Fibrocell Science, Inc.
Form 10-K
March 27, 2019

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

FORM 10-K

ý 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
¨ Transition Report Pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934

Fibrocell Science, Inc.
(Exact name of registrant as specified in its Charter.)

Delaware	001-31564	87-0458888
(State or other jurisdiction of incorporation)	(Commission File Number)	(I.R.S. Employer Identification No.)

405 Eagleview Boulevard
Exton, Pennsylvania 19341
(Address of principal executive offices, including zip code)
(484) 713-6000
(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, $.001 par value	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, as amended, 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 (§ 229.405) 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	¨	Accelerated filer	¨
Non-accelerated filer	x	Smaller reporting company	x
		Emerging growth company	¨

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. ¨

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

The aggregate market value of the registrant's common stock held by non-affiliates was $16.8 million as of June 29, 2018 (the last business day of the registrant's most recently completed second fiscal quarter), based on a total of 6,185,684 shares of common stock held by non-affiliates and on a closing price of $2.71 as reported on the Nasdaq Capital Market on June 29, 2018.

As of March 20, 2019, there were 9,758,332 shares of the registrant's common stock, par value $0.001 per share, outstanding.

DOCUMENTS INCORPORATED BY REFERENCE

Portions of the registrant's definitive proxy statement for its 2019 annual meeting of stockholders are incorporated by reference into Part III of this Form 10-K where indicated. Such definitive proxy statement will be filed with the U.S. Securities and Exchange Commission within 120 days after the year ended December 31, 2018.

TABLE OF CONTENTS

Unless the context otherwise indicates, references in this Annual Report on Form 10-K to "Fibrocell," "the Company," "we," "us" and "our" refer to Fibrocell Science, Inc. and its subsidiaries.

Fibrocell, Fibrocell Science and LAVIV® are trademarks of Fibrocell. Other trademarks, trade names and service marks appearing in this Annual Report on Form 10-K are the property of their respective owners.

Unless otherwise indicated, all share amounts and the exercise or conversion price of any of our securities reflect, as applicable, the occurrence of a 1-for-25 reverse split of our common stock that occurred on April 30, 2013, the occurrence of a 1-for-3 reverse split of our common stock that occurred on March 10, 2017 and the occurrence of a 1-for-5 reverse split of our common stock that occurred on May 24, 2018.

Table of Contents

NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Annual Report on Form 10-K (this Form 10-K) contains “forward-looking statements” within the meaning of the Private Securities Litigation Reform Act of 1995. These statements include, among others, statements about:

- our review of strategic alternatives, including the possible sale or merger of our company;
- our expectation that our existing cash resources will be sufficient to enable us to fund our operations into the fourth quarter of 2019;
- our future expenses and capital expenditures;
- our estimates regarding expenses, future revenues, capital requirements and needs for, and ability to obtain, additional financing;
- our plans to address our future capital requirements and the consequences of failing to do so;
- our plans to initiate a Phase 3 clinical trial for FCX-007 in the second quarter of 2019;
- our expectations regarding the clinical trial design of the Phase 3 clinical trial for FCX-007, including our expectation to enroll 15-20 patients;
- our plans to complete enrollment of Phase 1 adult patients in a Phase 1/2 clinical trial for FCX-013 in the third quarter of 2019;
- our product development goals under our collaborations with Intrexon Corporation for our product candidates;
- the potential benefits of Fast Track, Orphan Drug and Rare Pediatric Disease designations;
- the potential advantages of our product candidates and technologies; and
- the effect of legal and regulatory developments;

as well as other statements relating to our future operations, financial performance or financial condition, prospects or other future events. Forward-looking statements appear primarily in the sections of this Form 10-K entitled “Item 1—Business,” “Item 1A—Risk Factors,” “Item 7—Management’s Discussion and Analysis of Financial Condition and Results Operations,” “Item 7A—Quantitative and Qualitative Disclosures About Market Risk,” and “Item 8—Financial Statements and Supplementary Data.” In some cases, you can identify forward-looking statements by words such as “may,” “will,” “could,” “would,” “should,” “expect,” “intend,” “plan,” “anticipate,” “believe,” “estimate,” “predict,” “project,” “potential,” “continue,” “scheduled” and similar expressions, although not all forward-looking statements contain these identifying words.

Forward-looking statements are based upon current expectations and assumptions and are subject to a number of known and unknown risks, uncertainties and other factors that could cause actual results to differ materially and adversely from those expressed or implied by such statements. Factors that could cause or contribute to such differences include, but are not limited to, those discussed in this Form 10-K and in particular the risks and uncertainties discussed under "Item 1A-Risk Factors" of this Form 10-K. As a result, you should not place undue reliance on forward-looking statements.

Additionally, the forward-looking statements contained in this Form 10-K represent our views only as of the date of this Form 10-K (or any earlier date indicated in such statement). While we may update certain forward-looking

statements from time to time, we specifically disclaim any obligation to do so, even if new information becomes available in the future. However, you are advised to consult any further disclosures we make on related subjects in the periodic and current reports that we file with the Securities and Exchange Commission.

The foregoing cautionary statements are intended to qualify all forward-looking statements wherever they may appear in this Form 10-K. For all forward-looking statements, we claim protection of the safe harbor for the forward-looking statements contained in the Private Securities Litigation Reform Act of 1995.

This Form 10-K also contains estimates, projections and other information concerning our industry, our business, and the markets for certain diseases, including data regarding the estimated size of those markets, and the incidence and prevalence of certain medical conditions. Information that is based on estimates, forecasts, projections, market research or similar

Table of Contents

methodologies is inherently subject to uncertainties and actual events or circumstances may differ materially from events and circumstances reflected in this information. Unless otherwise expressly stated, we obtained this industry, business, market and other data from reports, research surveys, studies and similar data prepared by market research firms and other third parties, industry, medical and general publications, government data and similar sources.

Table of Contents

Part I
Item 1. Business
Overview

We are an autologous cell and gene therapy company focused on translating personalized biologics into medical breakthroughs for diseases affecting the skin and connective tissue. Our distinctive approach to personalized biologics is based on our proprietary autologous fibroblast technology. Fibroblasts are the most common cell in skin and connective tissue and are responsible for synthesizing extracellular matrix proteins, including collagen and other growth factors, that provide structure and support. Because fibroblasts naturally reside in the localized environment of the skin and connective tissue, they represent an ideal delivery vehicle for proteins targeted to these areas. We target the underlying cause of disease by using fibroblast cells from a patient's skin and genetically modifying them to create localized therapies that are compatible with the unique biology of the patient (i.e., which are autologous).

We are focused on discovering and developing localized therapies for diseases affecting the skin and connective tissue, where there are high unmet needs, to improve the lives of patients and their families. In that regard, we commit significant resources to our research and development programs. Currently, all of our research and development operations and focus are on gaining regulatory approvals to commercialize our product candidates in the United States; however, we may seek to expand into international markets in the future.

Our current pipeline consists of the following product candidates which we are developing in collaboration with Intrexon Corporation (Intrexon):

Our most advanced product candidate, FCX-007, is currently in a Phase 1/2 clinical trial for the treatment of recessive dystrophic epidermolysis bullosa (RDEB). In May 2018, we reported on interim adult data and provided a Phase 1 trial update which included presenting at the 7th International Investigative Dermatology meeting on May 19, 2018. We completed the targeted enrollment of six patients ages seven and older in the Phase 2 portion of the Phase 1/2 clinical trial, and have over-enrolled by one patient for a total of seven patients. In addition, we completed a Type C meeting with the FDA to discuss the design of a Phase 3 clinical trial protocol for FCX-007, and we received the official minutes from this meeting with the FDA in November 2018. Based on FDA's feedback, we prepared a Phase 3 clinical trial protocol for FCX-007 and filed it as part of the briefing package for the Type B meeting. In March 2019, we completed a Type B end-of-Phase 2 face-to-face meeting with the FDA to discuss the design of a Phase 3 clinical trial for FCX-007 to support a Biologics License Application (BLA) filing. Furthermore, we reported additional positive safety and wound healing data for our ongoing Phase 1/2 trial. We plan to initiate a Phase 3 clinical trial for FCX-007 in the second quarter of 2019.

Our second gene therapy product candidate, FCX-013, is in clinical development for the treatment of moderate to severe localized scleroderma. We submitted an investigational new drug (IND) application for FCX-013 to the United States Food and Drug Administration (FDA) in January 2018 and in March 2018, the FDA allowed the IND to progress to clinical trials. We initiated the first investigator site for clinical enrollment for an open label, single arm Phase 1/2 clinical trial, and we

Table of Contents

are currently enrolling the Phase 1 portion of a Phase 1/2 trial for FCX-013. We expect to complete enrollment of Phase 1 adult patients in the third quarter of 2019. In addition, we have a third program in the research phase for the treatment of arthritis and related conditions. See further discussion of our gene therapy product candidates under the heading “Development Programs” included within section “Item 1—Business” of this Form 10-K.

Table of Contents

Our Strategy

Our strategy is to develop and commercialize transformational therapies for diseases affecting the skin and connective tissue to improve the lives of patients and their families. Key elements of our strategy are:

- Leveraging our proprietary autologous fibroblast technology and patented manufacturing process;
- Advancing our clinical stage gene therapy product candidates, FCX-007 and FCX-013, through human clinical trials;
- Advancing our research stage gene therapy program focused on arthritis and related conditions through research and into pre-clinical development; and
- Leveraging our FDA-compliant current Good Manufacturing Practices (cGMP) manufacturing facility and our expertise in cell therapy manufacturing to advance the development of our autologous cell and gene therapy pipeline.

Our Platform

Our proprietary autologous fibroblast technology is the foundation for creating personalized biologics for diseases of the skin and connective tissue. This technology uses a patented manufacturing process, which involves collecting small skin biopsies from patients, isolating cells and expanding them in culture, transducing the fibroblast cells with an integrative lentiviral vector to express a targeted protein, followed by continued expansion of the gene modified cells in culture. In this manner, each patient is treated with cells that were cultivated from his or her own dermal tissue (i.e., autologous).

The Science of Autologous Fibroblasts

Fibroblasts are the basis of our personalized biologics platform because they are the most common cell in skin and connective tissue and are responsible for synthesizing extracellular matrix proteins, including collagen and other growth factors, that provide structure and support.

Personalized Biologics Approach

Because fibroblasts naturally reside in the localized environment of the skin and connective tissue, they represent an ideal delivery vehicle for proteins targeted to these areas. Utilizing our proprietary autologous fibroblast technology, we use a patient’s fibroblast cells to create localized gene therapies that are compatible with the unique biology of the patient and have the potential to address the underlying cause of disease.

Table of Contents

We believe our personalized biologics approach provides the following distinct advantages for creating gene therapies:

- Localized administration—avoids side effects typically associated with systemic therapy
- Reduced rejection and immunogenicity concerns—because autologous fibroblasts are compatible with the unique biology of each patient
- Fibroblast cells are genetically modified ex vivo—to enable testing for safety and confirmation of protein expression levels prior to administration to the patient
- Demonstrated expertise in manufacturing our fibroblast cell therapy

We are developing all of our gene therapy product candidates in collaboration with Intrexon, a leader in synthetic biology. Through our collaboration with Intrexon, we have access to:

- Intrexon's proprietary vector technology, which is designed to facilitate the assembly and delivery of the necessary target gene constructs for delivery to autologous fibroblast cells. Access to this technology allows us to rapidly screen and construct genetic therapeutic solutions.
- Intrexon's proprietary RheoSwitch Therapeutic System® (RTS®) technology. The RTS® biologic switch is activated by an orally-administered compound (Veledimex) to control level and timing of protein expression in those diseases where such control is ideal.

