include the expected life of the award, the expected stock price volatility over the expected life of the awards, and risk-free interest rate. We estimate the expected life of options by calculating the average of the vesting term and contractual term of the options. We estimate the expected stock price volatility based on the historical volatility of our common stock. The risk-free interest rate is determined using U.S. Treasury rates where the term is consistent with the expected life of the stock options. Expected dividend yield is not considered as we have not made any dividend payments and have no plans of doing so in the foreseeable future. The fair value of each stock option is amortized into compensation expense on a straight-line basis between the grant date for the award and each vesting date. During the first quarter of 2017, we 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 are now being 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. We 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 statements of operations for the years ended December 31, 2018, 2017 and 2016:

	Years ended December 31,		
	2018	**2017**	**2016**
	(in thousands)		
Research and development expenses	$ 807	$ 1,171	$ 1,260
General and administrative expenses	1,556	2,146	1,829
Total share-based compensation	$ 2,363	$ 3,317	$ 3,089

Share-based compensation expense recorded in the statement of operations as general and administrative expense for the years ended December 31, 2018, 2017 and 2016 included share-based compensation expense related to deferred compensation arrangements for our non-employee directors of $166,000, $166,000 and $132,000, respectively. At December 31, 2018, the total compensation cost related to non-vested stock options not yet recognized was approximately $7.7 million with a weighted average expense recognition period of 2.95 years.

Income Taxes

We account for deferred taxes by recognition of deferred tax assets and liabilities for the expected future tax consequences of events that have been included in the financial statements or tax returns. Under this method, deferred tax assets and liabilities are determined based on the difference between the financial statement and tax basis of assets and liabilities using enacted tax rates in effect for the year in which the differences are expected to reverse. A valuation allowance is provided when it is more likely than not that some portion or all of the deferred tax assets will not be realized. Accordingly, at December 31, 2018 and 2017, net of the valuation allowance, the net deferred tax assets were reduced to zero.

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Results of Operations

Research and Development Expenses

The following table identifies the research and development expenses for our SARD program and our discontinued SARM program, 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	Years Ended December 31, 2018	2017	2016
			(in thousands)	
Enobosarm Treatment of postmenopausal women with SUI (1 mg and 3 mg)	**SARM**	$ 25,576	$ 11,279	$ 1,286
Enobosarm Treatment of women with ER positive and AR positive advanced breast cancer (9 mg and 18 mg)	**SARM**	1,957	5,541	7,316
SARDs Treatment of castration resistant prostate cancer	**SARD**	1,052	1,772	2,157
Enobosarm Treatment of women with advanced AR positive TNBC (18 mg)	**SARM**	801	2,348	4,853
Other research and development		283	527	1,616
Total research and development expenses		$ 29,669	$ 21,467	$ 17,228

Research and development expenses increased 38% to $29.7 million for the year ended December 31, 2018 from $21.5 million for the year ended December 31, 2017. Research and development expenses increased 25% to $21.5 million for the year ended December 31, 2017 from $17.2 million for the year ended December 31, 2016.

Research and development expenses for enobosarm for the treatment of postmenopausal women with SUI substantially increased from the years ended December 31, 2017 and 2016 due to the initiation of a placebo-controlled Phase 2 clinical trial of enobosarm to treat postmenopausal women with SUI, which opened for enrollment in the third quarter of 2017 and completed enrollment in the second quarter of 2018, and due to the related durability and open-label safety extension studies, which were initiated in the second quarter of 2018. During the third quarter of 2018, we announced that the ASTRID trial failed to achieve statistical significance on the primary endpoint of the proportion of patients with a greater than 50% reduction in incontinence episodes per day compared to placebo. The years ended December 31, 2016 and 2017 also included expenses related to 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.

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Research and development expenses for enobosarm for the treatment of women with ER positive and AR positive advanced breast cancer decreased from the years ended December 31, 2017 and 2016 due primarily to the timing and nature of activities related to conducting the 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.

Research and development expenses for the SARD program for the year ended December 31, 2018 decreased from the prior years due to fewer drug formulation and preclinical research expenses being incurred during 2018 than in the comparable periods. If the proposed Merger is not completed, we expect increased research and development expenses for the SARD program in 2019 as we plan to complete ongoing mechanistic preclinical studies, and to select the most appropriate SARD compounds to move forward with IND-enabling preclinical studies.

Research and development expenses for enobosarm for the treatment of women with AR positive TNBC decreased from the years ended December 31, 2017 and 2016 due to the timing and nature of activities related to conducting the first stage of the 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.

General and Administrative Expenses

General and administrative expenses for the year ended December 31, 2018 of $9.4 million remained relatively consistent with the year ended December 31, 2017 of $9.2 million. General and administrative expenses increased 6% to $9.2 million for the year ended December 31, 2017 from $8.7 million for the year ended December 31, 2016. The increase during the year ended December 31, 2017 from the prior year was due primarily to an increase in share-based compensation expense.

Other Income (Expense), Net

Other income, net for the years ended December 31, 2018, 2017, and 2016 was $641,000, $216,000 and $46,000, respectively, and consisted of interest earned on our cash, cash equivalents and short-term investments, foreign currency transaction gains and losses, and other non-operating income or expense. The increase in other income, net for each year over year was primarily due to interest earned on the net proceeds received from issuances of common stock by the Company.

Liquidity and Capital Resources

We have financed our operations to date primarily through public offerings and private placements of our securities, as well as payments from our former collaborators. We have incurred significant losses since our inception in 1997 as we have devoted substantially all of our resources to research and development, including our clinical trials. As of December 31, 2018, we had an accumulated deficit of $600.1 million, which resulted primarily from:

our research and development activities associated with:

the preclinical development of our SARD program;

the preclinical and clinical development of our SARM compounds, including enobosarm;

the preclinical and clinical development of our discontinued GTx-758 product candidate for the treatment of advanced prostate cancer;

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- the development of our discontinued toremifene 80 mg product candidate to reduce fractures and treat other estrogen deficiency side effects of androgen deprivation therapy in men with prostate cancer, including two Phase 2 clinical trials, a Phase 3 clinical trial, and the preparation and submission of a NDA to the FDA;

- the development of our discontinued toremifene 20 mg product candidate for the prevention of prostate cancer in high risk men with high grade prostatic intraepithelial neoplasia, including a Phase 2b clinical trial and a Phase 3 clinical trial; and

- the preclinical development of other product candidates; and

- general and administrative expenses.

We expect to incur significant operating losses for the foreseeable future depending on the extent of our preclinical and any clinical development activities and, if any such development activities are successful, potentially seeking regulatory approval of any potential future product candidates. These losses, among other things, have had and will continue to have an adverse effect on our stockholders' equity and working capital. We do not expect to receive regulatory approval for the commercial sale of any product candidates for the foreseeable future, if at all.

At December 31, 2018, we had cash, cash equivalents and short-term investments of $28.5 million, compared to $43.9 million at December 31, 2017 and $21.9 million at December 31, 2016.

In February 2018, we entered into the ATM Sales Agreement, pursuant to which we may offer and sell, from time to time, through Stifel, shares of our common stock having an aggregate offering price of up to $50 million. We are not obligated to sell any shares under the ATM Sales Agreement. Subject to the terms and conditions of the sales agreement, Stifel will use commercially reasonable efforts, consistent with its normal trading and sales practices, applicable state and federal law, rules and regulations and the rules of the NASDAQ Capital Market, to sell shares from time to time based upon our instructions, including any price, time or size limits specified by us. Under the ATM Sales Agreement, Stifel may sell shares by any method deemed to be an "at-the-market" offering as defined in Rule 415 under the Securities Act of 1933, as amended, or any other method permitted by law, including in privately negotiated transactions. We will pay Stifel a commission of up to 3.0% of the aggregate gross proceeds from each sale of shares. In May 2018, we sold 1.5 million shares of common stock under the ATM Sales Agreement for net proceeds of $24.5 million. As of December 31, 2018, we had approximately $25.0 million of common stock remaining available to be sold under the ATM Sales Agreement. However, it is unlikely we could raise sufficient funds under the ATM Sales Agreement to permit us to initiate and complete initial human clinical trials of a SARD compound, and given our currently-depressed stock price, the ATM Sales Agreement is not otherwise expected to be a practical source of liquidity for us at this time. Further, given our currently-depressed stock price, we are significantly limited in our ability to sell shares of common stock under the ATM Sales Agreement since the issuance and sale of our common stock under the ATM Sales Agreement, if it occurs, would be effected under a registration statement on Form S-3 that we filed with the Securities and Exchange Commission, and in accordance with the rules governing those registration statements, we generally can only sell shares of our common stock under that registration statement in an amount not to exceed one-third of our public float, which limitation for all practical purposes precludes our ability to obtain any meaningful funding through the ATM Sales Agreement at this time.

On September 29, 2017, we completed a private placement of units consisting of an aggregate of 5.5 million shares of common stock and warrants to purchase an aggregate of 3.3 million shares of our common stock for net proceeds to us of approximately $45.6 million. The purchasers in the registered

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direct offering consisted solely of accredited investors that included certain institutional and existing stockholders, including a member of our board of directors.

On October 14, 2016, we completed a registered direct offering of our common stock consisting of 1.7 million shares of our common stock for net proceeds of approximately $13.7 million. The purchasers in the registered direct offering consisted of certain existing GTx stockholders and certain members of the GTx management team and board of directors.

The following table shows a summary of our cash flows for the periods indicated:

	Years Ending December 31,		
	2018	2017	2016
	(in thousands)		
Net cash used in operating activities	$ (39,346)	$ (23,460)	$ (20,778)
Net cash provided by (used in) investing activities	27,883	(15,126)	2,151
Net cash provided by financing activities	23,905	45,492	13,481
Net increase (decrease) in cash and cash equivalents	$ 12,442	$ 6,906	$ (5,146)

Net cash used in operating activities in all periods resulted primarily from funding our operations.