Table of Contents

Development Programs

Our development programs are focused on diseases affecting the skin and connective tissue for which there are high unmet needs. Our programs consist of the following:

Program	Potential Indication	Status
FCX-007	RDEB	Phase 1/2
FCX-013	Moderate to severe localized scleroderma	Phase 1/2
Research Program	Arthritis	Research

FCX-007 for RDEB

RDEB is the most severe form of dystrophic epidermolysis bullosa (DEB), a congenital, progressive, devastatingly painful and debilitating genetic disorder that often leads to death. RDEB is caused by a mutation of the COL7A1 gene, the gene which encodes for type VII collagen (COL7), a protein that forms anchoring fibrils. Anchoring fibrils hold together the layers of skin, and without them, skin layers separate causing severe blistering, open wounds and scarring in response to friction, including normal daily activities like rubbing or scratching. Children who inherit this condition are often called “butterfly children” because their skin can be as fragile as a butterfly’s wings. We estimate that there are approximately 1,100 - 2,500 RDEB patients in the U.S. Currently, treatments for RDEB address only the sequelae, including daily bandaging (which can cost a patient in excess of $10,000 per month), hydrogel dressings, antibiotics, feeding tubes and surgeries.

Our lead product candidate, FCX-007, is in clinical development for the treatment of RDEB. FCX-007 is a genetically-modified autologous fibroblast that encodes the gene for COL7 for localized treatment of RDEB and is being developed in collaboration with Intrexon. By genetically modifying autologous fibroblasts ex vivo to produce COL7, culturing them and then treating blisters and wounds locally via injection, FCX-007 offers the potential to address the underlying cause of the disease by providing high levels of COL7 directly to the affected areas, thereby avoiding systemic treatment. In addition, we believe the autologous nature of the cells, localized delivery, use of an integrative vector and the low turnover rate of the protein will contribute to long-term persistence of the COL7 produced by FCX-007.

FCX-007 has received Orphan Drug Designation for the treatment of DEB, including RDEB, Rare Pediatric Disease Designation for the treatment of RDEB and Fast Track Designation for the treatment of RDEB from the FDA.

In September 2018, the FDA’s Office of Orphan Products Development (OOPD) awarded us a $1.4 million clinical trial research grant for our continued development of FCX-007. This grant, which will be distributed over a four year period ending in August 2022, was awarded by the FDA through the OOPD’s Orphan Products Clinical Trials Grants Program. This program supports the clinical development of products for use in rare diseases or conditions for which “no current therapy exists or where the proposed product will be superior to the existing therapy”.

In December 2018, we received a $900,000 investment from EB Research Partnership, Inc. (EBRP) and Epidermolysis Bullosa Medical Research Foundation (EBMRF) to help further the progress of FCX-007. Under the terms of the investment,

Table of Contents

EBRP and EBMRF received an aggregate of 443,350 shares of our common stock. We received this funding following a competitive application and evaluation process managed by EBRP's Scientific Advisory Board (SAB), a prominent panel of physicians and scientists specializing in genetics, hematology, protein therapy and dermatology. The SAB awards research grants to products and therapies that offer notable commercial promise for treating and/or curing epidermolysis bullosa (EB).

Phase 1/2 Trial of FCX-007 for RDEB

The primary objective of this open-label trial is to evaluate the safety of FCX-007 in RDEB patients. Additionally, the trial will assess (i) the pharmacology of FCX-007 through the presence of vector DNA or COL7 mRNA evaluation of COL7 expression and/or the presence of anchoring fibrils and (ii) the efficacy of FCX-007 through intra-subject paired analysis of target wound areas by comparing FCX-007 treated wounds to untreated wounds in Phase 1 and to wounds administered with sterile saline in Phase 2 through the evaluation of digital imaging of wounds. Prior to treating pediatric patients in this trial, we were required to and obtained allowance from the FDA by submitting evidence of FCX-007 safety and benefit in the adult patients and data from our completed pre-clinical toxicology study. After submission of the requested data, the FDA granted allowance to include pediatric patients in the clinical trial in January 2018.

In May 2018, we reported on interim adult data and provided a Phase 1 trial update which included presenting at the 7th International Investigative Dermatology meeting on May 19, 2018. We reported that four adult RDEB patients (n=7 wounds) aged 20 to 37 were dosed with FCX-007 in the margins of and across targeted wounds, as well as in separate intact skin sites. Three of these patients received a single intradermal injection session at baseline. One of these patients received a second injection session in the remaining unhealed areas of wounds at 25 weeks post-administration, as allowed by the clinical trial protocol.

Safety data from these patients show FCX-007 was well-tolerated up to 52 weeks post-administration. There were no serious adverse events (SAEs) and no product related adverse events reported. No COL7 autoantibody response was noted. Various COL7 expression signals were detected throughout the data set using either immunofluorescence (IF) or immunoelectron microscopy (IEM) up to 52 weeks post-administration. Anchoring fibril structures were also observed using IEM.

Wounds were evaluated during a monitoring period prior to dosing and they were observed to be open for up to eight months. Compared to the baseline measurement collected at Day 0 before the administration of FCX-007, the percentage of dosed wounds healing > 50% when compared to baseline were observed as follows (number of wounds reflect the observations at that point in time):

- 100% (7/7) at 4 weeks post-administration
- 86% (6/7) at 12 weeks
- 67% (2/3) at 25/32 weeks
- 100% (1/1) at 52 weeks

A similar trend was also observed for treated wounds healing > 75% when compared to baseline. Untreated wounds of similar size to the treated wounds were selected and monitored as controls on each patient. The percentage of untreated control wounds healing > 50% when compared to baseline were observed as follows (number of wounds reflect the observations at that point in time):

- 14% (1/7) at 4 weeks post-administration
- 17% (1/6) at 12 weeks
- 0% (0/2) at 25/32 weeks

- 0% (0/1) at 52 weeks

Based on safety, pharmacology and wound healing data from the Phase 1 portion of the trial, we have incorporated learnings on dose and administration in our clinical trial protocol, including an increase in the overall cells administered and a reduction of the interval between injections.

Table of Contents

We completed the targeted enrollment of six patients ages seven and older in the Phase 2 portion of the Phase 1/2 clinical trial for FCX-007, and have over-enrolled by one patient for a total of seven patients. The Phase 2 population consists of one adult and six pediatric patients.

In March 2019, we reported additional positive safety data and wound healing data from our ongoing Phase 1/2 trial. To date, FCX-007 has been evaluated in eight wounds across five adult RDEB patients in the trial. Consistent with previously reported results, no product-related serious adverse events or circulating autoantibodies to COL7 have been reported.

The proportion of wounds healing at increasing closure percentages 12-week post-administration of a single injection session of FCX-007 are as follows:

	Wound Closure Percentage		
	>50%	>75%	Complete
FCX-007 Treated Wounds	88% (7/8)	75% (6/8)	63% (5/8)
Untreated Control Wounds	29% (2/7)	14% (1/7)	0% (0/7)

The complete wound closure result (63% treated vs 0% untreated) was the basis for the primary endpoint design in our proposed Phase 3 clinical trial for FCX-007.

The proportion of wounds with >50% closure at various post-administration visits are as follows:

	Study Visit			
	4 Weeks	12 Weeks	25 Weeks	52 Weeks
FCX-007 Treated Wounds	100% (8/8)	88% (7/8)	67% (2/3)	66% (2/3)
Untreated Control Wounds	13% (1/8)	29% (2/7)	0% (0/2)	33% (1/3)

We recently completed dosing of a sixth patient—the first pediatric patient dosed with FCX-007—in the current Phase 1/2 trial. Remaining Phase 2 patients who have not received dosing will be contacted to determine if they would agree to reconsent into the Phase 3 trial.

Proposed Phase 3 Trial Design

In October 2018, we completed a Type C meeting with the FDA to discuss the design of a Phase 3 clinical trial protocol for FCX-007. The meeting was facilitated by the then-current data in our ongoing Phase 1/2 clinical trial of FCX-007 and the recent publication of draft guidance from the FDA in the areas of gene therapy and EB. The FDA provided us with guidance on various design aspects of our proposed Phase 3 clinical trial. In addition, the FDA offered guidance on Chemistry, Manufacturing and Control (CMC) requirements for the proposed Phase 3 clinical trial and a potential future Biologics License Application (BLA) for FCX-007.

In November 2018, we received the official minutes from the FDA for the Type C meeting. Based on the FDA’s feedback, we prepared a Phase 3 clinical trial protocol for FCX-007 and filed it as part of the briefing package for the Type B meeting.

In March 2019, we completed a Type B end-of-Phase 2 face-to-face meeting with the FDA to discuss the design of a Phase 3 clinical trial for FCX-007 to support a BLA filing. In the Type B meeting, the FDA provided guidance on various design aspects of our proposed Phase 3 clinical trial, named DEFI-RDEB (dermal fibroblasts-RDEB). The trial is designed as an open label, multi-centered, intra-patient controlled trial expected to enroll 15-20 patients. Selected wounds will be monitored prior to dosing to confirm they are non-healing. For each patient, up to three pairs of wounds will be identified at baseline and randomized, with one wound receiving FCX-007 and the other wound left as the untreated control. Two doses of FCX-007 will be administered four weeks apart to the treated wounds. Both treated and untreated wounds will also receive standard of care, including routine skin care and bandaging.

The proposed primary outcome measure for the DEFI-RDEB trial is a comparison of the proportion of FCX-007 treated wounds and untreated matched wounds with complete closure at 12 weeks post-administration of the first dose. Secondary endpoints include evaluation of the proportion of wounds achieving >50% wound closure, a patient reported outcome measure and an analysis of durability out to 24 weeks. The presence of COL7 will be assessed from biopsy samples in a subpopulation of patients as an exploratory endpoint.

Table of Contents

We plan to submit a revised clinical trial protocol and statistical analysis plan based upon the FDA's feedback and requested CMC information to the Investigational New Drug (IND) application. We plan to continue the remaining follow-up visits with all Phase 1/2 patients, but do not intend to dose additional patients as part of the trial. Furthermore, we plan to initiate a Phase 3 clinical trial for FCX-007 in the second quarter of 2019.

We have designated our existing cGMP cell therapy manufacturing facility in Exton, PA as the production site for FCX-007 in our IND application. The FCX-007 drug product dosed in the fourth quarter of 2017 was produced and distributed from our Exton, PA facility. This multi-product, gene therapy manufacturing facility will be used for the remaining clinical and, if approved, future commercial manufacture of FCX-007, as we have sufficient cGMP vector supply to complete our clinical trials and existing manufacturing capacity to serve the U.S. market for RDEB. The approximately 13,000 square foot facility previously supported commercial autologous fibroblast manufacturing, with multiple FDA inspections conducted at the site. The facility includes cleanroom cell therapy manufacturing, quality control testing, cryogenic storage, shipping/receiving and warehousing space.

FCX-013 for Moderate to Severe Localized Scleroderma

Localized scleroderma is a chronic autoimmune skin disorder that manifests as excess production of extracellular matrix, specifically collagen, resulting in thickening of the skin and connective tissue. Localized scleroderma encompasses several subtypes which are classified based on the depth and pattern of the lesion(s). The moderate to severe forms of the disorder include any subtype that affects function or produces symptoms of discomfort, tightness and pain. Current treatments for localized scleroderma include systemic or topical corticosteroids which target inflammation, UVA light therapy and physical therapy. There are few treatment options to address excessive collagen accumulation in the skin and connective tissue. We estimate that there are approximately 90,000 patients in the U.S. considered to have moderate to severe localized scleroderma.

Our second clinical stage, gene therapy product candidate, FCX-013, is in development for the treatment of moderate to severe localized scleroderma. FCX-013 is an autologous fibroblast genetically-modified using lentivirus and encoded for matrix metalloproteinase 1 (MMP-1), the protein responsible for breaking down collagen. FCX-013 incorporates Intrexon's proprietary RheoSwitch Therapeutic System® (RTS®), a biologic switch activated by an orally administered compound (Veledimex) to control protein expression at the site of localized scleroderma lesions. FCX-013 is designed to be injected under the skin at the location of the fibrotic lesions where the genetically-modified fibroblast cells will produce MMP-1 to break down excess collagen accumulation. With the FCX-013 therapy, the patient will take Veledimex to facilitate protein expression. Once the fibrosis is resolved, the patient will stop taking Veledimex which will halt further MMP-1 production.

We previously completed a proof-of-concept study for FCX-013 in which the primary objective was to determine whether FCX-013 had the potential to reduce dermal thickness in fibrotic tissue. In this study, FCX-013 was evaluated in a bleomycin-induced scleroderma model utilizing severe combined immunodeficiency (SCID) mice. Data from the study demonstrated that FCX-013 reduced dermal thickness of fibrotic tissue to levels similar to that of the non-bleomycin treated control and further reduced the thickness of the sub-dermal muscle layer. Based upon these data and the FDA's feedback to our pre-IND briefing package, we advanced FCX-013 into a pre-clinical dose-ranging study which has been completed.

In December 2017, we completed a good laboratory practice (GLP) toxicology/biodistribution study that assessed FCX-013 in a bleomycin fibrosis model using immunocompromised (NOD/SCID) mice. Data from this study showed no test article-related clinical observations, body weight changes, changes in clinical pathology parameters, gross observations or organ weight change. In addition, there was no significant vector biodistribution to target organs.

We submitted an IND for FCX-013 to FDA in January 2018, and in March 2018, the FDA allowed the IND to progress to clinical trials. We initiated the first investigator site for clinical enrollment for an open label, single arm Phase 1/2 clinical trial. The primary objective of the trial is to evaluate the safety of FCX-013. Secondary analyses consist of several fibrosis assessments including histology, skin scores, ultrasound and additional measurements of targeted sclerotic lesions and control sites at various time points up to 16 weeks post-administration of FCX-013.

We are currently enrolling the Phase 1 portion of the Phase 1/2 clinical trial for FCX-013, and expect to complete enrollment of Phase 1 adult patients in the third quarter of 2019. We are targeting ten patients with any subtype of localized scleroderma for enrollment (approximately 5 patients per Phase). The Phase 1 portion will enroll adult patients, and dosing for the first three adult patients will be staggered prior to dosing the rest of the trial's population. We intend to include pediatric patients in the Phase 2 portion of the trial after submission and approval of safety and activity data from the adult Phase 1 patients to the FDA and the Data Safety Monitoring Board (DSMB) for the trial. We plan to manufacture FCX-013 at our Exton, PA cGMP manufacturing facility.

Table of Contents

FCX-013 has received Orphan Drug Designation from the FDA for the treatment of localized scleroderma and Rare Pediatric Disease Designation and in September 2018, Fast Track Designation for moderate to severe localized scleroderma.

Gene Therapy Research Program for Arthritis and Related Conditions

Arthritis is a broad term that covers a group of more than 100 different types of diseases that affect the joints, as well as connective tissues and organs, including the skin. According to the Centers for Disease Control and Prevention, arthritis—characterized by joint inflammation, pain and decreased range of motion—is the United States' most common cause of disability affecting more than 52 million adults as well as 300,000 children at a cost exceeding $120 billion.

Our third gene therapy program is in the research phase and is focused on the treatment of arthritis and related conditions. Our goal is to deliver a protein therapy locally to the joint to provide sustained efficacy while avoiding key side effects typically associated with systemic therapy.

Intrexon Collaborations

2012 Exclusive Channel Collaboration Agreement

In October 2012, we entered into an Exclusive Channel Collaboration Agreement, with Intrexon, which was amended in June 2013 and January 2014 (as amended, the 2012 ECC) pursuant to which we are Intrexon's exclusive channel collaborator in the research, development and commercialization of products in the following areas (the 2012 Fields):

- the enhanced production and purification of autologous fibroblasts (without genetic modification) for all aesthetic and therapeutic indications;
- the enhanced production and purification of autologous dermal cells (without genetic modification) for aesthetic and therapeutic treatment of dermal, vocal cord, and periodontal indications;
- the development of genetically modified autologous fibroblasts for all aesthetic and therapeutic indications where an autologous fibroblast itself is the principal effector of the product in contrast to the use of autologous fibroblasts as the source of expression of a systemically available therapeutic protein in which that protein (and not the fibroblast) is the principal therapeutic effector;
- the development of genetically modified autologous dermal cells for aesthetic and therapeutic treatment of dermal, vocal cord, and periodontal indications;
- autologous fibroblasts genetically modified to express a therapeutic protein and/or bioactive ribonucleic acid for the treatment of autoimmune and non-infectious inflammatory disorders that manifest in cutaneous tissues, fascia and/or muscle; and
- autologous human fibroblasts with gene therapy to express bioactive Tenascin-X locally to correct connective tissue disorders associated with Ehlers-Danlos Syndrome (hypermobility type).

Pursuant to the terms of the 2012 ECC, Intrexon has granted us a license to use its proprietary technologies and other intellectual property to research, develop and commercialize products in the 2012 Fields within the United States. We are responsible for all costs incurred in connection with the research, development and commercialization of products under the 2012 ECC and own all clinical data, regulatory filings and regulatory approvals relating to such products. We engage Intrexon for support services for the research and development of products under the 2012 ECC, and reimburse Intrexon for its cost for time and materials for such services.

We are required to pay Intrexon quarterly cash royalties on all products developed under the 2012 ECC in an amount equal to 7% of quarterly net sales up to $25 million, plus 14% on quarterly net sales greater than $25 million. We are also required to pay Intrexon half of any sublicensing revenues we receive from third parties in consideration for

sublicenses granted by us with respect to products developed under the 2012 ECC, but only to the extent such sublicensing revenues are not included in net sales subject to royalties. Sales from other products that we develop and commercialize outside of the 2012 ECC are not subject to royalty payments unless we are able to reduce the product's cost of goods sold through the 2012 ECC, in which case, we are required to pay quarterly cash royalties on such products equal to one-third of the cost of goods sold savings less any such savings developed by us outside of the 2012 ECC.

Table of Contents

The 2012 ECC may be terminated by Intrexon if we fail to exercise diligent efforts in developing products through the collaboration or if we elect not to pursue the development of a therapy identified by Intrexon within the 2012 Field and that qualifies as a "Superior Therapy" as defined in the 2012 ECC. Upon such termination, the products covered by the 2012 ECC in active and ongoing Phase 2 clinical trials or later stage development shall be entitled to be continued by us with a continuation of the related milestone, royalty and other payment obligations for such products, and all rights to products covered by the 2012 ECC still in an earlier stage of development shall revert to Intrexon.

In September 2015, we and Intrexon entered into a letter of agreement pursuant to which we mutually agreed to terminate our collaboration with respect to the development of potential therapies to treat Ehlers-Danlos Syndrome (hypermobility type) due to technical hurdles. As a result, we no longer have any rights or obligations under the 2012 ECC with respect to the development of "autologous human fibroblasts genetically modified to express bioactive Tenascin-X locally to correct connective tissue disorders."

Currently, we are in development of two gene therapy product candidates, FCX-007 and FCX-013, under the 2012 ECC, as more fully described under the heading "Development Programs" within "Item 1—Business" of this Form 10-K.

2015 Exclusive Channel Collaboration Agreement

In December 2015, we entered into an additional Exclusive Channel Collaboration Agreement with Intrexon (the 2015 ECC) pursuant to which we are Intrexon's exclusive channel collaborator in the research, development and commercialization of products for the treatment of chronic inflammation and degenerative diseases of human joints through intra-articular or other local administration of genetically-modified fibroblasts (the 2015 Field). The collaboration leverages our autologous fibroblast technology with Intrexon's synthetic biology technology to identify and develop cell-based therapeutics that will be genetically modified to express one or more proteins at sites of joint inflammation. We believe this treatment approach has the potential to overcome the limitations of existing therapies for chronic inflammation and degenerative diseases of the joint, including arthritis and related conditions.

Pursuant to the terms of the 2015 ECC, Intrexon has granted us a license to use its proprietary technologies and other intellectual property to develop and commercialize products in the 2015 Field throughout the world. We are responsible for all costs incurred in connection with the research, development and commercialization of products under the 2015 ECC and own all clinical data, regulatory filings and regulatory approvals relating to such products. We engage Intrexon for support services in connection with the research and development of products under the 2015 ECC, and reimburse Intrexon for its cost for time and materials for such services.

In consideration for the license and the other rights that we receive under the 2015 ECC, we paid Intrexon an up-front technology access fee of $10 million in cash in January 2016. For each product that we develop under the 2015 ECC, we are required to pay Intrexon development milestones of up to $30 million for the first product developed under the 2015 ECC (and development milestones up to $55 million for each subsequent product developed under the 2015 ECC) and commercialization milestones of up to $22.5 million, for each product developed, a low double-digit royalty on our net sales of such products and half of any sublicensing revenues we receive from third parties in consideration for sublicenses granted by us with respect to such products but only to the extent such sublicensing revenues are not included in net sales subject to royalties.

The 2015 ECC may be terminated by Intrexon if we fail to exercise diligent efforts in developing products through the collaboration or if we elect not to pursue the development of a therapy identified by Intrexon within the 2015 Field and that qualifies as a "Superior Therapy" as defined in the 2015 ECC. Upon such termination, the products covered by the 2015 ECC in active and ongoing Phase II clinical trials or later stage development shall be entitled to be continued by us with a continuation of the related milestone, royalty and other payment obligations for such products, and all rights to products covered by the 2015 ECC still in an earlier stage of development shall revert to Intrexon.

To date, we have only conducted research for a gene therapy product candidate for arthritis and related conditions under the 2015 ECC. We have deferred further development under the 2015 ECC in order to focus our efforts and our resources on our ongoing development of FCX-007 and FCX-013.

Table of Contents

Manufacturing

We lease and operate our own cGMP cell manufacturing facility located in Exton, Pennsylvania. We have historically used this facility to manufacture our non-genetically modified products and during 2016 began using this facility for pre-clinical manufacturing of our gene therapy product candidate, FCX-013. We designated this multi-product, gene therapy cGMP manufacturing facility in Exton, PA as the production and distribution site for FCX-007 in the fourth quarter of 2017. We are currently producing the drug product for the clinical development of FCX-007 and FCX-013 in our Exton facility. This facility will be used for the remaining clinical, and if approved, future commercial manufacture of FCX-007, as we have sufficient cGMP vector supply to complete our clinical trials and existing manufacturing capacity to serve the U.S. market for RDEB.

The fibroblast cells that constitute our product candidates are cultured by our proprietary cGMP manufacturing process, beginning with the collection of skin biopsies from the patient's skin. Fibroblasts are extracted from the biopsies and cultured using standard culture techniques to increase the cell population. A viral transduction is then performed to introduce targeted genes to the cells. The fibroblasts are then further expanded and cryopreserved for storage. When a treatment is requested, the cells are thawed, washed and prepared for shipment.

All component parts, including raw materials and other supplies utilized in our manufacturing process are available from various third party suppliers and manufacturers in quantities adequate to meet our needs. We seek to ensure continuity of supply of such component parts, raw materials and supplies using a strategy of dual sourcing, where possible. Some of our raw materials are currently sourced from one vendor; however, alternate vendors are available should they be required, although we would need sufficient lead time to qualify those vendors.

We use certain hazardous chemicals and biological materials in our manufacturing process which are subject to a variety of federal, state and local laws and regulations governing, among other matters, the use, generation, manufacture, transportation, storage, handling, disposal of and human exposure to these materials, including regulation by governmental regulatory agencies, such as the Occupational Safety and Health Administration and the U.S. Environmental Protection Agency. We incur capital and operating expenditures and other costs in the ordinary course of our business in complying with these laws and regulations. We dispose of minimal hazardous biological waste as a result of our manufacturing process.

Intellectual Property

We believe that patents, trademarks, copyrights and other proprietary rights are important to our business. We also rely on trade secrets, know-how and continuing technological innovations to develop and maintain our competitive position. We seek to protect our intellectual property rights by a variety of means, including obtaining patents, maintaining trade secrets and proprietary know-how and technological innovation to operate without infringing on the proprietary rights of others and to prevent others from infringing on our proprietary rights.