Net cash provided by investing activities was $27.9 million for the year ended December 31, 2018 and resulted primarily from maturities of short-term investments of $72.0 million offset by the purchase of short-term investments of $44.2 million. Net cash used in investing activities for the year ended December 31, 2017 primarily resulted from the purchase of short-term investments of $39.3 million offset by the maturities of short-term investments of $24.2 million. Net cash provided by investing activities for the year ended December 31, 2016 primarily resulted from the maturities of short-term investments of $37.6 million offset by the purchase of short-term investments of $35.4 million.

Net cash provided by financing activities for the year ended December 31, 2018 of $23.9 million resulted from the sale of common stock under the ATM Sales Agreement with Stifel and proceeds from the exercise of stock options of $103,000, offset slightly by $672,000 of tax payments related to shares withheld for vested restricted stock units. Net cash provided by financing activities for the year ended December 31, 2017 reflected net proceeds of $45.6 million from the issuance of common stock and warrants related to the September 2017 private placement, partially offset by $156,000 of employee withholding tax payments related to vested RSUs. Net cash provided by financing activities for the year ended December 31, 2016 reflected net proceeds of $13.7 million from the issuance of common stock related to the October 2016 registered direct offering, partially offset by $208,000 of employee withholding tax payments related to vested RSUs.

To conserve our cash resources, we have substantially reduced our workforce since November 2018 and have ceased our SARM development activities and all other operations except for day-to-day business operations, completing ongoing mechanistic SARD preclinical studies and those activities necessary to complete the proposed Merger. If the proposed Merger is not completed, based on our current business plan and spending assumptions as a standalone company, we estimate that our current cash, cash equivalents and short-term investments, together with interest thereon, will be sufficient to meet our projected operating requirements for at least the next 12 months. 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 sooner than currently anticipated.

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While we believe that our existing capital resources will be adequate to enable us to conduct and complete planned IND-enabling preclinical studies of SARD compounds, we will require significant additional financial resources in order to initiate and complete initial human clinical trials of a SARD compound and to otherwise further the development of our SARD program. If we are unable to raise sufficient additional funds for the development of our SARD program, whether through potential collaborative, partnering or other strategic arrangements or otherwise, or if we otherwise determine to discontinue the development of our SARD program, we will likely determine to cease operations.

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 Annual Report on Form 10-K. 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 potential future product candidates, if any. Our future funding requirements will depend on many factors, including:

- our ability to successfully complete the Merger;
- the scope, rate of progress and cost of our preclinical and potential future clinical development programs;
- the terms and timing of any potential collaborative, partnering 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;
- potential future clinical trial results;
- the cost and timing of regulatory filings and/or approvals to commercialize any potential future 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 have no ongoing collaborations for the development and commercialization of any product candidates and no source of revenue, nor do we expect to generate product revenue for the foreseeable future. We do not have any commitments for future external funding.

Until we can generate a sufficient amount of product revenue, which we may never do, we will need to finance future cash needs through potential collaborative, partnering or other strategic arrangements, as well as through public or private equity offerings or debt financings or a combination of the foregoing. If we are unable to raise additional funds, we will need to continue to reduce our expenditures in order to preserve our cash. Further cost-cutting measures that we may take may not be

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sufficient to enable us to meet our cash requirements, and they may negatively affect our business and our ability to derive any value from our SARD program. In any event, in order to further the development of our SARD program, we will need to raise substantial additional capital. Our failure to do so would likely result in our determining to cease operations.

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 and intellectual property rights thereof, or grant licenses on terms that are not favorable to us, any of which could result in our stockholders having little or no continuing interest in our SARD program and/or SARM assets 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 substantially dilutive private placements of our common stock and warrants in March 2014, November 2014 and September 2017, in addition to a registered direct offering of our common stock that we completed in October 2016 and the sale of our common stock pursuant to the ATM Sales Agreement. Our stockholders will 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 have been severely harmed by the failure of the ASTRID trial to meet its primary endpoint and the resulting significant uncertainty regarding our prospects to continue as a going concern. If we are unable to complete the proposed Merger, 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, uncertainties with respect to the prospects for our early-stage SARD program, the sufficiency of our capital resources, potential future management turnover, and volatility and instability in the global financial markets. As a result of these and other factors, there is no guarantee that sufficient additional funding will be available to us on acceptable terms, or at all.

Contractual Obligations

At December 31, 2018, we had contractual obligations as follows:

	Payment Due by Period (in thousands)				
Contractual Obligations[1]	**Total**	**Less than 1 year**	**1-3 years**	**4-5 years**	**More than 5 years**
Operating lease obligations[2]	$ 162	$ 162	$	$	$

(1) This table does not include any royalty obligations under our SARM and SARD license agreements with UTRF as the timing and likelihood of such payments are not known. In addition to the minimum payments due under our SARM and SARD license agreements, we may be required to pay royalties on any net sales of product if we receive regulatory approval for a SARM, including enobosarm, or SARD product candidate and successfully market the product. Additionally, if we sublicense rights under our SARM or SARD license agreements, we also are obligated to pay a sublicense royalty on any licensing fee or milestone payments we may receive from a sublicensee.

(2) Our operating lease obligations consist of payments relating to a lease for office space at 175 Toyota Plaza, Memphis, Tennessee, which expires on April 30, 2019.

Off-Balance Sheet Arrangements

We have not engaged in any off-balance sheet arrangements, including the use of standard finance, special purpose entities or variable interest entities.

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ITEM 7A. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK

Our exposure to market risk for changes in interest rates relates to our cash equivalents on deposit in highly liquid money market funds and investments in Federal Deposit Insurance Corporation insured certificates of deposit. The primary objective of our cash investment activities is to preserve principal while at the same time maximizing the income we receive from our invested cash without significantly increasing risk of loss. We do not use derivative financial instruments in our investment portfolio. The effect of a hypothetical decrease of ten percent in the average yield earned on our cash equivalents and short-term investments would have resulted in an immaterial decrease in our interest income for the year ended December 31, 2018.

In addition, we have exposure to fluctuations in certain foreign currencies in countries in which we conduct clinical trials. Most of our foreign expenses incurred were associated with initiating or conducting clinical trials for enobosarm at clinical trial sites in Europe. Consequently, changes in exchange rates could result in material exchange losses and could unpredictably, materially and adversely affect our financial position, results of operations and cash flows. A hypothetical 10% increase or decrease in foreign exchange rates would result in an immaterial change in our financial assets and liabilities denominated in foreign currencies. This potential change is based on a sensitivity analysis performed on our financial position at December 31, 2018. Actual results may differ materially. We have elected not to hedge our exposure to foreign currency fluctuations.

ITEM 8. FINANCIAL STATEMENTS AND SUPPLEMENTARY DATA

Our financial statements and the reports of our independent registered public accounting firm are included in this Annual Report on Form 10-K beginning on page F-1. The index to these reports and our financial statements is included in Part IV, Item 15 below.

ITEM 9. CHANGES IN AND DISAGREEMENTS WITH ACCOUNTANTS ON ACCOUNTING AND FINANCIAL DISCLOSURE

None.

ITEM 9A. CONTROLS AND PROCEDURES

Disclosure Controls and Procedures

We maintain disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) of the Securities Exchange Act of 1934, as amended (the "Exchange Act")) that are designed to ensure that information required to be disclosed in the reports that we file or submit under the Exchange Act is recorded, processed, summarized, and reported within the time periods specified in the SEC's rules and forms and that such information is accumulated and communicated to our management, including our 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 as of December 31, 2018.

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Management's Report on Internal Control Over Financial Reporting

We, as management of GTx, Inc., are responsible for establishing and maintaining adequate internal control over financial reporting, as such term is defined in Securities Exchange Act Rule 13a-15(f). Internal control over financial reporting is a process designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with United States generally accepted accounting principles. Any system of internal control, no matter how well designed, has inherent limitations, including the possibility that a control can be circumvented or overridden and misstatements due to error or fraud may occur and not be detected. Also, because of changes in conditions, internal control effectiveness may vary over time. Accordingly, even an effective system of internal control will provide only reasonable assurance that the objectives of the internal control system are met.

Under the supervision and with the participation of management, including our principal executive officer and principal financial officer, we conducted an evaluation of the effectiveness of our internal control over financial reporting as of December 31, 2018 using the criteria for effective internal control over financial reporting as described in "Internal Control Integrated Framework," issued by the Committee of Sponsoring Organizations of the Treadway Commission (2013 framework). Based on this evaluation, we concluded that, as of December 31, 2018, our internal control over financial reporting was effective. The effectiveness of our internal control over financial reporting has been audited by Ernst & Young LLP, independent registered public accounting firm.

Attestation Report of the Independent Registered Public Accounting Firm

Ernst & Young LLP, an independent registered public accounting firm, has issued an audit report on our internal control over financial reporting, which report is included elsewhere herein.

Changes in Internal Control Over Financial Reporting

There were no changes in our internal control over financial reporting during the fourth quarter of 2018 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

ITEM 9B. OTHER INFORMATION

None.

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PART III

Certain information required by Part III is omitted from this Annual Report on Form 10-K and incorporated by reference to our definitive proxy statement for our 2019 Annual Meeting of Stockholders, or our 2019 Proxy Statement, to be filed pursuant to Regulation 14A of the Securities Exchange Act of 1934, as amended, or Exchange Act. If our 2019 Proxy Statement is not filed within 120 days after the end of the fiscal year covered by this Annual Report on Form 10-K, the omitted information will be included in an amendment to this Annual Report on Form 10-K filed not later than the end of such 120-day period.

ITEM 10. DIRECTORS, EXECUTIVE OFFICERS AND CORPORATE GOVERNANCE

(1) The information required by this Item concerning our directors and nominees for director, including information with respect to our audit committee and audit committee financial experts, may be found under the section entitled "Election of Directors" and "Additional Information About the Board of Directors and Certain Corporate Governance Matters" appearing in the 2019 Proxy Statement. Such information is incorporated herein by reference.