As of December 31, 2018, we own or license 9 issued U.S. patents, 7 pending U.S. patent applications, 3 granted foreign patents, 1 pending international patent application and 20 pending foreign patent applications. Our issued patents and patent applications primarily cover the method of using autologous cell fibroblasts for the repair of skin and soft tissue defects and the use of autologous fibroblast cells for tissue regeneration. In particular, we own an issued patent in the U.S. that is directed to methods of long-term augmentation of subcutaneous or dermal tissue by injecting an effective amount of a suspension of autologous passaged dermal fibroblasts into subadjacent tissue, which is set to expire in July 2020. In addition, we own an issued U.S. patent, issued patents in Australia, Canada and Japan and pending applications in China, Europe, India, South Korea, Hong Kong and the U.S. directed to dosage formulations for injection containing particular amounts of autologous human fibroblasts and uses thereof, which naturally expire in 2030 and 2031. We also own pending applications in the U.S. and several foreign countries related to topical formulations of autologous dermal fibroblasts and uses thereof, the earliest of which, if issued, would

naturally expire in 2027. We have also in-licensed from Intrexon, a portfolio of applications pending in the U.S., Europe, Australia, Canada, China, Israel, Japan, Singapore, South Korea and South Africa directed to compositions and methods for treating Type VII collagen deficiencies which, if issued, would expire in 2037. We have also in-licensed from Intrexon an international patent application directed to delivery of autologous cells comprising matrix metalloproteinase-1 for treating scleroderma which, if a patent would issue therefrom, would expire in 2038.
Competition

There is significant competition in the biopharmaceutical industry which can be attributed to companies ranging from small specialized biotechnology firms to large well-established pharmaceutical companies. More specifically, there are many companies currently competing in drug development for new therapies for the treatment of diseases affecting the skin, connective tissue and joints, our focus area. Some of our competitors have substantially greater financial resources and larger

Table of Contents

research and development organizations. In addition, our experience in clinical trials, obtaining FDA and other regulatory approvals, manufacturing and commercialization of products may be more limited.

Product competition is based on a variety of factors, including but not limited to: product safety, efficacy, convenience of dosing, availability, price, as well as brand recognition. Our product candidates, if approved for commercial use, will contend with treatments offered by our competitors. Although we believe the autologous nature and localized treatment approach of our product candidates provide advantages over our competitors, existing and new treatments may also possess certain advantages. Additionally, the development of other drug technologies and methods of treating diseases are occurring at a rapid pace. These developments may render our products or technologies obsolete or noncompetitive. Currently, we believe the primary competitors for our product candidates are as follows:

FCX-007 for RDEB. Our product candidate FCX-007 is being developed for the treatment of RDEB. Current treatments for RDEB, which include bandaging, antibiotics, feeding tubes, and surgery (hand and esophageal), only address the symptoms of this disorder. There are currently no products approved by the FDA for the treatment of RDEB. We are aware of a potentially competing product candidate, EB-101, which is a genetically-modified keratinocyte graft being developed by Abeona Therapeutics (Abeona). In May 2018, Abeona provided updated clinical data from its Phase 1/2 trial for EB-101 at the American Society for Gene and Cell Therapy annual meeting. According to Abeona's press release, the data showed "robust and durable clinical effects throughout various timepoints post-administration." Together with the FDA, Abeona worked to finalize the design of a Pivotal Phase 3 trial with the goal of initiating the study in 2019. Also in May 2018, Abeona announced the opening of its commercial GMP manufacturing facility for gene and cell therapies in Cleveland, Ohio.

Another potential competing product candidate is being developed by Krystal Biotech (Krystal). KB103 is a topical gene therapy for the treatment of the broad dystrophic epidermolysis bullosa (DEB) population that includes both recessive and dominant forms of the disease. In December 2018, Krystal reported four patients, ages 5 and older, were dosed in the GEM-Phase 2 study of KB103. Top-line results are expected in the first half of 2019 and will enable selection of endpoints moving into the pivotal study in the second half of 2019. In January 2019, Krystal announced construction was completed of its new GMP manufacturing facility in Pittsburgh, PA. Following completion of the first engineering run, the facility will officially open during the first quarter of 2019 and will support clinical and commercial manufacturing of KB103.

We are aware of several other products in development for the treatment of various forms of epidermolysis bullosa (including DEB and RDEB); however, we believe Abeona and Krystal are the primary potential competitors.

FCX-013 for Moderate to Severe Localized Scleroderma. Our product candidate FCX-013 is being developed for the treatment of moderate to severe localized scleroderma. Current treatments for localized scleroderma include systemic or topical corticosteroids which target inflammation, UVA light therapy, and physical therapy. There are few treatment options to address excessive collagen accumulation in the skin and connective tissue. There are currently no products approved by the FDA for the treatment of localized scleroderma. We are aware of a potentially competing product, Habeo Cell Therapy (formerly ECCS-50 Cellular Therapy), which is being developed by Cytori Therapeutics (Cytori). In 2017, Cytori announced that it expanded its investigation of ECCS-50 for the treatment of systemic scleroderma that affects the hands to include secondary Raynaud's phenomenon, a disorder that causes decreased blood flow to the fingers. In March 2018, Cytori announced that it met with the FDA to discuss the outcome of the STAR (Scleroderma Treatment with Celution Processed Adipose Derived Generative Cells) Pivotal Phase 3 clinical trial. Cytori has reported that the FDA provided verbal feedback that was generally consistent with the Cytori's belief that a clinical trial focused on more severely affected diffuse systemic sclerosis patients could be an appropriate next step given the results of the STAR clinical trial. Cytori has further reported that it planned to finalize meeting minutes and pursue additional dialogue with the FDA to clarify the parameters and key aspects of a potential follow-on clinical trial of Habeo Cell Therapy before making financial commitments to further pursue it.

We are also aware that miRagen Therapeutics has a product candidate, MRG-201, which utilizes microRNA biology and completed a Phase 1clinical trial for the treatment of systemic and localized scleroderma. In March 2018, miRagen Therapeutics reported the Phase 1 findings which supported advancement of MRG-201 into a Phase 2 trial in cutaneous fibrosis/scarring; subsequently, miRagen Therapeutics announced initiation of a Phase 2 clinical trial in July 2018 and that it anticipates presentation of data from the trial in 2019.

Government Regulation

We are subject to extensive government regulation, principally by the FDA and state and local authorities in the United States and by comparable agencies in foreign countries. Governmental authorities in the United States extensively regulate the pre-clinical and clinical testing, safety, efficacy, research, development, manufacturing, labeling, storage, record-keeping,

Table of Contents

advertising, promotion, import, export, marketing and distribution, among other things, of pharmaceutical and biologic products under various federal laws including the Federal Food, Drug and Cosmetic Act (FFDCA), the Public Health Service Act (PHSA) and under comparable laws by the states and in most foreign countries.

Domestic Regulation

In the United States, the FDA, under the FFDCA, the PHSA, and other federal statutes and regulations, exercises rigorous oversight of pharmaceutical and biologic products. If we do not comply with applicable requirements, we may be subjected to various enforcement actions, such as product seizures and court injunctions, the government may refuse to approve our marketing applications, and we could even be criminally prosecuted in certain circumstances. The FDA also has the authority to suspend or revoke a BLA, issue adverse publicity, and take other measures if we fail to comply with applicable regulatory standards.

FDA Approval Process

To obtain approval of a new drug product from the FDA, we must, among other requirements, submit data demonstrating its safety, purity, and potency, which includes efficacy, as well as detailed information on the manufacture and composition of the product candidate. In most cases, this entails extensive laboratory tests and pre-clinical and clinical trials. This testing and the preparation of necessary applications and processing of those applications by the FDA are expensive and typically take many years to complete. The FDA may deny our applications or may not act quickly or favorably in reviewing these applications, and we may encounter significant difficulties or costs in our efforts to obtain FDA approvals that could delay or preclude us from marketing any products we may develop. The FDA also may require post-marketing testing and surveillance to monitor the effects of approved products or place conditions on any approvals that could restrict the commercial applications of these products. Regulatory authorities may withdraw product approvals if we fail to comply with regulatory standards or if we encounter problems following initial marketing. With respect to patented products or technologies, delays imposed by the governmental approval process may materially reduce the period during which we may have the exclusive right to exploit the products or technologies.

The FDA does not apply a single regulatory scheme to human cells and tissues and products derived from human cells and tissue. On a product-by-product basis, the FDA may regulate such products as drugs, biologic products, or medical devices, in addition to regulating them as human cells, tissues, or cellular or tissue-based products (HCT/Ps), depending on whether or not the particular product triggers any of an enumerated list of regulatory factors. A fundamental difference in the treatment of products under these classifications is that the FDA permits certain HCT/Ps, sometimes referred to as section 361 HCT/Ps, that do not trigger any of those regulatory factors to be commercially distributed without regulatory approval. In contrast, HCT/P products that trigger those factors, such as if they are more than minimally manipulated, are regulated as drugs, biologics, or medical devices and require FDA approval prior to commercial distribution. We have determined that our product candidates trigger regulatory factors that make them biologic products, in addition to HCT/Ps, and consequently, we must obtain approval from the FDA before marketing such products and must also satisfy all regulatory requirements for HCT/Ps.

The process required by the FDA before a new drug or biologic product may be marketed in the United States generally involves the following:

- completion of pre-clinical laboratory tests or studies and formulation studies;
- submission to the FDA of an IND application for a new drug or biologic product, which must become effective before human clinical trials may begin;
-

performance of adequate and well-controlled human clinical trials to establish the safety and efficacy of the proposed drug, or safety, purity and potency of the proposed biologic product for its intended use;

- detailed information on product characterization and manufacturing process; and
- submission and approval of a New Drug Application (NDA) for a drug, or a BLA for a biologic product.

Pre-clinical tests include laboratory evaluation of product chemistry formulation and stability, as well as animal and other studies to evaluate toxicity. Under FDA regulations, the results of any pre-clinical testing, together with manufacturing information and analytical data, are submitted to the FDA as part of an IND application. The FDA requires a 30-day waiting period after the filing of each IND application before clinical trials may begin, in order to ensure that human research patients will not be exposed to unreasonable health risks. At any time during this 30-day period or at any time thereafter, the FDA may

Table of Contents

halt proposed or ongoing clinical trials, may authorize trials only on specified terms, or may require additional trials. The IND application process may become extremely costly and substantially delay development of our products. Moreover, positive results of pre-clinical tests will not necessarily forecast positive results in clinical trials.

Human clinical trials typically are conducted in three sequential phases, which may overlap. These phases generally include the following:

- Phase 1: The product candidate is usually first introduced into healthy humans or, on occasion, into patients, and is tested for safety, absorption, distribution, excretion and metabolism;

- Phase 2: The product candidate is introduced into a limited patient population to:
  - assess preliminary efficacy in specific, targeted indications;
  - assess dosage tolerance and optimal dosage; and
  - identify possible adverse effects and safety risks.

- Phase 3: These are commonly referred to as pivotal studies. If a product candidate is found to have an acceptable safety profile and to be potentially effective in Phase 2 clinical trials, clinical trials in Phase 3 will be initiated to further demonstrate clinical efficacy, optimal dosage and safety within an expanded and diverse patient population at geographically dispersed clinical trial sites and to provide a basis for product labeling; and

- If the FDA does ultimately approve the product candidate, it may require post-marketing testing, including potentially expensive Phase 4 studies, to confirm or further evaluate its safety and effectiveness. Continued ability to commercialize the product may be based on the successful completion of these additional studies.