(2) The information required by this Item concerning compliance with Section 16(a) of the Securities Exchange Act of 1934 may be found in the section entitled "Section 16(a) Beneficial Ownership Reporting Compliance" appearing in the 2019 Proxy Statement. Such information is incorporated herein by reference.

(3) The information required by this Item concerning our executive officers is set forth in the section entitled "Executive Officers of the Registrant" in Part I, Item 1 of this Form 10-K.

(4) Our Board has adopted a Code of Business Conduct and Ethics applicable to all officers, directors and employees as well as Guidelines on Governance Issues. These documents are available on our Web site (www.gtxinc.com) under "Investors" at "Corporate Governance." We will provide a copy of these documents to any person, without charge, upon request, by writing to us at GTx, Inc., Chief Legal Officer, 175 Toyota Plaza, Suite 700, Memphis, Tennessee 38103. We intend to satisfy the disclosure requirement under Item 5.05 of Form 8-K regarding an amendment to, or waiver from, a provision of the Code of Business Conduct and Ethics by posting such information on our Web site at the address and the location specified above.

ITEM 11. EXECUTIVE COMPENSATION

The information required by this Item concerning director and executive compensation is incorporated herein by reference to the information from the 2019 Proxy Statement under the sections entitled "Executive Compensation" and "Director Compensation."

ITEM 12. SECURITY OWNERSHIP OF CERTAIN BENEFICIAL OWNERS AND MANAGEMENT AND RELATED STOCKHOLDER MATTERS

(1) The information required by this Item with respect to security ownership of certain beneficial owners and management is incorporated herein by reference to the information from the 2019 Proxy Statement under the section entitled "Security Ownership of Certain Beneficial Owners and Management."

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(2) The information required by this Item with respect to securities authorized for issuance under our equity compensation plans is incorporated herein by reference to the information from the 2019 Proxy Statement under the section entitled "Equity Compensation Plan Information."

ITEM 13. CERTAIN RELATIONSHIPS AND RELATED TRANSACTIONS, AND DIRECTOR INDEPENDENCE

(1) The information required by this Item concerning related party transactions is incorporated herein by reference to the information from the 2019 Proxy Statement under the section entitled "Related Party Transactions and Indemnification."

(2) The information required by this Item concerning director independence is incorporated herein by reference to the information from the 2019 Proxy Statement under the section entitled "Additional Information About the Board of Directors and Certain Corporate Governance Matters Director Independence."

ITEM 14. PRINCIPAL ACCOUNTING FEES AND SERVICES

The information required by this Item is incorporated herein by reference to the information from the 2019 Proxy Statement under the section entitled "Ratification of Appointment of Independent Registered Public Accounting Firm."

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PART IV

ITEM 15. EXHIBITS AND FINANCIAL STATEMENT SCHEDULES

(a)(1) Index to Financial Statements

(a)(2) Financial statement schedules are omitted as they are not applicable.

(a)(3) See Item 15(b) below.

(b) Exhibits The following exhibits are included herein or incorporated herein by reference:

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
2.2**	Agreement and Plan of Merger and Reorganization, dated March 6, 2019, by and among the Registrant, Oncternal Therapeutics, Inc. and Grizzly Merger Sub, Inc.	8-K	000-50549	2.1	03/07/2019
2.3**	Form of CVR Agreement by and between the Registrant, Marc S. Hanover, as the Holders' Representative, and Computershare Investor Services, as Rights Agent.	8-K	000-50549	2.2	03/07/2019
2.4	Form of GTx Voting Agreement, dated March 6, 2019, by and between Oncternal Therapeutics, Inc., the Registrant and each of the parties named in each agreement therein	8-K	000-50549	2.3	03/07/2019
2.5	Form of Oncternal Voting Agreement, dated March 6, 2019, by and between the Registrant, Oncternal Therapeutics, Inc. and each of the parties named in each agreement therein	8-K	000-50549	2.4	03/07/2019

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Exhibit Number	Exhibit Description	Incorporation By Reference			
		Form	SEC File No.	Exhibit	Filing Date
2.6	Form of Lock-Up Agreement, dated March 6, 2019, by each of the parties named in each agreement therein	8-K	000-50549	2.5	03/07/2019
3.1	Restated Certificate of Incorporation of GTx, Inc.	S-3	333-127175	4.1	08/04/2005
3.2	Certificate of Amendment of Restated Certificate of Incorporation of GTx, Inc.	8-K	000-50549	3.2	05/06/2011
3.3	Certificate of Amendment of Restated Certificate of Incorporation of GTx, Inc.	8-K	000-50549	3.3	05/09/2014
3.4	Certificate of Amendment of Restated Certificate of Incorporation of GTx, Inc.	10-Q	000-50549	3.4	05/11/2015
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	3.1	03/07/2019
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 among Registrant, 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

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Exhibit Number	Exhibit Description	Incorporation By Reference			
		Form	SEC File No.	Exhibit	Filing Date
4.7	Consent, Waiver and Amendment Agreement between Registrant and J.R. Hyde, III and Pittco Associates, L.P., dated August 4, 2014	10-Q	000-50549	4.8	08/05/2014
4.8	Form of Common Stock Warrant, issued by Registrant pursuant to the Purchase Agreement, dated November 9, 2014, between Registrant and the purchasers identified in Exhibit A therein	10-K	000-50549	4.9	03/16/2015
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	05/10/2016
4.10	Form of Common Stock Warrant, issued by Registrant pursuant to the Purchase Agreement, dated September 25, 2017, between Registrant and the purchasers identified in Exhibit A therein	S-3	333-221040	4.9	10/20/2017
10.1	Consolidated, Amended, and Restated License Agreement dated July 24, 2007, between Registrant and University of Tennessee Research Foundation	10-Q	000-50549	10.40	11/09/2007
10.2	First Amendment, dated December 29, 2008, to the Consolidated, Amended and Restated License Agreement dated July 24, 2007 between the Registrant and University of Tennessee Research Foundation	10-K	000-50549	10.47	03/03/2009
10.3*	Form of Indemnification Agreement	S-1	333-109700	10.12	12/22/2003
10.4*	Genotherapeutics, Inc. 1999 Stock Option Plan, as amended through December 10, 2009 (refiled to reflect reverse stock split effected on December 5, 2016), and Form of Stock Option Agreement	10-K	000-50549	10.4	03/24/2017
10.5*	GTx, Inc. 2000 Stock Option Plan, as amended through December 10, 2009 (refiled to reflect reverse stock split effected on December 5, 2016), and Form of Stock Option Agreement	10-K	000-50549	10.5	03/24/2017

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Exhibit Number	Exhibit Description	Incorporation By Reference			
		Form	SEC File No.	Exhibit	Filing Date
10.6*	GTx, Inc. 2001 Stock Option Plan, as amended through November 3, 2009 (refiled to reflect reverse stock split effected on December 5, 2016), and Form of Stock Option Agreement	10-K	000-50549	10.6	03/24/2017
10.7*	GTx, Inc. 2002 Stock Option Plan, as amended through November 3, 2009 (refiled to reflect reverse stock split effected on December 5, 2016), and Form of Stock Option Agreement	10-K	000-50549	10.7	03/24/2017
10.8*	GTx, Inc. 2004 Equity Incentive Plan, as originally adopted, and Form of Stock Option Agreement	S-1	333-109700	10.5	01/15/2004
10.9*	GTx, Inc. 2004 Equity Incentive Plan, as amended effective April 30, 2008	8-K	000-50549	10.6	05/06/2008
10.10*	GTx, Inc. 2004 Equity Incentive Plan, as amended effective November 4, 2008 (refiled to reflect reverse stock split effected on December 5, 2016) and Form of Stock Option Agreement	10-K	000-50549	10.10	03/24/2017
10.11*	GTx, Inc. 2004 Non-Employee Directors' Stock Option Plan and Form of Stock Option Agreement, as originally adopted	S-1	333-109700	10.6	01/15/2004
10.12*	Amended and Restated GTx, Inc. 2004 Non-Employee Directors' Stock Option Plan, effective April 26, 2006	8-K	000-50549	10.1	04/27/2006
10.13*	Form of Stock Option Agreement under the Amended and Restated GTx, Inc. 2004 Non-Employee Directors' Stock Option Plan	10-Q	000-50549	10.35	08/09/2006
10.14*	Amended and Restated GTx, Inc. 2004 Non-Employee Directors' Stock Option Plan, as amended effective November 4, 2008 (refiled to reflect reverse stock split effected on December 5, 2016)	10-K	000-50549	10.14	03/24/2017
10.15*	GTx, Inc. 2013 Equity Incentive Plan, as originally adopted	S-8	333-188377	99.1	05/06/2013

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Exhibit Number	Exhibit Description	Incorporation By Reference			
		Form	SEC File No.	Exhibit	Filing Date
10.16*	GTx, Inc. 2013 Equity Incentive Plan, as amended effective May 6, 2015 (refiled to reflect reverse stock split effected on December 5, 2016)	10-K	000-50549	10.16	03/24/2017
10.17*	Form of Stock Option Grant Notice and Option Agreement under the GTx, Inc. 2013 Equity Incentive Plan (Standard Form)	10-Q	000-50549	10.2	07/22/2013
10.18*	Form of Retention Stock Option Grant Notice and Option Agreement under the GTx, Inc. 2013 Equity Incentive Plan	10-Q	000-50549	10.3	11/12/2013
10.19*	Form of Retention Restricted Stock Unit Grant Notice and Restricted Stock Unit Award Agreement under the GTx, Inc. 2013 Equity Incentive Plan	10-Q	000-50549	10.4	11/12/2013
10.20*	Form of Restricted Stock Unit Grant Notice and Restricted Stock Unit Award Agreement under the GTx, Inc. 2013 Equity Incentive Plan	10-Q	000-50549	10.5	05/11/2015
10.21*	GTx, Inc. 2013 Non-Employee Director Equity Incentive Plan, as originally adopted (refiled to reflect reverse stock split effected on December 5, 2016)	10-K	000-50549	10.21	03/24/2017
10.22*	Form of Stock Option Grant Notice and Option Agreement under the GTx, Inc. 2013 Non-Employee Director Equity Incentive Plan	10-Q	000-50549	10.4	07/22/2013
10.23*	Employment Agreement dated February 12, 2015, between Registrant and Robert J. Wills	10-Q	000-50549	10.4	05/11/2015
10.24*	Employment Agreement dated July 13, 2015, between Registrant and Diane C. Young	10-Q	000-50549	10.1	11/09/2015
10.25*	Amended and Restated Employment Agreement dated February 12, 2015, between Registrant and Marc S. Hanover	10-K	000-50549	10.25	03/16/2015
10.26*	Amended and Restated Employment Agreement dated February 14, 2013, between Registrant and Henry P. Doggrell	10-K	000-50549	10.22	03/05/2013

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Exhibit Number	Exhibit Description	Incorporation By Reference			
		Form	SEC File No.	Exhibit	Filing Date
10.27*	Employment Agreement dated January 6, 2017 between Registrant and Jason T. Shackelford	10-K	000-50549	10.28	03/24/2017
10.28*	Form of Retention Benefits Letter Agreement for Mitchell S. Steiner and Marc S. Hanover	10-Q	000-50549	10.1	11/12/2013
10.29*	Form of Retention Benefits Letter Agreement for Jason T. Shackelford and Henry P. Doggrell	10-Q	000-50549	10.2	11/12/2013
10.30*	Amended and Restated GTx, Inc. Executive Bonus Compensation Plan, effective November 4, 2008	10-K	000-50549	10.53	03/03/2009
10.31*	2017 Compensation Information for Registrant's Executive Officers	10-Q	000-50549	10.2	05/15/2017
10.32*	Directors' Deferred Compensation Plan, as amended and restated effective February 14, 2013	10-K	000-50549	10.28	03/05/2013
10.33*	Directors' Deferred Compensation Plan, as amended and restated effective February 18, 2016 (refiled to reflect reverse stock split effected on December 5, 2016)	10-K	000-50549	10.34	03/24/2017
10.34*	Non-Employee Director Compensation Policy of GTx, Inc., effective January 1, 2016	10-K	000-50549	10.39	03/15/2016
10.35	Lease agreement, dated April 13, 2015, between Registrant and Hertz Memphis Three LLC	10-Q	000-50549	10.1	08/10/2015
10.36	Purchase Agreement, dated November 9, 2014, between Registrant and the purchasers identified in Exhibit A therein	8-K	000-50549	10.1	11/10/2014
10.37	Form of Subscription Agreement for October 2016 registered direct offering	8-K	000-50549	10.1	10/12/2016
10.38	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	10-Q	000-50549	10.1	08/14/2017
10.39	Securities Purchase Agreement, dated as of September 25, 2017, between Registrant and the purchasers identified on Exhibit A	8-K	000-50549	10.1	09/29/2017

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Exhibit Number	Exhibit Description	Incorporation By Reference			
		Form	SEC File No.	Exhibit	Filing Date
10.40	At-the-Market Equity Offering Sales Agreement, dated February 9, 2018, by and between Registrant and Stifel, Nicolaus & Company, Incorporated	8-K	000-50549	10.1	02/09/2018
10.41+	License Agreement, effective March 1, 2015, between the Registrant and University of Tennessee Research Foundation	-	-	-	-
10.42+	Amendment #1 to the License Agreement, dated November 12, 2015, between the Registrant and University of Tennessee Research Foundation	-	-	-	-
10.43+	Amendment #2 to the License Agreement, as amended, dated August 12, 2016, between the Registrant and University of Tennessee Research Foundation	-	-	-	-
10.44+	Amendment #3 to the License Agreement, as amended, dated April 6, 2017, between the Registrant and University of Tennessee Research Foundation	-	-	-	-
10.45+	Amendment #4 to the License Agreement, as amended, dated October 23, 2018, between the Registrant and University of Tennessee Research Foundation	-	-	-	-
23.1+	Consent of Independent Registered Public Accounting Firm	-	-	-	-
24.1+	Power of Attorney (included on the signature pages hereto)	-	-	-	-
31.1+	Certification of Chief 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 Chief Executive Officer, as required by Rule 13a-14(b) or Rule 15d-14(b) and Section 1350 of Chapter 63 of Title 18 of the United States Code (18 U.S.C. §1350)[1]	-	-	-	-

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Exhibit Number	Exhibit Description	Incorporation By Reference			
		Form	SEC File No.	Exhibit	Filing Date
32.2+	Certification of Principal Financial Officer, as required by Rule 13a-14(b) or Rule 15d-14(b) and Section 1350 of Chapter 63 of Title 18 of the United States Code (18 U.S.C. §1350)(1)	-	-	-	-
101.INS+	XBRL Instance Document	-	-	-	-
101.SCH+	XBRL Taxonomy Extension Schema Document	-	-	-	-
101.CAL+	XBRL Taxonomy Extension Calculation Linkbase Document	-	-	-	-
101.DEF+	XBRL Taxonomy Extension Definition Linkbase Document	-	-	-	-
101.LAB+	XBRL Taxonomy Extension Labels Linkbase Document	-	-	-	-
101.PRE+	XBRL Taxonomy Extension Presentation Linkbase Document	-	-	-	-

**

Schedules and exhibits have been omitted pursuant to Item 601(b)(2) of Regulation S-K. A copy of any omitted schedule and/or exhibit will be furnished to the SEC upon request.

Confidential treatment has been granted with respect to certain portions of this exhibit. This exhibit omits the information subject to the related confidentiality order. Omitted portions have been filed separately with the SEC.

Confidential treatment has been requested with respect to certain portions of this exhibit. This exhibit omits the information subject to this confidentiality request. Omitted portions have been filed separately with the SEC.

*

Indicates a management contract or compensation plan or arrangement.

+

Filed herewith

(1)

This certification accompanies the Form 10-K 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-K), irrespective of any general incorporation language contained in such filing.

ITEM 16. FORM 10-K SUMMARY

None provided.

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SIGNATURES

Pursuant to the requirements of Section 13 or 15(d) 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.

By /s/ Marc S. Hanover

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

Date: March 18, 2019

POWER OF ATTORNEY

KNOW ALL PERSONS BY THESE PRESENT, that each person whose signature appears below constitutes and appoints Marc S. Hanover and Jason T. Shackelford, and each of them, acting individually, as his attorney-in-fact, each with full power of substitution and resubstitution, for him or her and in his or her name, place and stead, in any and all capacities, to sign any and all amendments to this Annual Report on Form 10-K, and to file the same, with all exhibits thereto, and other documents in connection therewith, with the Securities and Exchange Commission, granting unto said attorneys-in-fact and agents, and each of them, full power and authority to do and perform each and every act and thing requisite and necessary to be done in connection therewith and about the premises, as fully to all intents and purposes as he or she might or could do in person, hereby ratifying and confirming all that said attorneys-in-fact and agents, or any of them, or their or his or her substitute or substitutes, may lawfully do or cause to be done by virtue hereof.

Pursuant to the requirements of the Securities Exchange Act of 1934, this report has been signed below by the following persons on behalf of the registrant and in the capacities and on the dates indicated.

/s/ Marc S. Hanover Marc S. Hanover	Chief Executive Officer (Principal Executive Officer)	March 18, 2019
/s/ Jason T. Shackelford Jason T. Shackelford	Vice President, Finance and Accounting and Principal Financial and Accounting Officer (Principal Financial and Accounting Officer)	March 18, 2019
/s/ Robert J. Wills Robert J. Wills, B.S., M.S., Ph.D.	Executive Chairman of the Board of Directors	March 18, 2019
/s/ Michael G. Carter Michael G. Carter, M. D.	Director	March 18, 2019
/s/ J. Kenneth Glass J. Kenneth Glass	Director	March 18, 2019

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/s/ J. R. Hyde, III	Director	March 18, 2019
J. R. Hyde, III		
/s/ Garry A. Neil	Director	March 18, 2019
Garry A. Neil, M.D.		
/s/ Kenneth S. Robinson	Director	March 18, 2019
Kenneth S. Robinson, M.D.		

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GTx, Inc.

INDEX TO FINANCIAL STATEMENTS

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MANAGEMENT'S REPORT ON
INTERNAL CONTROL OVER FINANCIAL REPORTING

We, as management of GTx, Inc., are responsible for establishing and maintaining adequate internal control over financial reporting, as such term is defined in Securities Exchange Act Rule 13a-15(f). Internal control over financial reporting is a process designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with United States generally accepted accounting principles. Any system of internal control, no matter how well designed, has inherent limitations, including the possibility that a control can be circumvented or overridden and misstatements due to error or fraud may occur and not be detected. Also, because of changes in conditions, internal control effectiveness may vary over time. Accordingly, even an effective system of internal control will provide only reasonable assurance that the objectives of the internal control system are met.

Under the supervision and with the participation of management, including our principal executive officer and principal financial officer, we conducted an evaluation of the effectiveness of our internal control over financial reporting as of December 31, 2018 using the criteria for effective internal control over financial reporting as described in "Internal Control Integrated Framework," issued by the Committee of Sponsoring Organizations of the Treadway Commission (2013 framework). Based on this evaluation, we concluded that, as of December 31, 2018, our internal control over financial reporting was effective. The effectiveness of our internal control over financial reporting has been audited by Ernst & Young LLP, independent registered public accounting firm who also audited the Company's financial statements included in this Annual Report on Form 10-K. Ernst & Young LLP's report on the Company's internal control over financial reporting is included in this Annual Report on the 10-K.

/s/ Marc S. Hanover	/s/ Jason T. Shackelford
Marc S. Hanover Chief Executive Officer Principal Executive Officer Memphis, Tennessee	**Jason T. Shackelford** Vice President, Finance and Accounting Principal Financial and Accounting Officer

March 18, 2019

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Report of Independent Registered Public Accounting Firm

The Board of Directors and Stockholders of GTx, Inc.