Before proceeding with a trial, the sponsor may seek a written agreement from the FDA regarding the design, size, and conduct of a clinical trial. This is known as a Special Protocol Assessment (SPA). Among other things, SPAs can cover clinical trials for pivotal studies whose data will form the primary basis to establish a product's efficacy. SPAs thus help establish up-front agreement with the FDA about the adequacy of a clinical trial design to support a regulatory approval, but the agreement is not binding if new circumstances arise. There is no guarantee that a study will ultimately be adequate to support an approval, even if the study is subject to a SPA. The FDA retains significant latitude and discretion in interpreting the terms of the SPA and the data and results from any study that is the subject of the SPA. Circumstances under which a SPA may be revoked or altered include:

- a substantial scientific issue essential to determining the safety or efficacy of the drug has been identified after testing has begun;

- the protocol that was agreed upon with the FDA has not been followed by a sponsor;

- the relevant data, assumptions, or information provided by a sponsor in a request for a SPA change are found to be false or misleading, or are found to exclude relevant facts; or

- the FDA and sponsor agree in writing to modify the protocol and such modification is intended to improve the study.

Clinical trials must meet requirements for Institutional Review Board (IRB) oversight, patient informed consent and the FDA's Good Clinical Practice (GCP) requirements. Prior to commencement of each clinical trial, the sponsor must submit to the FDA a clinical study plan, or protocol, accompanied by the approval of the committee responsible for overseeing clinical trials at the clinical trial sites. The FDA or the IRB at each institution at which a clinical trial is being performed may order the temporary or permanent discontinuation of a clinical trial at any time if it believes that the clinical trial is not being conducted in accordance with FDA requirements or presents an unacceptable risk to the

clinical trial patients. Data safety monitoring boards, which monitor certain studies to protect the welfare of study patients, may also require that a clinical trial be discontinued or modified.

The sponsor must submit to the FDA the results of the pre-clinical and clinical trials, together with, among other things, detailed information on the manufacturing and composition of the product, and proposed labeling, in the form of an NDA, or, in the case of a biologic product, a BLA. The applicant must also submit with the NDA or BLA a substantial user fee payment, unless a waiver or reduction applies. In some cases, a sponsor may be able to expand the indications in an approved

Table of Contents

NDA or BLA through a submission of a Prior Approval Supplement. Each NDA or BLA submitted for FDA approval is usually reviewed for administrative completeness and reviewability within 60 days following submission of the application. If deemed complete, the FDA will "file" the NDA or BLA, thereby triggering substantive review of the application. The FDA can refuse to file any NDA or BLA that it deems incomplete or not properly reviewable. Once the submission has been accepted for filing, the FDA will review the application in accordance with performance goals the FDA, industry and Congress have negotiated for the review of NDAs and BLAs. For NDAs for new molecular entity drugs and for original BLA submissions, FDA's performance goal is to review and act on 90% of such applications within six months from the filing date for priority applications and ten months from the filing date for standard applications. The review process is often significantly extended by FDA requests for additional information, pre-clinical studies or clinical trials, or clarification, or by changes to the application submitted by the applicant in the form of amendments. The FDA may refer applications for novel product candidates which present difficult questions of safety or efficacy to an advisory committee, typically a panel that includes clinicians and other experts, for review, evaluation, and a recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions.

Before approving an NDA or BLA, the FDA generally inspects the facilities at which the product is manufactured and will not approve the product unless the manufacturing facilities are in compliance with cGMP requirements which govern the manufacture, holding and distribution of a product. Manufacturers of human cellular or tissue-based biologics also must comply with the FDA's Good Tissue Practices, as applicable, and the general biologic product standards.

The FDA reviews these applications and, when and if it decides that adequate data are available to show that the product is both safe and effective for its proposed use and that other applicable requirements have been met, approves the drug or biologic product for marketing.

The FDA may, during its review of an NDA or BLA, ask for additional study data. If the FDA does ultimately approve the product, approval may be subject to limitations based on the FDA's interpretation of the existing pre-clinical and clinical data and the FDA may require post-marketing testing, including potentially expensive Phase IV studies, to confirm or otherwise further evaluate the safety and effectiveness of the product. The FDA also may require, as a condition to approval or continued marketing of a drug, a risk evaluation and mitigation strategy (REMS) to ensure that the benefits of a drug or biologic product outweigh its risks. A REMS may include additional materials for patients such as Medication Guides and Patient Package Inserts, a plan for communicating information to healthcare professionals, and restricted distribution of the product. In addition, the FDA may, in some circumstances, impose elements to assure safe use (ETASU), which may be difficult and expensive to administer. Following approval, the FDA may require labeling changes or impose new post-approval study, risk management, or distribution restriction requirements.

It is possible that our product candidates will not successfully proceed through this approval process or that the FDA will not approve them in any specific period of time, or at all. The FDA may deny or delay approval of applications that do not meet applicable regulatory criteria, or if the FDA determines that the clinical data do not adequately establish the safety and efficacy of the product. Satisfaction of FDA pre-market approval requirements for a new biologic product is a process that may take a number of years and the actual time required may vary substantially based upon the type, complexity and novelty of the product or disease. Government regulation may delay or prevent marketing of potential products for a considerable period of time and imposes costly procedures upon our activities. Success in early stage clinical trials does not assure success in later stage clinical trials. Data obtained from clinical activities are not always conclusive and may be susceptible to varying interpretations that could delay, limit or prevent regulatory approval. Upon approval, a product candidate may be marketed only for those indications approved in the NDA or BLA and will be subject to labeling and promotional requirements or limitations, including warnings,

precautions, contraindications and use limitations, which could materially impact profitability. Once approved, the FDA may withdraw the product approval if compliance with pre- and post-market regulatory standards and requirements are not maintained or if safety, efficacy or other problems occur after the product reaches the marketplace.

The FDA has developed several programs intended to expedite the development and review of drugs that address unmet medical needs for serious or life threatening conditions, especially when the drugs are the first available treatment or have advantages over existing treatments:

- Accelerated Approval. The FDA may grant accelerated approval to drugs or biologic products that treat serious or life-threatening illnesses and that provide meaningful therapeutic benefits to patients over existing treatments. Under this program, the FDA may approve a product based on surrogate endpoints or clinical endpoints that can be measured earlier than mortality or irreversible morbidity. When approval is based on surrogate endpoints or clinical endpoints that can be measured earlier than mortality or irreversible morbidity, the sponsor will be required to conduct additional post-approval clinical trials to verify and describe clinical benefit. Under the

Table of Contents

agency's accelerated approval regulations, if the FDA concludes that a product that has been shown to be effective can be safely used only if distribution or use is restricted, it may require certain post-marketing restrictions as necessary to assure safe use. In addition, for products approved under accelerated approval, sponsors will be required to submit all copies of their promotional materials, including advertisements, to the FDA at least thirty days prior to initial dissemination unless otherwise informed by the FDA. After a hearing, the FDA may withdraw a previously granted accelerated approval if, for instance, post-marketing studies fail to verify any clinical benefit, it becomes clear that restrictions on the distribution of the product are inadequate to ensure its safe use, or if a sponsor fails to comply with the conditions of the accelerated approval.

- Breakthrough Therapy. The FDA may grant "breakthrough therapy" status to drugs or biologic products designed to treat, alone or in combination with another drug(s) or biologic(s), a serious or life-threatening disease or condition and for which preliminary evidence suggests a substantial improvement on clinically-meaningful endpoints over existing therapies. Such products need not address an unmet need, but are nevertheless eligible for expedited review if they offer the potential for an improvement over existing therapies. Breakthrough therapy status entitles the sponsor to earlier and more frequent meetings with the FDA regarding the development of nonclinical and clinical data and permits the FDA to offer product development or regulatory advice for the purpose of potentially shortening the time to product approval. The FDA may rescind breakthrough therapy designation if it believes the designated product no longer meets the qualifying criteria. Breakthrough therapy status does not guarantee that a product will be developed or reviewed more quickly and does not ensure FDA approval.

- Fast Track. The FDA may grant "fast track" status to drugs or biologic products that are intended to treat serious diseases or illness and demonstrate the potential to fill an unmet medical need. Fast track is a process designed to expedite the review of such products by providing, among other things, more frequent meetings with the FDA to discuss the product's development plan, more frequent written correspondence from the FDA about trial design, potential eligibility for accelerated approval, and rolling review, which allows submission of individually completed sections of a NDA or BLA for the FDA's review before the entire filing is completed. Fast track status does not ensure that a product will be developed more quickly or receive FDA approval more quickly, if at all.

- Priority Review. The FDA may grant "priority review" status to products that, if approved, would be significant improvements in safety or effectiveness of the treatment, diagnosis or prevention of serious conditions. Priority review is intended to reduce the time it takes for the FDA to review an NDA or BLA.

- Regenerative Medicine Advanced Therapy. A product may be eligible for RMAT designation if:

a. The drug is a regenerative medicine therapy, which is defined as a cell therapy, therapeutic tissue engineering product, human cell and tissue product, or any combination product using such therapies or products, except for those regulated solely under Section 361 of the Public Health Service Act and part 1271 of Title 21, Code of Federal Regulations;

b. The drug is intended to treat, modify, reverse, or cure a serious or life-threatening disease or condition; and

c. Preliminary clinical evidence indicates that the drug has the potential to address unmet medical needs for such disease or condition

Advantages of the RMAT designation include all of the benefits of the fast track and breakthrough therapy designation programs, including early interactions with FDA.

Additionally, there are various incentives to support development and approval of certain product candidates, including, but is not limited to, orphan drug designation and rare pediatric disease designation.

Orphan Drug Designation

Under the U.S. Orphan Drug Act, the FDA may grant orphan drug designation to drugs or biologic products intended to treat a “rare disease or condition,” which is generally defined as having a prevalence of less than 200,000 individuals in the U.S. Orphan drug designation must be requested before submitting an NDA or BLA for the product. Orphan drug designation does not shorten the regulatory review and approval process, nor does it provide any advantage in the regulatory review and approval process. However, if an orphan-designated drug is the first such drug to receive approval for the indication for which

Table of Contents

it has designation, the drug qualifies for orphan drug exclusivity, which means FDA may not approve any other applications to market the same drug for the same indication, except in very limited circumstances, for seven years in the U.S. Although obtaining approval to market a product with orphan drug exclusivity may be advantageous, we cannot be certain:

- that we will be the first to obtain approval for any drug for which we obtain orphan drug designation;
- that orphan drug designation will result in any commercial advantage or reduce competition; or
- that the limited exceptions to this exclusivity will not be invoked by the relevant regulatory authority.

Additionally, orphan drug exclusive marketing rights may be lost under certain conditions, such as if the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the drug.

FCX-007 and FCX-013 have received orphan drug designation from the FDA for the treatment of DEB (including RDEB) and localized scleroderma, respectively.

Rare Pediatric Disease Designation

FCX-007 has received rare pediatric disease designation from the FDA for the treatment of RDEB and FCX-013 has received rare pediatric disease designation from the FDA for the treatment of moderate to severe localized scleroderma. The FDA generally defines a “rare pediatric disease” as a serious or life-threatening disease in which the serious or life-threatening manifestations primarily affect individuals under the age of 18 years old, and that is a rare disease or condition as defined in the Orphan Drug Act (e.g., that affects fewer than 200,000 individuals in the U.S.)Under the FDA’s Rare Pediatric Disease Priority Review Voucher (PRV) program, upon the approval of an NDA or BLA for a product for the treatment of a rare pediatric disease, the sponsor of such application is eligible for a PRV. Currently, the PRV can be used to obtain priority review for any subsequent NDA or BLA and may be sold or transferred an unlimited number of times. Under the 21st Century Cures Act, Congress extended the PRV program for rare pediatric diseases through 2020. A drug designated as a drug for a rare pediatric disease by September 30, 2020 and approved by September 30, 2022, may receive a PRV. Because this program has been subject to criticism, including by the FDA, it is possible that even if we obtain approval for FCX-007 and FCX-013 and qualify for such a PRV, the program may no longer be in effect at the time of approval.