Opinion on Internal Control over Financial Reporting

We have audited GTx, Inc.'s internal control over financial reporting as of December 31, 2018, based on criteria established in Internal Control Integrated Framework issued by the Committee of Sponsoring Organizations of the Treadway Commission (2013 framework), (the COSO criteria). In our opinion, GTx, Inc. (the Company) maintained, in all material respects, effective internal control over financial reporting as of December 31, 2018, based on the COSO criteria.

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States) (PCAOB), the balance sheets of the Company as of December 31, 2018 and 2017, the related statements of operations, stockholders' equity and cash flows for each of the three years in the period ended December 31, 2018, and the related notes and our report dated March 18, 2019 expressed an unqualified opinion thereon.

Basis for Opinion

The Company's management is responsible for maintaining effective internal control over financial reporting and for its assessment of the effectiveness of internal control over financial reporting included in the accompanying Management's Report on Internal Control Over Financial Reporting. Our responsibility is to express an opinion on the Company's internal control over financial reporting based on our audit. We are a public accounting firm registered with the PCAOB and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audit in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether effective internal control over financial reporting was maintained in all material respects.

Our audit included obtaining an understanding of internal control over financial reporting, assessing the risk that a material weakness exists, testing and evaluating the design and operating effectiveness of internal control based on the assessed risk, and performing such other procedures as we considered necessary in the circumstances. We believe that our audit provides a reasonable basis for our opinion.

Definition and Limitations of Internal Control Over Financial Reporting

A company's internal control over financial reporting is a process designed 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. A company's internal control over financial reporting includes those policies and procedures that (1) pertain to the maintenance of records that, in reasonable detail, accurately and fairly reflect the transactions and dispositions of the assets of the company; (2) provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that receipts and expenditures of the company are being made only in accordance with authorizations of management and directors of the company; and (3) provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use, or disposition of the company's assets that could have a material effect on the financial statements.

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Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Also, projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate.

/s/ Ernst & Young LLP

Memphis, Tennessee
March 18, 2019

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Report of Independent Registered Public Accounting Firm

The Board of Directors and Stockholders of GTx, Inc.

Opinion on the Financial Statements

We have audited the accompanying balance sheets of GTx, Inc. as of December 31, 2018 and 2017, the related statements of operations, stockholders' equity and cash flows for each of the three years in the period ended December 31, 2018, and the related notes (collectively referred to as the "financial statements"). In our opinion, the financial statements present fairly, in all material respects, the financial position of GTx, Inc. at December 31, 2018 and 2017, and the results of its operations and its cash flows for each of the three years in the period ended December 31, 2018, in conformity with U.S. generally accepted accounting principles.

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States) (PCAOB), the Company's internal control over financial reporting as of December 31, 2018, based on criteria established in Internal Control-Integrated Framework issued by the Committee of Sponsoring Organizations of the Treadway Commission (2013 framework) and our report dated March 18, 2019 expressed an unqualified opinion thereon.

Basis for Opinion

These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's financial statements based on our audits. We are a public accounting firm registered with the PCAOB and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. Our audits included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audits provide a reasonable basis for our opinion.

/s/ Ernst & Young LLP

We have served as the Company's auditor since 1998.

Memphis, Tennessee
March 18, 2019

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

	December 31, 2018	December 31, 2017
ASSETS		
Current assets:		
Cash and cash equivalents	$ 28,258	$ 15,816
Short-term investments	200	28,083
Prepaid expenses and other current assets	2,750	2,178
Total current assets	31,208	46,077
Property and equipment, net	19	51
Intangible assets, net	94	108
Total assets	$ 31,321	$ 46,236
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	$ 3,279	$ 2,604
Accrued expenses and other current liabilities	1,931	5,371
Total current liabilities	5,210	7,975
Commitments and contingencies		
Stockholders' equity:		
Common stock, $0.001 par value: 60,000,000 shares authorized at December 31, 2018 and December 31, 2017; 24,051,844 and 21,541,909 shares issued and outstanding at December 31, 2018 and December 31, 2017, respectively	24	22
Additional paid-in capital	626,142	599,876
Accumulated deficit	(600,055)	(561,637)
Total stockholders' equity	26,111	38,261
Total liabilities and stockholders' equity	$ 31,321	$ 46,236

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

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

	Years Ended December 31,		
	2018	**2017**	**2016**
Expenses:			
Research and development expenses	$ 29,669	$ 21,467	$ 17,228
General and administrative expenses	9,390	9,188	8,705
Total expenses	39,059	30,655	25,933
Loss from operations	(39,059)	(30,655)	(25,933)
Other income, net	641	216	46
Gain on change in fair value of warrant liability	-	-	8,163
Net loss	$ (38,418)	$ (30,439)	$ (17,724)
Net loss per share:			
Basic and Diluted	$ (1.65)	$ (1.75)	$ (1.22)
Weighted average shares outstanding:			
Basic and Diluted	23,346,231	17,441,280	14,559,541

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

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GTx, Inc.
STATEMENTS OF STOCKHOLDERS' EQUITY
For the Years Ended December 31, 2018, 2017 and 2016
(in thousands, except share data)

	Stockholders' Equity				
	Common Stock		Additional Paid-in Capital	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount			
Balances at January 1, 2016	14,037,411	$ 14	$ 515,319	$ (513,474)	$ 1,859
Issuance of common stock in October 2016 registered direct offering, net of offering costs	1,728,395	2	13,690	-	13,692
Vesting of restricted stock units, net of shares withheld for tax payments	154,170	-	(208)	-	(208)
Directors' deferred compensation	-	-	132	-	132
Share-based compensation	-	-	2,957	-	2,957
Warrant liability reclassification	-	-	19,186	-	19,186
Settlement of fractional shares upon reverse stock split	(404)	-	(3)	-	(3)
Net loss	-	-	-	(17,724)	(17,724)
Balances at December 31, 2016	15,919,572	16	551,073	(531,198)	19,891
Issuance of common stock and warrants in September 2017 private placement, net of offering costs	5,483,320	6	45,642	-	45,648
Vesting of restricted stock units, net of shares withheld for tax payments	139,017	-	(156)	-	(156)
Directors' deferred compensation	-	-	166	-	166
Share-based compensation	-	-	3,151	-	3,151
Net loss	-	-	-	(30,439)	(30,439)
Balances at December 31, 2017	21,541,909	22	599,876	(561,637)	38,261
Issuance of common stock upon exercise of warrants	674,579	1	(1)	-	-
Exercise of stock options	6,000	-	103	-	103
Issuance of shares under "At-The-Market" sales agreement, net of issuance costs	1,501,501	1	24,473	-	24,474
Vesting of restricted stock units, net of shares withheld for tax payments	327,855	-	(672)	-	(672)
Directors' deferred compensation	-	-	166	-	166
Share-based compensation	-	-	2,197	-	2,197
Net loss	-	-	-	(38,418)	(38,418)
Balances at December 31, 2018	24,051,844	$ 24	$ 626,142	$ (600,055)	$ 26,111

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

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

	Years Ended December 31,		
	2018	**2017**	**2016**
Cash flows from operating activities:			
Net loss	$ (38,418)	$ (30,439)	$ (17,724)
Adjustments to reconcile net loss to net cash used in operating activities:			
Gain on change in fair value of warrant liability	-	-	(8,163)
Share-based compensation	2,197	3,151	2,957
Directors' deferred compensation	166	166	132
Depreciation and amortization	46	47	28
Changes in assets and liabilities:			
Prepaid expenses and other assets	(572)	251	204
Accounts payable	675	1,384	838
Accrued expenses and other liabilities	(3,440)	1,980	950
Net cash used in operating activities	(39,346)	(23,460)	(20,778)
Cash flows from investing activities:			
Purchase of property and equipment	-	(2)	(90)
Purchase of short-term investments, held to maturity	(44,155)	(39,283)	(35,404)
Proceeds from maturities of short-term investments, held to maturity	72,038	24,159	37,645
Net cash provided by (used in) investing activities	27,883	(15,126)	2,151
Cash flows from financing activities:			
Net proceeds from the issuance of common stock and warrants	24,474	45,648	13,692
Tax payments related to shares withheld for vested restricted stock units	(672)	(156)	(208)
Proceeds from exercise of employee stock options	103	-	-
Settlement of fractional shares upon reverse stock split	-	-	(3)
Net cash provided by financing activities	23,905	45,492	13,481
Net increase (decrease) in cash and cash equivalents	12,442	6,906	(5,146)
Cash and cash equivalents, beginning of period	15,816	8,910	14,056
Cash and cash equivalents, end of period	$ 28,258	$ 15,816	$ 8,910

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

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

1. Business

GTx, Inc. ("GTx" or the "Company"), a Delaware corporation incorporated on September 24, 1997 and headquartered in Memphis, Tennessee, is a biopharmaceutical company dedicated to the discovery, development and commercialization of medicines to treat serious and/or significant unmet medical conditions.

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 or antagonize multiple forms of androgen receptor to treat those patients who do not respond or are resistant to current androgen targeted therapies by inhibiting tumor growth in patients with progressive castration-resistant prostate cancer ("CRPC"). The Company is in the process of completing ongoing mechanistic preclinical studies in order to select the most appropriate SARD compounds to move forward into the additional preclinical studies required to submit an investigational new drug application ("IND"), and potentially advance one of its SARD compounds into a first-in-human clinical trial.

The Company had been developing selective androgen receptor modulators ("SARMs"), including enobosarm (GTx-024). Most recently, enobosarm was evaluated in post-menopausal women with stress urinary incontinence ("SUI") compared to placebo. During the third quarter of 2018, the Company announced that the Phase 2 double-blind, placebo-controlled clinical trial of orally-administered enobosarm (3 mg or 1 mg) in post-menopausal women with SUI (the "ASTRID trial") did not achieve statistical significance on the primary endpoint for the trial. The Company has completed the ASTRID trial, including its review of the full data sets from the clinical trial, and has determined that there is not a sufficient path forward to warrant additional clinical development of enobosarm to treat SUI. The Company has therefore discontinued further development of enobosarm to treat SUI, including discontinuing the related durability and open-label safety extension studies that the Company initiated before it received topline data from the ASTRID trial. The Company has also discontinued any further development of its SARM program generally.

Following the announcement of the ASTRID trial results, the Company's board of directors commenced a process of evaluating strategic alternatives to maximize stockholder value. To assist with this process, the Company's board of directors engaged a financial advisory firm to help explore the Company's available strategic alternatives, including possible mergers and business combinations, a sale of part or all of the Company's assets, and collaboration and licensing arrangements. On March 6, 2019, the Company and Oncternal Therapeutics Inc. ("Oncternal") announced the signing of an Agreement and Plan of Merger and Reorganization (the "Merger Agreement"). Upon the terms and subject to the satisfaction of the conditions described in the Merger Agreement, including approval of the transaction by the Company's stockholders and Oncternal's stockholders, a wholly-owned subsidiary of the Company will be merged with and into Oncternal (the "Merger"), with Oncternal surviving the Merger as a wholly-owned subsidiary of the Company. See Note 2, Significant Accounting Policies Subsequent Events, for further discussion regarding the proposed Merger.

At December 31, 2018, the Company had cash, cash equivalents and short-term investments of $28,458 compared to $43,899 at December 31, 2017. To conserve its cash resources, the Company has substantially reduced its workforce since November 2018 and has ceased its SARM development

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activities and all other operations except for day-to-day business operations, completing ongoing mechanistic SARD preclinical studies and those activities necessary to complete the proposed Merger.

2. Significant Accounting Policies

Basis of Presentation

The accompanying financial statements have been prepared in accordance with U.S. generally accepted accounting principles ("U.S. GAAP"). Additionally, GTx operates in one business segment.

Use of Estimates

The preparation of financial statements in conformity with U.S. GAAP 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 financial statements, and the reported amounts of revenues and expenses during the reporting period. Actual amounts and results could differ from those estimates.

Cash and Cash Equivalents

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

Short-term Investments

At December 31, 2018 and 2017, short-term investments consisted of Federal Deposit Insurance Corporation ("FDIC") insured certificates of deposit with original maturities of greater than three months and less than one year.

Property and Equipment

Property and equipment is stated at cost. Amortization of leasehold improvements is recognized over the shorter of the estimated useful life of the leasehold improvement or the lease term. Depreciation is computed using the straight-line method over the estimated useful lives as follows:

Office equipment	3 to 5 years
Leasehold improvements	3 to 7 years
Furniture and fixtures	5 years
Computer equipment and software	3 years

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

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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 as of 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.

Fair Value of Financial Instruments and Warrant Liability

The carrying amounts of the Company's financial instruments (which include cash, cash equivalents, short-term investments, and accounts payable) and its prior warrant liability approximate their fair values. The fair value of the warrant liability was estimated using the Black-Scholes-Merton Model. See Note 6, Stockholders' Equity, for additional disclosure on the valuation methodology and significant assumptions. The Company's financial assets and liabilities are classified within a three-level fair value hierarchy that prioritizes the inputs used to measure fair value, which is defined as follows:

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

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.

Concentration of Risk

Financial instruments that potentially subject the Company to a concentration of credit risk consist of cash and cash equivalents and short-term investments. The Company has established guidelines relating to diversification and maturities of its cash equivalents and short-term investments which are designed to manage risk. The Company's cash and cash equivalents consist of bank deposits, certificates of deposit, and money market mutual funds. Bank deposits may at times be in excess of FDIC insurance limits. The Company's short-term investments consist of FDIC insured certificates of deposit with original maturities of greater than three months and less than one year.

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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.

Patent Costs

The Company expenses patent costs, including legal expenses, in the period in which they are incurred. Patent expenses are included in general and administrative expenses in the Company's statements of operations.

Income Taxes

The Company accounts for deferred taxes by recognition of deferred tax assets and liabilities for the expected future tax consequences of events that have been included in the financial statements or tax returns. Under this method, deferred tax assets and liabilities are determined based on the difference between the financial statement and tax basis of assets and liabilities using enacted tax rates in effect for the year in which the differences are expected to reverse. A valuation allowance is provided when it is more likely than not that some portion or all of the deferred tax assets will not be realized. Accordingly, at December 31, 2018 and December 31, 2017, net of the valuation allowance, the net deferred tax assets were reduced to zero. See Note 8, *Income Taxes*, for further discussion.

Share-Based Compensation

The Company has stock option and equity incentive plans that provide for the purchase or acquisition of the Company's common stock by certain of the Company's employees and non-employees. The Company recognizes compensation expense for its share-based payments based on the fair value of the awards over the period during which an employee or non-employee is required to provide service in exchange for the award. See Note 3, *Share-Based Compensation*, for further discussion.

Other Income (Expense), Net

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

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

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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 11,191,431, 9,438,236, and 8,162,347 were excluded from the calculation of diluted net loss per share for the years ended December 31, 2018, 2017 and 2016, respectively, as inclusion of the potential shares would have had an anti-dilutive effect on the net loss per share for the periods. At December 31, 2018, the Company had 24,051,844 shares of common stock outstanding.

Comprehensive Loss

For all periods presented, there were no differences between net loss and comprehensive loss.

Recent Accounting Pronouncements

In February 2016, the Financial Accounting Standards Board issued Accounting Standard Update ("ASU") 2016-02, *Leases (Topic 842).* This ASU requires that lessees recognize assets and liabilities on the balance sheet for the present value of the rights and obligations created by all leases with terms of more than 12 months. The ASU also will require disclosures designed to give financial statement users information on the amount, timing, and uncertainty of cash flows arising from leases. This new guidance will be effective for the Company as of January 1, 2019. The Company does not expect the adoption of the standard update to have a significant impact on its financial position or results of operations.

Subsequent Events

The Company has evaluated all events or transactions that occurred after December 31, 2018 up through the date the financial statements were issued. Other than as set forth below, there were no material recognizable or nonrecognizable subsequent events during the period evaluated.

Merger Agreement with Oncternal and Related Matters

Merger Agreement

On March 6, 2019, the Company entered into the Merger Agreement with Oncternal and Grizzly Merger Sub, Inc., a wholly-owned subsidiary of the Company ("Merger Sub"). Upon the terms and subject to the satisfaction of the conditions described in the Merger Agreement, including approval of the transaction by the Company's stockholders and Oncternal's stockholders, Merger Sub will be merged with and into Oncternal, with Oncternal surviving the Merger as a wholly-owned subsidiary of the Company.

Subject to the terms and conditions of the Merger Agreement, at the effective time of the Merger (the "Effective Time"): (i) each share of Oncternal common stock outstanding immediately prior to the Effective Time (excluding shares held by the Company, Merger Sub or Oncternal and dissenting shares) will be converted solely into the right to receive a number of shares of the Company's common stock (the "Shares") equal to the exchange ratio described below, (ii) each outstanding Oncternal stock option will be assumed by the Company, and (iii) each outstanding Oncternal warrant will be assumed by the Company.

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Under the exchange ratio formula in the Merger Agreement, the former Oncternal stockholders immediately before the Merger are expected to own approximately 75% of the outstanding capital stock of the Company, and the stockholders of the Company immediately before the Merger are expected to own approximately 25% of the outstanding capital stock of the Company, subject to certain assumptions. The exchange ratio formula excludes Oncternal's outstanding stock options and warrants and the Company's outstanding stock options and warrants.

Under certain circumstances further described in the Merger Agreement, the ownership percentages may be adjusted upward or downward based on cash levels of the respective companies at the closing of the Merger (the "Closing").

The Merger Agreement contains customary representations, warranties and covenants made by the Company and Oncternal, including covenants relating to obtaining the requisite approvals of the stockholders of the Company and Oncternal, indemnification of directors and officers, the Company's and Oncternal's conduct of their respective businesses between the date of signing of the Merger Agreement and the Closing. The Closing is subject to satisfaction or waiver of certain conditions included in the Merger Agreement.

Following the Closing, Oncternal's Chief Executive Officer, Chief Financial Officer, and Chief Operating Officer will serve in these positions for the Company. Additionally, following the Closing, the Company's board of directors will consist of nine directors, including two current GTx board members.

The Merger Agreement also includes termination provisions for both the Company and Oncternal. In connection with a termination of the Merger Agreement under specified circumstances, either party may be required to pay the other party a termination fee ranging between $500 to $2,000.

Contingent Value Rights Agreement

At the Effective Time, the Company will enter into a Contingent Value Rights Agreement (the "CVR Agreement"). Pursuant to the CVR Agreement, for each share of the Company's common stock held, the Company's stockholders of record as of immediately prior to the Effective Time will receive one contingent value right ("CVR") entitling such holders to receive in the aggregate 50% of any net proceeds received during the 15-year period after closing from the grant, sale or transfer of rights to the Company's SARD or SARM technology that occurs during the 10-year period after the Closing (or in the 11th year if based on a term sheet approved during the initial 10-year period) and, if applicable, to receive royalties on the sale of any SARD products by the combined company during the 15-year period after Closing. The CVR Agreement will be effective prior to the Closing and will continue in effect until the payment of all amounts payable thereunder, unless terminated upon termination of the Merger Agreement.

Workforce Reduction

In the first quarter of 2019, due to the entry into the Merger Agreement with Oncternal, the Company's board of directors committed to reducing its workforce by seven employees. All employees affected by the workforce reduction will be eligible to receive, among other things, specified severance payments based on the applicable employee's level and years of service with the Company and the continuation of group health insurance coverage. In addition, the affected employees will also be

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eligible for full vesting acceleration of their outstanding stock options as well as an extension of the post-termination exercise period for their outstanding stock options.

As a result of the workforce reduction and prior termination of three employees earlier in the first quarter of 2019, the Company estimates that it will incur total severance-related charges for these employees of approximately $1,000 in the first quarter of 2019 and up to an additional $500 contingent upon the closing of the Merger. The Company does not expect to record a non-cash charge related to the modification of outstanding stock options in connection with the workforce reduction.