FDA Guidances

FDA recently has issued several guidance documents that we believe are relevant to our development programs. For example, in June 2018, the FDA published the first draft guidance for industry on the development of products for EB, entitled Epidermolysis Bullosa: Developing Drugs for Treatment of Cutaneous Manifestations. The draft guidance provides sponsors with direction regarding, among other things, clinical trial design, including trial population, efficacy endpoints and special considerations for trials involving patients with different types of EB. For example, the draft guidance advises sponsors to “develop clinical trials that minimize study visits and maximize patient comfort, as travel can exacerbate skin damage for patients with the condition.” The agency also noted that it is "critically important" for sponsors to reach an agreement with the FDA on the primary efficacy endpoint(s) and magnitude of change that constitute a clinically meaningful improvement before beginning clinical trials. Development of this draft guidance followed a Patient-Focused Drug Development meeting with the FDA in April 2018, which provided a forum for the FDA to hear directly from patients, advocates, and caretakers about the symptoms that matter most to them, the impact the disease has on patients’ daily lives, and patients’ experiences with currently available treatments. In addition, in July 2018, the FDA published several draft guidances for industry to aid in development of new gene therapies, including:

Chemistry, Manufacturing, and Control (CMC) Information for Human Gene Therapy Investigational New Drug Applications (INDs); Long Term Follow-up After Administration of Human Gene Therapy Products; Testing of Retroviral Vector-Based Human Gene Therapy Products for Replication Competent Retrovirus During Product

Manufacture and Patient Follow-up; and Human Gene Therapy for Rare Diseases.

These draft guidances can help sponsors be more efficient and effective when developing gene therapy clinical trials. For example, Human Gene Therapy for Rare Diseases provides recommendations for manufacturing, preclinical, and clinical trial design issues for a clinical development program for a human gene therapy product intended to treat a rare disease.

Furthermore, in January 2019, FDA updated a 2015 draft guidance, Rare Diseases: Common Issues in Drug Development, to help medical product developers further understand and advance the development of treatments for rare diseases, including the potential for novel endpoints or trial designs focusing on commonalities across a variety of rare diseases. Overall, we view these guidances as positive signals from the FDA regarding its commitment to advance the development of gene therapies for rare diseases, including for EB, and we plan to incorporate, as appropriate, the principles from these guidances into our regulatory strategy going forward.

Table of Contents

Biosimilars

The Biologics Price Competition and Innovation Act of 2009 (BPCIA), established an abbreviated pathway for the approval of follow-on biological products. This abbreviated regulatory pathway establishes legal authority for the FDA to review and approve biosimilar biologics, including the possible designation of a biosimilar as "interchangeable" to an existing brand product, or reference product. Under the BPCIA, an application for a biosimilar product cannot be approved by the FDA until 12 years after the reference product was approved under a BLA. These market and data exclusivities do not prevent another company from developing a product that is highly similar to the original branded product, generating its own data and seeking approval. Market and data exclusivity only assures that another company cannot rely upon the data within the innovator's application to support the biosimilar product's approval.

FDA's implementation and interpretation of the BPCIA continues to evolve and could have a material adverse effect on the future commercial prospects for our product candidates, if they are approved.

Ongoing FDA Requirements and Post-Marketing Obligations

All approved drug products are subject to continuing regulation by the FDA, including record-keeping requirements, reporting of adverse experiences with the product, compliance with cGMP, 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, submitting periodic reports to the FDA, maintaining and providing updated safety and efficacy information to the FDA, and complying with FDA promotion and advertising requirements. Failure to comply with applicable statutory and regulatory requirements can subject a manufacturer to possible legal or regulatory action, such as warning letters, suspension of manufacturing, seizure of product, injunction, criminal prosecution, or civil penalties.

The FDA may require post-marketing studies or clinical trials to develop additional information regarding the safety of a product. These studies or trials may involve continued testing of a product and development of data, including clinical data, about the product's effects in various populations and any side effects associated with long-term use. The FDA may require post-marketing studies or trials to investigate possible or known serious risks or signals of serious risks, or to identify unexpected serious risks, and may require periodic status reports if new safety information develops. Failure to conduct these studies in a timely manner may result in substantial civil penalties, or withdrawal of product approval.

Also, newly discovered or developed safety or efficacy data may require changes to a product's approved labeling, including the addition of new warnings and contraindications, additional pre-clinical studies or clinical trials, or even in some instances, withdrawal of the approval. Violations of regulatory requirements at any stage, including after approval, may result in various adverse consequences, including the FDA's withdrawal of an approved product from the market, other voluntary or FDA-initiated action that could delay or restrict further marketing, and the imposition of civil fines and criminal penalties against the manufacturer or the NDA or BLA holder. In addition, later discovery of previously unknown problems may result in restrictions on the product, manufacturer or NDA or BLA holder, including withdrawal of the product from the market.

The labeling, advertising, promotion, marketing and distribution of a drug or biologic product also must be in compliance with FDA requirements which address, among other things, promotional activities, direct-to-consumer advertising, promotional activities involving the internet, and industry sponsored scientific and educational activities. In general, all product promotion must be consistent with the labeling approved by the FDA for such product, contain a balanced presentation of information on the product's uses, benefits, risks, and important safety information and limitations of use, and otherwise be truthful and non-misleading. The FDA has very broad enforcement authority, and

failure to abide by these regulations can result in penalties, including the issuance of a warning letter directing a company to correct deviations from regulatory standards and enforcement actions that can include seizures, injunctions and criminal prosecution. Failure to comply with applicable FDA requirements and restrictions also may subject a company to adverse publicity and enforcement action by the U.S. Department of Justice (DOJ) or the Office of the Inspector General of the U.S. Department of Health and Human Services (HHS) as well as state authorities. This could subject the company to a range of penalties that could have a significant commercial impact, including civil and criminal fines and agreements that materially restrict the manner in which a company promotes or distributes its products.

Drug and biologic product manufacturers and, depending on their role, their subcontractors, are required to register their establishments with the FDA and certain state agencies, and to list their products with the FDA. The FDA periodically inspects manufacturing facilities in the United States and abroad in order to assure compliance with the applicable cGMP regulations and other requirements. These cGMP requirements apply to all stages of the manufacturing process, including production, processing, sterilization, packaging, labeling, storage and shipment. Facilities also are subject to inspections by

Table of Contents

other federal, foreign, state and local agencies. In complying with cGMP, manufacturers must continue to assure that the product meets applicable specifications, regulations and other post-marketing requirements. Failure to comply with these requirements subjects the manufacturer to possible legal, regulatory or voluntary action, such as suspension of manufacturing, recall or seizure of product.

Sponsors and their third-party contractors are also subject to various laws and regulations governing laboratory practices, the experimental use of animals and the use and disposal of hazardous or potentially hazardous substances in connection with their research. The FDA has regulatory and enforcement authority to disqualify nonclinical laboratory studies performed by a violative facility from being considered by FDA in support of any application for a research or marketing permit; to publicly disclose the fact of such disqualification; and to pursue any other available and appropriate judicial proceeding or regulatory action, such as court-ordered injunctions, denial or withdrawal of regulatory approvals, and referral to other federal, state or local government law enforcement or regulatory agencies.

Furthermore, new government requirements may be established that could delay or prevent regulatory approval of our products under development, or affect the conditions under which approved products are marketed.

HIPAA Requirements

Other federal legislation may affect our ability to obtain certain health information in conjunction with our research activities. We may be subject to data privacy and security regulation by both the federal government and the states in which we conduct our business. The Health Insurance Portability and Accountability Act of 1996 , as amended by the Health Information Technology for Economic and Clinical Health Act of 2009 (HITECH), and its implementing regulations (collectively referred to as HIPAA), imposes obligations, including mandatory contractual terms, with respect to safeguarding the privacy, security and transmission of individually identifiable health information. HIPAA also prohibits knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false, fictitious or fraudulent statements or representation, or making or using any false writing or document knowing the same to contain any materially false, fictitious or fraudulent statement or entry in connection with the delivery of or payment for healthcare benefits, items or services. We may obtain health information from third parties, such as research institutions, that are subject to privacy and security requirements under HIPAA. Although we are not directly subject to HIPAA - other than with respect to providing certain employee benefits - we could potentially be subject to criminal penalties if we, our affiliates, or our agents knowingly obtain or disclose individually identifiable health information maintained by a HIPAA-covered entity in a manner that is not authorized or permitted by HIPAA.

In addition, numerous federal and state laws and regulations that address privacy and data security, including state data breach notification laws, state health information privacy laws, and federal and state consumer protection laws (e.g., Section 5 of the Federal Trade Commission Act), govern the collection, use, disclosure and protection of health-related and other personal information. Failure to comply with data protection laws and regulations could result in government enforcement actions and create liability for us (which could include civil and/or criminal penalties), private litigation and/or adverse publicity that could negatively affect our business.

Failure to achieve and sustain compliance with applicable federal and state privacy, security and fraud laws could result in government enforcement actions and create liability for us (which could include civil and/or criminal penalties), private litigation and/or adverse publicity that could negatively affect our operating results and business.

Other U.S. Regulatory Requirements

In the United States, the research, manufacturing, distribution, sale, and promotion of drug and biologic products are potentially subject to regulation by various federal, state and local authorities in addition to the FDA, including the Centers for Medicare and Medicaid Services (CMS), other divisions of the HHS (e.g., the Office of Inspector

General), the DOJ and individual U.S. Attorney offices within the DOJ, and state and local governments. For example, sales, marketing and scientific/educational grant programs must comply with the federal Anti-Kickback Statute, the False Claims Act and other federal and state fraud and abuse laws, as described in detail below.

If we successfully commercialize any of our products, we may participate in the Medicaid Drug Rebate Program. Participation is required for federal funds to be available under Medicaid and Medicare Part B for our products that we successfully commercialize. Under the Medicaid Drug Rebate Program, we would be required to pay a rebate to each state Medicaid program for our covered outpatient drugs that are dispensed to Medicaid beneficiaries and paid for by a state

Table of Contents

Medicaid program as a condition of having federal funds being made available to the states for our drugs under Medicaid and Part B of the Medicare program.

Federal law requires that any company that participates in the Medicaid Drug Rebate Program also participate in the Public Health Service's 340B drug pricing program in order for federal funds to be available for the manufacturer's drugs under Medicaid and Medicare Part B. The 340B drug pricing program requires participating manufacturers to agree to charge statutorily-defined covered entities no more than the 340B "ceiling price" for the manufacturer's covered outpatient drugs. The 340B ceiling price generally represents a significant discount. These 340B covered entities include a variety of community health clinics and other entities that receive health services grants from the Public Health Service, as well as hospitals that serve a disproportionate share of low-income patients.

Under Section 603 of the Veterans Health Care Act of 1992 (VHCA), drug companies that participate in Medicaid or Medicare Part B or sell to certain federal agencies and certain federal grantees are required to offer their "covered drugs" (biologic products and innovator drugs) for sale on a Federal Supply Schedule contract at a statutorily reduced price to four federal agencies including the U.S. Department of Veterans Affairs, the U.S. Department of Defense, the Public Health Service and the Coast Guard. Participation under Section 603 the VHCA requires submission of pricing data and calculation of discounts pursuant to complex statutory formulas, as well as the entry into government procurement contracts governed by the Federal Acquisition Regulation. In addition, pursuant to regulations issued by the Department of Defense TRICARE Management Activity, now the Defense Health Agency, to implement Section 703 of the National Defense Authorization Act for Fiscal Year 2008, manufacturers are required to pay quarterly rebates on "covered drug" prescriptions dispensed to TRICARE beneficiaries by TRICARE network retail pharmacies. All of these activities are also potentially subject to federal and state consumer protection, unfair competition, and other laws.