Termination of Directors' Deferred Compensation Plan

Prior to the Effective Time (but in no event more than 30 days prior to the Effective Time), the Company's board of directors will take all actions necessary to terminate and liquidate the Company's 2018 Amended and Restated Directors' Deferred Compensation Plan (the "Directors' Deferred Compensation Plan") and all rights or other deferrals thereunder effective immediately prior to the Effective Time, subject to the consummation of the Merger. Any future board compensation under the Directors' Deferred Compensation Plan on or after January 3, 2019 shall be settled only in cash.

3. Share-Based Compensation

Share-based payments include stock option and RSU grants under the Company's stock option and equity incentive plans and deferred compensation arrangements for the Company's non-employee directors.

The Company has granted to employees and non-employees options to purchase common stock under various plans at prices equal to the fair market value of its common stock on the dates the options are granted as determined in accordance with the terms of the applicable plan. The options have a term of ten years from the grant date and generally vest over three years from the grant date for director and non-employee options and over periods of up to five years from the grant date for employee options. Under the terms of the Company's stock option and equity incentive plans, employees generally have three months after the employment relationship ends to exercise all vested options except in the case of voluntary retirement, disability or death, where post-termination exercise periods are generally longer. The Company issues new shares of common stock upon the exercise of options. The Company estimates the fair value of stock option awards as of the date of the grant by applying the Black-Scholes Model. The application of this valuation model involves assumptions that are judgmental and highly sensitive in the determination of compensation expense.

The fair value of each stock option is amortized into compensation expense on a straight-line basis between the grant date for the award and each vesting date. During 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 are now being 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 and prior periods have not been adjusted. The adoption of this guidance did not have a material impact on the Company's financial position or results of operations.

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Additionally, the Company periodically grants RSUs to its employees. The Company estimates the fair value of RSUs using the closing price of its common stock on the grant date. The fair value of the RSUs is amortized on a straight-line basis over the requisite service period of the awards. All RSUs were fully vested at December 31, 2018.

The following table summarizes share-based compensation expense included within the statements of operations for each of the three years in the period ended December 31, 2018:

	Years Ended December 31,		
	2018	2017	2016
Research and development expenses	$ 807	$ 1,171	$ 1,260
General and administrative expenses	1,556	2,146	1,829
Total share-based compensation	$ 2,363	$ 3,317	$ 3,089

Share-based compensation expense recorded in the statement of operations as general and administrative expense for the years ended December 31, 2018, 2017 and 2016 included share-based compensation expense related to deferred compensation arrangements for the Company's non-employee directors of $166, $166 and $132, respectively. See Note 9, *Directors' Deferred Compensation Plan*, for further discussion of deferred compensation arrangements for the Company's non-employee directors.

For the years ended December 31, 2018, 2017 and 2016, the weighted average grant date fair value per share of stock options granted was $10.36, $3.80 and $5.45, respectively. The key assumptions used in determining the grant date fair value of options granted in 2018, 2017 and 2016, and a summary of the methodology applied to develop each assumption is as follows:

	Years Ended December 31,		
	2018	2017	2016
Expected price volatility	93.1%	88.6%	91.3%
Risk-free interest rate	2.4%	2.2%	2.0%
Weighted average expected life in years	6.9 years	6.9 years	6.9 years
Dividend yield	0%	0%	0%

Expected Price Volatility This is a measure of the amount by which a price has fluctuated or is expected to fluctuate. The Company based its determination of expected volatility on its historical stock price volatility. An increase in the expected price volatility will increase compensation expense.

Risk-Free Interest Rate This is determined using U.S. Treasury rates where the term is consistent with the expected life of the stock options. An increase in the risk-free interest rate will increase compensation expense.

Expected Life This is the period of time over which the options granted are expected to remain outstanding and is determined by calculating the average of the vesting term and the contractual term of the options. The Company has utilized this method due to the lack of historical option exercise

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information related to the Company's stock option and equity incentive plans. Options granted have a maximum term of ten years. An increase in the expected life will increase compensation expense.

Dividend Yield The Company has not made any dividend payments nor does it have plans to pay dividends in the foreseeable future. An increase in the dividend yield will decrease compensation expense.

The following is a summary of stock option transactions for all of the Company's stock option and equity incentive plans for the three year period ended December 31, 2018:

	Number of Shares	Weighted Average Exercise Price Per Share
Options outstanding at January 1, 2016	798,309	$ 38.80
Options granted	363,500	6.94
Options forfeited or expired	(71,829)	54.65
Options exercised	-	-
Options outstanding at December 31, 2016	1,089,980	27.13
Options granted	977,350	4.97
Options forfeited or expired	(166,834)	48.71
Options exercised	-	-
Options outstanding at December 31, 2017	1,900,496	13.84
Options granted	472,000	13.32
Options forfeited or expired	(31,049)	168.76
Options exercised	(6,000)	17.23
Options outstanding and vested or expected to vest at December 31, 2018	2,335,447	$ 11.67

The following table summarizes information about stock options outstanding at December 31, 2018:

	Options Outstanding			Options Exercisable	
Exercise Price	Number Outstanding	Weighted Average Remaining Contractual Life (years)	Weighted Average Exercise Price	Number Exercisable	Weighted Average Exercise Price
$4.29 - $4.29	56,250	8.36	$ 4.29	18,750	$ 4.29
$4.71 - $4.71	825,000	8.02	4.71	22,500	4.71
$4.77 - $12.71	826,800	7.67	9.97	134,404	8.25
$12.95 - $108.90	627,397	4.20	23.71	508,691	25.59
	2,335,447	6.88	11.67	684,345	20.91

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At December 31, 2018, the aggregate intrinsic value of all outstanding options was zero with a weighted average remaining contractual term of 6.88 years. Of the Company's outstanding options, 684,345 options were exercisable and had a weighted average remaining contractual term of 4.05 years and no aggregate intrinsic value. Additionally, the Company's vested and expected to vest options had a weighted average remaining contractual term of 6.88 years and no aggregate intrinsic value.

Options to purchase 6,000 shares were exercised during the years ended December 31, 2018. The total intrinsic value of options exercised during the years ended December 31, 2018 was $39. At December 31, 2018, the total compensation cost related to non-vested options not yet recognized was $7,697, with a weighted average expense recognition period of 2.95 years. Shares available for future issuance under the Company's stock option and equity incentive plans were 1,167,162 at December 31, 2018. On January 1, 2019, shares available for future issuance under the 2013 equity incentive plan and the 2013 non-employee director equity incentive plan increased by an aggregate of 1,012,074 shares in accordance with the automatic increase provisions of such plans.

The following is a summary of the RSU transactions for all of the Company's equity incentive plans for the three year period ended December 31, 2018:

	Number of Shares
Nonvested RSUs outstanding at January 1, 2016	820,000
RSUs granted	11,000
RSUs vested	(184,001)
RSUs forfeited	(62,000)
Nonvested RSUs outstanding at December 31, 2016	584,999
RSUs granted	-
RSUs vested	(168,499)
RSUs forfeited	(36,000)
Nonvested RSUs outstanding at December 31, 2017	380,500
RSUs granted	-
RSUs vested	(380,500)
RSUs forfeited	-
Nonvested RSUs outstanding at December 31, 2018	-

The number of RSUs vested during 2018, 2017, and 2016 included 52,645, 29,482, and 29,829 shares, respectively, that were withheld on behalf of the Company's employees to satisfy the statutory tax withholding requirements.

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4. Property and Equipment, Net

Property and equipment, net consisted of the following:

	December 31,	
	2018	2017
Computer equipment and software	$ 1,225	$ 1,299
Furniture and fixtures	853	853
Leasehold improvements	355	355
Office equipment	211	211
	2,644	2,718
Less: accumulated depreciation	(2,625)	(2,667)
	$ 19	$ 51

Depreciation and amortization expense for the years ended December 31, 2018, 2017 and 2016 was $32, $32, and $14, respectively. Of these amounts, $1, $2 and $2, respectively, were included in research and development expenses in the statements of operations.

5. Accrued Expenses and Other Current Liabilities

Accrued expenses and other current liabilities consisted of the following:

	December 31,	
	2018	2017
Clinical trials	$ 1,492	$ 4,742
General and administrative	101	314
Research and development	272	312
Employee compensation	66	3
	$ 1,931	$ 5,371

6. Stockholders' Equity

Authorized Capital

On December 5, 2016, the Company filed a Certificate of Amendment to the Company's Restated Certificate of Incorporation with the Secretary of State of the State of Delaware to effect a one-for-ten reverse stock split of its outstanding common stock and to effect a reduction in the number of authorized shares of common stock from 400,000,000 to 60,000,000 shares. The Company's certificate of incorporation currently authorizes the Company to issue 60,000,000 shares of common stock, $0.001 par value per share, and 5,000,000 shares of preferred stock, $0.001 par value per share.

Common Stock

On February 9, 2018, the Company entered into an At-the-Market Equity Offering Sales Agreement (the "ATM Sales Agreement") with Stifel, Nicolaus & Company, Incorporated, as sales agent ("Stifel"), pursuant to which the Company may offer and sell, from time to time, through Stifel, shares of the Company's common stock, having an aggregate offering price of up to $50,000. On

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May 16, 2018, the Company sold 1,501,501 shares of common stock under the ATM Sales Agreement for net proceeds of $24,474. As of December 31, 2018, the Company had approximately $25,000 of common stock remaining available to be sold under the ATM Sales Agreement.