In March 2010, President Obama signed into law the Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010 (together, the Affordable Care Act). The Affordable Care Act substantially changed the way healthcare is financed by both governmental and private insurers, and significantly impacts the pharmaceutical industry. The Affordable Care Act intended to broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against fraud and abuse, add new transparency requirements for health care and health insurance industries, impose new taxes and fees on the health industry and impose additional health policy reforms. The Affordable Care Act has resulted in, and we expect it will continue to result in, downward pressure on coverage and the price of products covered by Medicare and other government programs. Any reduction in reimbursement from Medicare and other government programs may result in a similar reduction in payments and coverage from private payors. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability, or commercialize our products. Some of the provisions of the Affordable Care Act have yet to be fully implemented, and certain provisions have been subject to judicial and Congressional challenges. In addition, there have been efforts by the Trump Administration to repeal or replace certain aspects of the Affordable Care Act and to alter the implementation of the Affordable Care Act and related laws. For example, the Tax Cuts and Jobs Act enacted on December 22, 2017, eliminated the tax-based shared responsibility payment for individuals who fail to maintain minimum essential coverage under section 5000A of the Internal Revenue Code of 1986, as amended (the Code), commonly referred to as the "individual mandate," effective January 1, 2019. Further, the Bipartisan Budget Act of 2018, among other things, amended the Medicare statute to reduce the coverage gap in most Medicare drug plans, commonly known as the "donut hole," by raising the required manufacturer point-of-sale discount from 50% to 70% off the negotiated price effective as of January 1, 2019. Additional legislative changes, regulatory changes, and judicial challenges related to the Affordable Care Act remain possible. It is unclear how the Affordable Care Act and its implementation, as well as efforts to repeal or replace, or invalidate, the Affordable Care Act, or portions thereof, will affect our business, financial condition and results of operations.

At such time as we market, sell and distribute any products for which we obtain marketing approval, it is possible that our business activities could be subject to scrutiny and enforcement under one or more federal or state health care fraud and abuse laws and regulations, which could affect our ability to operate our business. These include the following fraud and abuse laws and regulations:

- The federal Anti-Kickback Statute prohibits, among other things, knowingly and willfully offering, paying, soliciting or receiving remuneration to induce or in return for purchasing, leasing, ordering or arranging for the purchase, lease or order of any healthcare item or service reimbursable under Medicare, Medicaid or other federally financed healthcare programs. This statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on one hand and prescribers, purchasers, and formulary managers on the other. Although there are a number of statutory exemptions and regulatory safe harbors protecting some business arrangements from prosecution, the exemptions and safe harbors are drawn narrowly and practices that involve remuneration intended to induce prescribing, purchasing or recommending may be subject to scrutiny if they do

Table of Contents

not qualify for an exemption or safe harbor. Our practices may not in all cases meet all of the criteria for safe harbor protection from federal Anti-Kickback Statute liability. The Affordable Care Act, among other things, clarified that liability may be established under the federal Anti-Kickback Statute without proving actual knowledge of the statute or specific intent to violate it. In addition, the Affordable Care Act provides that the government may assert that a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the civil False Claims Act (discussed below) or the civil monetary penalties statute, which imposes penalties against any person who is determined to have presented or caused to be presented a claim to a federal health program that the person knows or should know is for an item or service that was not provided as claimed or is false or fraudulent.

The federal civil False Claims Act prohibits any person from, among other things, knowingly presenting, or causing to be presented, a false or fraudulent claim for payment of government funds; knowingly making, using, or causing to be made or used a false record or statement material to a false or fraudulent claim to the federal government; or knowingly concealing or knowingly and improperly avoiding, decreasing or concealing an obligation to pay money to the federal government. The False Claims Act also permits a private individual acting as a "whistleblower" to bring actions on behalf of the federal government alleging violations of the statute and to share in any monetary recovery. Many pharmaceutical and other healthcare companies have been investigated and have reached substantial financial settlements with the federal government under the civil False Claims Act for a variety of alleged improper marketing activities, including: providing free product to customers with the expectation that the customers would bill federal programs for the product; providing sham consulting fees, grants, free travel and other benefits to physicians to induce them to prescribe the company's products; and inflating prices reported to private price publication services, which are used to set drug payment rates under government healthcare programs. In addition, in recent years the government has pursued civil False Claims Act cases against a number of pharmaceutical companies for causing false claims to be submitted as a result of the marketing of their products for unapproved, and thus non-reimbursable, uses. False Claims Act liability is potentially significant in the healthcare industry because the statute provides for treble damages and mandatory penalties in the tens of thousands of dollars. Pharmaceutical and other healthcare companies also are subject to other federal false claim laws, including, among others, federal criminal healthcare fraud and false statement statutes that extend to non-government health benefit programs.

- HIPAA created federal criminal statutes that prohibit among other actions, knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program, including private third-party payors, knowingly and willfully embezzling or stealing from a healthcare benefit program, willfully obstructing a criminal investigation of a healthcare offense, and knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false, fictitious or fraudulent statement in connection with the delivery of or payment for healthcare benefits, items or services.

- Analogous state laws and regulations, such as state anti-kickback and false claims laws, may apply to items or services reimbursed under Medicaid and other state programs or, in several states, apply regardless of the payor. Some state laws also require pharmaceutical companies to report expenses relating to the marketing and promotion of pharmaceutical products and to report gifts and payments to certain healthcare providers in the states. Other states prohibit providing meals to prescribers or other marketing related activities. Other states restrict the ability of manufacturers to offer co-pay support to patients for certain prescription drugs. Still other states and cities require identification or licensing of sales representatives. In addition, California, Connecticut, Nevada and Massachusetts require pharmaceutical companies to implement compliance programs or marketing codes of conduct.

- The federal Physician Payment Sunshine Act, being implemented as the Open Payments Program, requires certain pharmaceutical manufacturers to engage in extensive tracking of payments and other transfers of value to physicians and teaching hospitals, and to submit such data to CMS, which will then make all of this data publicly available on the CMS website. Pharmaceutical manufacturers with products for which payment is available under Medicare, Medicaid

or the State Children's Health Insurance Program are required to track reportable payments and must submit a report to CMS on or before the 90th day of each calendar year disclosing reportable payments made in the previous calendar year. Failure to comply with the reporting obligations may result in civil monetary penalties.

- The federal Foreign Corrupt Practices Act of 1997 and other similar anti-bribery laws in other jurisdictions generally prohibit companies and their intermediaries from providing money or anything of value to officials of foreign governments, foreign political parties, or international organizations with the intent to obtain or retain

Table of Contents

business or seek a business advantage. Recently, there has been a substantial increase in anti-bribery law enforcement activity by U.S. regulators, with more frequent and aggressive investigations and enforcement proceedings by both DOJ and the U.S. Securities and Exchange Commission (SEC). Violations of United States or foreign laws or regulations could result in the imposition of substantial fines, interruptions of business, loss of supplier, vendor or other third-party relationships, termination of necessary licenses and permits and other legal or equitable sanctions. Other internal or government investigations or legal or regulatory proceedings, including lawsuits brought by private litigants, may also follow as a consequence.

Violations of any of the laws described above or any other governmental regulations are punishable by significant civil, criminal and administrative penalties, damages, fines and exclusion from government-funded healthcare programs, such as Medicare and Medicaid. Although compliance programs can mitigate the risk of investigation and prosecution for violations of these laws, the risks cannot be entirely eliminated. Moreover, achieving and sustaining compliance with applicable federal and state privacy, security and fraud laws may prove costly.

International Regulation

The regulation of our product candidates outside of the United States varies by country. Certain countries regulate human tissue products as a pharmaceutical product, which would require us to make extensive filings and obtain regulatory approvals before selling our product candidates. Certain other countries classify our product candidates as human tissue for transplantation but may restrict its import or sale. Other countries may have no application regulations regarding the import or sale of products similar to our product candidates, creating uncertainty as to what standards we may be required to meet.

Employees

As of December 31, 2018, we had 19 full-time employees, all located in the United States. Of these full-time employees, 15 are engaged in research, development and manufacturing (including facilities) functions and 4 are engaged in finance, legal, human resources, information technology, and other general administrative functions. None of our employees are covered by a collective bargaining agreement, and we consider our relations with our employees to be good.

Corporate Information

We were incorporated under the laws of the State of Delaware in September 1992. Our corporate office is located at 405 Eagleview Boulevard, Exton, Pennsylvania 19341. Our telephone number is (484) 713-6000. We maintain an Internet website at www.fibrocell.com. The information contained on our website is not incorporated by reference into this Form 10-K.

Table of Contents

Item 1A. Risk Factors

Our business is subject to substantial risks and uncertainties. The occurrence of any of the following risks and uncertainties, either alone or taken together, could materially and adversely affect our business, financial condition, results of operations or prospects. In these circumstances, the market price of our common stock could decline and you may lose all or part of your investment. The risks and uncertainties described below are not the only ones we face. Risks and uncertainties of general applicability and additional risks and uncertainties not currently known to us or that we currently deem to be immaterial may also materially and adversely affect our business, financial condition, results of operations or prospects.

Risks Related to our Financial Position and Need for Additional Capital

There can be no assurance that our review of strategic alternatives will result in any additional stockholder value, and speculation and uncertainty regarding the outcome of our review of strategic alternatives may adversely impact our business, financial condition and results of operations.

On April 18, 2018, we announced that our Board of Directors (Board) would begin conducting a review of strategic alternatives to maximize stockholder value. There can be no assurances that the strategic alternatives process will result in the announcement or consummation of any strategic transaction, or that any resulting plans or transactions will yield additional value for stockholders. Any potential transaction would be dependent on a number of factors that may be beyond our control, including, among other things, market conditions, industry trends, the interest of third parties in a potential transaction and the availability of financing to potential buyers on reasonable terms. If we fail to successfully complete a strategic transaction, we may not be able to otherwise source adequate liquidity to fund our operations, meet our obligations (including our debt payment obligations) and continue as a going concern.

The process of exploring strategic alternatives could adversely impact our business, financial condition and results of operations. We could incur substantial expenses associated with identifying and evaluating potential strategic alternatives, including those related to equity compensation, severance pay and legal, accounting and financial advisory fees. In addition, the process may be time consuming and disruptive to our business operations, could divert the attention of management and the Board from our business, could negatively impact our ability to attract, retain and motivate key employees, and could expose us to potential litigation in connection with this process or any resulting transaction. Further, speculation regarding any developments related to the review of strategic alternatives and perceived uncertainties related to our future could cause our stock price to fluctuate significantly.

We need to obtain additional capital to continue as a going concern. If we are unable to obtain sufficient capital, we will need to curtail and reduce our operations and costs, and modify our business strategy.

Our principal sources of liquidity are cash and cash equivalents of $14.4 million as of December 31, 2018. As of December 31, 2018, we had working capital of $12.5 million. We believe that our existing cash and cash equivalents will be sufficient to fund our operations into the fourth quarter of 2019. However, changing circumstances may cause us to consume capital faster than we currently anticipate, and we may need to spend more money than currently expected because of such circumstances.

To meet our capital needs, we are considering multiple alternatives, including but not limited to, equity financings, debt financings, corporate collaborations, partnerships and other strategic transactions and funding opportunities. However, there can be no assurance that we will be able to complete any such transaction on acceptable terms or otherwise. These factors raise substantial doubt about our ability to continue as a going concern. Consequently, the audit report prepared by our independent registered public accounting firm relating to our Consolidated Financial Statements for the year ended December 31, 2018 includes a paragraph related to the substantial doubt about our ability to continue as a going concern.

If we raise additional funds by issuing equity securities, our stockholders will experience dilution. Debt financing, if available, will result in increased fixed payment obligations and may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends. Any debt financing or additional equity that we raise may contain terms, such as liquidation, dividends and other rights or preferences, which are not favorable to us or our stockholders. If we raise additional funds through collaboration or partnership arrangements with third parties, it may be necessary to relinquish valuable rights to our technologies, future revenue streams or product candidates or to grant licenses on terms that may not be favorable to us.

If we are unable to raise additional capital in sufficient amounts or on terms acceptable to us, we will need to curtail and reduce our operations and costs, and modify our business strategy which may require us to, among other things:

Table of Contents

- significantly delay, scale back or discontinue the development or commercialization of one or more of our product candidates or one or more of our other research and development initiatives;

- seek collaborators for one or more of our current or future product candidates at an earlier stage than otherwise would be desirable or on terms that are less favorable than might otherwise be available;

- sell or license on unfavorable terms our rights to technologies or product candidates that we otherwise would seek to develop or commercialize ourselves; or

- seek bankruptcy protection which may result in the termination of agreements pursuant to which we license important intellectual property rights including our exclusive collaboration agreements with Intrexon.