On September 29, 2017, the Company completed a private placement of units consisting of an aggregate of 5,483,320 shares of common stock and warrants to purchase an aggregate of 3,289,988 shares of its common stock for net proceeds of $45,648, after deducting placement agent fees and other offering expenses. The purchasers in the private placement consisted solely of accredited investors that included certain institutional and existing stockholders, including a member of the Company's board of directors. The warrants, which have five year terms expiring on September 29, 2022, are immediately exercisable and have a per share exercise price of $9.02. The Company assessed whether the warrants require accounting as derivatives. The Company determined that the warrants were indexed to the Company's own stock. As such, the Company has concluded the warrants meet the scope exception for determining whether the instruments require accounting as derivatives and are classified in stockholders' equity. The fair value of the warrants was estimated at $21,069 using the Black-Scholes Model with the following assumptions: expected volatility of 97%, risk free interest rate of 1.92%, expected life of five years and no dividends. The net proceeds from the private placement were allocated to the common stock and warrants based upon their relative fair values.

On October 14, 2016, the Company completed a registered direct offering of its common stock. Under the terms of the offering, the Company sold 1,728,395 shares of its common stock for net proceeds of $13,692, after deducting offering expenses.

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

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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. In March 2018, certain holders of warrants issued in November 2014 exercised warrants covering 1,111,082 shares of common stock in a cashless exercise for which the Company issued an aggregate of 674,579 shares of common stock upon exercise.

Each of these completed offerings included certain existing GTx stockholders and/or certain members of the GTx management team and/or board of directors.

7. License Agreements

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 has been 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.

In accordance with the terms of the SARM License Agreement that the Company entered into with UTRF in July 2007, the Company paid a one-time up-front fee of $290, which was recorded as an intangible asset by the Company. This intangible asset, net at December 31, 2018 and 2017 was $94 and $108, respectively.

The Company and UTRF also entered into a 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 (the "SARD License Agreement"). 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.

8. Income Taxes

The Tax Cuts and Jobs Act ("Tax Reform Act") was enacted on December 22, 2017. The Tax Reform Act significantly revised the U.S. corporate income tax regime by, among other things, lowering the U.S. corporate tax rate from 35% to 21% effective January 1, 2018. On December 22, 2017, the SEC staff issued Staff Accounting Bulletin No. 118 ("SAB 118") to address the application of U.S. GAAP in situations when a registrant does not have the necessary information available, prepared,

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or analyzed (including computations) in reasonable detail to complete the accounting for certain income tax effects of the Tax Reform Act. The Company recognized the provisional tax impacts related to the revaluation of deferred tax assets and liabilities and included these amounts in its financial statements for the year ended December 31, 2017. During 2018 the Company completed the accounting under the Tax Reform Act as allowed under SAB 118 to revalue its deferred tax assets and liabilities which resulted in no change to the amounts previously recorded by the Company for year ended December 31, 2017.

Deferred income taxes reflect the net tax effects of temporary differences between the carrying amounts of assets and liabilities for financial reporting purposes and the amounts used for income tax purposes. The principal components of the Company's net deferred income tax assets and liabilities consisted of the following:

	December 31,	
	2018	**2017**
Deferred income tax assets:		
Net federal and state operating loss carryforwards	$ 120,555	$ 110,145
Research and development credits	16,383	14,757
Share-based compensation	3,130	3,994
Depreciation and amortization	17	21
Total deferred tax assets	140,085	128,917
Deferred income tax liabilities:		
Other	461	92
Total deferred tax liabilities	461	92
Net deferred tax assets	139,624	128,825
Valuation allowance	(139,624)	(128,825)
	$ -	$ -

Realization of deferred income tax assets is dependent upon future earnings, if any, the timing and amount of which are uncertain. Accordingly, due to the Company's history of net operating losses, the net deferred tax assets have been fully offset by a valuation allowance. The valuation allowance increased by $10,799 in 2018, decreased by $47,132 in 2017 and increased in 2016 by $9,347. The valuation allowance decrease in 2017 was due primarily to the passage of the Tax Reform Act and the reduction in the valuation of the Company's net deferred tax assets as a result of the lowering of the corporate tax rate from 35% to 21% effective January 1, 2018.

At December 31, 2018, the Company had net federal operating loss carryforwards of approximately $472,054. The federal operating loss carryforwards originating prior to 2018 will expire from 2019 to 2037 if not utilized. The Company had state operating loss carryforwards of approximately $411,396, which expire from 2019 to 2038 if not utilized. The Company also had research and development credits at December 31, 2018 of approximately $16,383, which expire from 2020 to 2038 if not utilized.

The Company will recognize the impact of a tax position in the financial statements if that position is more likely than not of being sustained on audit based on the technical merits of the position. As of

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December 31, 2018, the Company had no unrecognized tax benefits. Utilization of the Company's net operating loss carryforwards may be subject to a substantial annual limitation due to ownership change limitations provided by Section 382 of the Internal Revenue Code of 1986, as amended, and similar state provisions. The annual limitations may result in the expiration of net operating loss carryforwards before utilization. The Company completed a study of its net operating losses through December 31, 2016 to determine whether such amounts are likely to be limited by Section 382. As a result of this study and its analysis of subsequent ownership changes, the Company does not currently believe any Section 382 limitation exists through December 31, 2018 though the Company has not yet conducted an in-depth analysis since the last study. However, any future ownership changes under Section 382 may limit the Company's ability to fully utilize these tax benefits. The Company has not yet conducted an in-depth study of its research and development credits, although the Company periodically reviews assumptions used in its calculations to reflect its best estimate of expected credit. An in-depth study may result in an increase or decrease to the Company's research and development credits and until such study is conducted of the Company's research and development credits, no amounts are being presented as an uncertain tax position. The Company's net deferred income tax assets have been fully offset by a valuation allowance. Therefore, future changes to the Company's unrecognized tax benefits would be offset by an adjustment to the valuation allowance and there would be no impact on the Company's balance sheet, statement of operations, or cash flows. The Company does not expect its unrecognized tax benefits to change significantly over the next 12 months.

The Company is currently open to audit under the statute of limitations by the Internal Revenue Service and the appropriate state income taxing authorities for all years due to the net loss carryforwards from those years. The Company is currently not under examination by the Internal Revenue Service or any other taxing authorities. The Company has not recorded any interest and penalties on any unrecognized tax benefits since its inception.

9. Directors' Deferred Compensation Plan

Non-employee directors may defer all or a portion of their fees under the Company's Directors' Deferred Compensation Plan until termination of their status as directors. Deferrals can be made into a cash account, a stock account, or a combination of both. Stock accounts will be paid out in the form of Company common stock, except that any fractional shares will be paid out in cash valued at the then current market price of the Company's common stock. Cash accounts and stock accounts under the Directors' Deferred Compensation Plan are credited with interest or the value of any cash and stock dividends, respectively. Non-employee directors are fully vested in any amounts that they elect to defer under the Directors' Deferred Compensation Plan.

For the years ended December 31, 2018, 2017 and 2016, the Company incurred non-employee director fee expense of $291, $291 and $257, respectively, of which $166, $166 and $132 was deferred into stock accounts and will be paid in common stock following separation from service as a director. At December 31, 2018, 122,725 shares of the Company's common stock had been credited to individual director stock accounts under the Directors' Deferred Compensation Plan, and no amounts had been credited to individual director cash accounts under the Directors' Deferred Compensation Plan.

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10. 401(k) Plan

The Company sponsors a 401(k) retirement savings plan that is available to all eligible employees. The plan is intended to qualify under Section 401(k) of the Internal Revenue Code of 1986, as amended. The plan provides that each participant may contribute up to a statutory limit of their pre-tax compensation which was $18.5 for employees under age 50 and $24.5 for employees 50 and older in calendar year 2018. Employee contributions are held in the employees' name and invested by the plan's trustee. The plan also permits the Company to make matching contributions, subject to established limits. The Company elected to match a portion of employee's contributions to the plan in the amount of $185, $186 and $200 in 2018, 2017 and 2016, respectively.

11. Commitments and Contingencies

Operating Lease Commitments

In 2015, the Company entered into a new office lease with respect to the Company's current office space. The new office lease term commenced on May 1, 2015 with a three year term ending on April 30, 2018, with an option to extend the lease for an additional three years, and was accounted for as an operating lease. In March 2018, the Company amended the lease to extend the term of the lease for an additional 12-month term expiring on April 30, 2019. Total rent expense under the operating leases was approximately $509, $506 and $495 for the years ended December 31, 2018, 2017 and 2016, respectively. As of December 31, 2018, future annual minimum payments under operating lease arrangements were $162.

12. Quarterly Financial Data (Unaudited) [1]

The following is a summary of the quarterly results of operations for the years ended December 31, 2018 and 2017:

	2018 Quarters Ended			
	March 31	**June 30**	**September 30**	**December 31**
Expenses:				
Research and development expenses	$ 11,000	$ 7,962	$ 7,467	$ 3,240
General and administrative expenses	2,688	2,196	2,160	2,346
Total expenses	13,688	10,158	9,627	5,586
Loss from operations	(13,688)	(10,158)	(9,627)	(5,586)
Other income, net	131	143	196	171
Net loss	$ (13,557)	$ (10,015)	$ (9,431)	$ (5,415)
Net loss per share:				
Basic and Diluted	$ (0.62)	$ (0.43)	$ (0.39)	$ (0.23)
Weighted average shares outstanding:				
Basic and Diluted	21,967,805	23,288,691	24,045,992	24,051,844

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	2017 Quarters Ended			
	March 31	**June 30**	**September 30**	**December 31**
Expenses:				
Research and development expenses	$ 4,193	$ 4,448	$ 5,914	$ 6,912
General and administrative expenses	2,087	1,997	2,617	2,487
Total expenses	6,280	6,445	8,531	9,399
Loss from operations	(6,280)	(6,445)	(8,531)	(9,399)
Other income, net	27	40	27	122
Net loss	$ (6,253)	$ (6,405)	$ (8,504)	$ (9,277)
Net loss per share:				
Basic and Diluted	$ (0.39)	$ (0.40)	$ (0.53)	$ (0.43)
Weighted average shares outstanding:				
Basic and Diluted	16,018,342	16,041,923	16,115,835	21,541,909

(1) The sum of quarterly earnings per share amounts may not equal the annual amounts as the quarterly amounts are computed independently for each quarter while the full year is based on the annual weighted average shares outstanding.