We have incurred significant losses since our inception and anticipate that we will continue to incur losses in the future.

We have incurred losses since our inception, have not generated significant revenue from commercial sales of our products, and have never been profitable. Investment in drug development is highly speculative because it entails substantial upfront capital expenditures and significant risk that a product candidate will fail to gain regulatory approval or become commercially viable. We continue to incur significant research, development and other expenses related to our ongoing operations including development of our product candidates and operation of our manufacturing facility. As a result, we are not profitable and have incurred losses in each period since we emerged from bankruptcy in September 2009. For the year ended December 31, 2018, we had a net loss of $10.3 million and used $12.7 million in operating activities and had an accumulated deficit of $189.1 million as of December 31, 2018.

We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future. We anticipate that our expenses will continue to be significant if and as we:

- continue our research and pre-clinical and clinical development of our product candidates;

- initiate additional pre-clinical, clinical or other studies or trials for our product candidates, including under our collaboration agreements with Intrexon;

- continue or expand our collaborations with Intrexon and our other collaborators;

- further develop the manufacturing process for our product candidates;

- continue to maintain a cGMP manufacturing facility;

- change or add additional manufacturers or suppliers;

- seek regulatory approvals for our product candidates that successfully complete clinical trials;

- establish a sales, marketing and distribution infrastructure to commercialize any products for which we may obtain regulatory approval;

- seek to identify and validate additional product candidates;

- acquire or in-license other product candidates and technologies;

- maintain, protect and expand our intellectual property portfolio;

- attract and retain skilled personnel;

- create additional infrastructure to support our product development and planned future commercialization efforts; and

- experience any delays or encounter issues with any of the above.

The net losses we incur may fluctuate significantly from quarter to quarter and year to year, such that a period-to-period comparison of our results of operations may not be a good indication of our future performance. In any particular quarter or quarters, our operating results could be below the expectations of securities analysts or investors, which could cause our stock price to decline.

Table of Contents

We do not generate significant revenues from product sales and may never be profitable.

Our ability to generate revenue and achieve profitability depends on our ability, alone or with strategic collaboration partners, to successfully complete the development of, and obtain the regulatory approvals necessary for, the manufacture and commercialization of our product candidates. We do not anticipate generating significant revenues from product sales for the foreseeable future, if ever. Our ability to generate future revenues from product sales depends heavily on our success in:

- completing research and pre-clinical and clinical development of our product candidates;
- seeking and obtaining regulatory approvals for product candidates for which we complete clinical trials;
- developing a sustainable, scalable, reproducible, and transferable manufacturing process for our product candidates;
- establishing and maintaining supply and manufacturing relationships with third parties that can provide adequate (in amount and quality) products and services to support clinical development and the market demand for our product candidates, if approved;
- launching and commercializing product candidates for which we obtain regulatory approval, either by collaborating with a partner or, if launched independently, by establishing a sales force, marketing, sales operations and distribution infrastructure;
- obtaining market acceptance of our product candidates and cell therapy and gene therapy as viable treatment options;
- addressing any competing technological and market developments;
- implementing additional internal systems and infrastructure, as needed;
- identifying and validating new product candidates;
- negotiating favorable terms in any collaboration, licensing or other arrangements into which we may enter;
- maintaining, protecting and expanding our portfolio of intellectual property rights, including patents, trade secrets and know-how; and
- attracting, hiring and retaining qualified personnel.

Even if one or more of the product candidates that we develop is approved for commercial sale, we anticipate incurring significant costs associated with commercializing any approved product candidate. Our expenses could increase beyond expectations if we are required by the FDA or other regulatory agencies, domestic or foreign, to perform clinical trials or other studies in addition to those that we currently anticipate. Even if we are able to generate revenues from the sale of any approved products, we may not become profitable and may need to obtain additional funding to continue operations.

We will seek to raise additional funds in the future, which may be dilutive to stockholders or impose operational restrictions.

We will need to raise additional capital in the future to help fund our clinical trials, our collaboration efforts with Intrexon and for the development and commercialization of our product candidates. If we raise additional capital

through the issuance of equity securities, the percentage ownership of our current stockholders will be reduced. We may also issue equity as part of license issue fees to our licensors, to compensate consultants or to settle outstanding payables. Our stockholders may experience additional dilution in net book value per share and any additional equity securities may have rights, preferences and privileges senior to those of the holders of our common stock. Debt financing, if available, will result in increased fixed payment obligations and may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends. Any debt financing or additional equity that we raise may contain terms, such as liquidation and other preferences, which are not favorable to us or our stockholders. If we raise additional funds through corporate collaboration, partnership or other strategic transactions, it may be necessary to relinquish valuable rights to our product candidates, our technologies or future revenue streams or to grant licenses or sell assets on terms that may not be favorable to us. If we cannot raise additional funds, we will have to delay our development activities or cease operations.

Table of Contents

Our failure to comply with the restrictive covenants or other terms of our outstanding convertible notes, including as a result of events beyond our control, could result in a default under the notes that could materially and adversely affect the ongoing viability of our business.

On September 7, 2016, we issued an aggregate of approximately $18.1 million in principal of convertible promissory notes (each a Note and collectively, the Notes) and accompanying warrants to purchase an aggregate of 1,205,840 shares of our common stock (the 2016 Private Placement Warrants) in a private placement (the 2016 Private Placement) to institutional and accredited investors (each an Investor and collectively, the Investors). The Notes bear interest at 4% per annum and have a stated maturity date of the earlier of (i) September 7, 2026 and (ii) one-hundred and eighty (180) days after the date on which our product candidate, FCX-007, is approved by the FDA for the treatment of RDEB. Each individual Note holder has the right to require us to repay all or any portion of the unpaid principal from time to time on or after September 7, 2021 (such right, a Put Right). With respect to accrued and unpaid interest on the Note, each Note holder may elect, at any time and from time to time, to have any accrued and unpaid interest converted into shares of our common stock. In addition, each Note holder may elect to accelerate the repayment of all unpaid principal and accrued interest under such holder's Note upon consummation of a specified change of control transaction or occurrence of certain events of default (as specified in the Notes), including, among other things:

- our default in a payment obligation under the Notes;
- our default in a payment obligation under our other debt in excess of $5 million;
- our breach of the restrictive covenants or other terms of the Notes;
- certain specified insolvency and bankruptcy-related events; and
- our common stock ceasing to be listed or quoted on Nasdaq or another national securities exchange.

In addition, upon an event of default, the base interest rate (excluding any additional interest) for the Notes automatically increases to twelve percent (12%) per annum. Subject to any applicable cure period set forth in the Notes, all amounts outstanding with respect to the Notes (principal and accrued interest) would become due and payable immediately upon an event of default. We cannot assure you that our assets or cash flow would be sufficient to fully repay our obligations under the Notes if the obligations thereunder are accelerated upon any events of default. Further, if we are unable to repay, refinance or restructure our obligations under the Notes, the holders of such Notes could proceed to protect and enforce their rights under the Notes by exercising such remedies as are available to the holders thereunder and in respect thereof under applicable law, either by suit in equity or by action at law, or both, whether for specific performance of any covenant or other agreement contained in the Notes or in aid of the exercise of any power granted in the Notes. The foregoing would materially and adversely affect the ongoing viability of our business.

We are subject to restrictive covenants that may restrict our ability to pursue business strategies that are in our long-term best interests.

The Notes and Purchase Agreement (as defined below) for the sale of our Series A Preferred Stock (as defined below) contain a number of restrictive covenants that impose significant restrictions on us and may limit our ability to engage in acts that may be in our long-term best interests. Subject to certain limited exceptions, the Notes and Purchase Agreement include covenants restricting, among other things, our ability to:

- pay cash dividends or make distributions on our capital stock or redeem or repurchase our capital stock;
- create, assume or suffer to exist at any time any lien upon any of our properties or assets;
- assign any accounts or other right to receive income;
- incur any senior and pari passu debt;

- enter into transactions with affiliates other than on terms and conditions approved by a majority of the disinterested members of our Board; and

- use the proceeds of the 2016 Private Placement or Series A Preferred Stock Offering (as defined below) for any purpose other than solely for the continued pre-clinical and clinical development of our product candidates and for other general corporate purposes.

Table of Contents

In addition, a breach of any of these restrictive covenants could result in a default under the Notes, entitling the holders to declare the Notes, together with accrued and unpaid interest and other amounts payable thereunder, to be immediately due and payable.

Provisions of the Notes and certain of our outstanding common stock purchase warrants provide for certain potential payments to the holders of such Notes and common stock purchase warrants that could impede a sale of the Company.

The 2016 Private Placement Warrants, the December 2017 Common Warrants (as defined below), the May 2018 Warrants (as defined below) and the July 2018 Warrants (as defined below) give each holder the option to receive a cash payment based on a Black-Scholes valuation upon our change of control. We are required, at the warrant holder's option, exercisable at any time concurrently with, or within 30 days after, the announcement of a change of control, to repurchase such warrants from the applicable holder by paying to the holder an amount of cash equal to the value of the unexercised portion of such holder's warrant as determined in accordance with the Black-Scholes option pricing model and the terms of the applicable warrant.

In addition, upon consummation of a specified change of control transaction, each holder of a Note may elect to accelerate the repayment of all unpaid principal and accrued interest under such holder's Note. If a holder does not elect to have us prepay its Note upon such change of control transaction, then we may prepay the Notes, in an amount equal to one hundred one percent (101%) of the outstanding principal due under the Notes (together with accrued and unpaid interest due thereon). These provisions may make it more costly for a potential acquirer to engage in a business combination transaction with us. Provisions that have the effect of discouraging, delaying or preventing a change in control could limit the opportunity for our stockholders to receive a premium for their shares of our common stock and could also affect the price that some investors are willing to pay for our common stock.

We may be subject to payment of liquidated damages if we fail to file and maintain an effective registration statement with respect to the securities covered under the registration rights agreements that we entered into in connection with the 2016 Private Placement.

In connection with the 2016 Private Placement, we entered into a registration rights agreement (the Registration Rights Agreement) with the investors that participated in the offering. The Registration Rights Agreement contains demand and piggyback registration rights requiring us to register shares of our common stock issuable upon the conversion of the Notes or the exercise of the 2016 Private Placement Warrants and any other shares of our common stock held by the investors for resale under the Securities Act of 1933, as amended. If we fail, under certain circumstances as described in the Registration Rights Agreement, to file and maintain an effective registration statement with respect to the securities covered under the Registration Rights Agreement, we have agreed to pay liquidated damages to each investor in an amount equal to one percent (1.0%) of the aggregate amount invested by such investor pursuant to the Notes then owned thereby for each 30-day period or pro rata for any portion thereof during which the failure to file or keep a registration statement effective continues.

We have a significant number of outstanding convertible notes, convertible preferred stock, warrants and stock options, and future sales of underlying shares of our common stock may cause substantial dilution to our existing stockholders.

We issued an aggregate of $18.1 million in principal of Notes and 2016 Private Placement Warrants to purchase a total of 1,205,840 shares of our common stock in connection with the 2016 Private Placement. Each 2016 Private Placement Warrant has a five year term ending on September 7, 2021 and is initially exercisable at $22.50 per share beginning March 8, 2017. Holders of the Notes have the right to convert unpaid principal of the Notes into shares of our common stock at any time at conversion prices ranging from $17.04375 to $18.39375 per share (the Conversion Price). The Notes bear interest at four percent (4%) per annum which we may elect to pay in cash or accrue. If we

elect for interest to accrue, then (i) we may elect to repay any such accrued and unpaid interest in cash at any time and from time to time and (ii) each holder of a Note may elect to have us repay any such accrued and unpaid interest by delivering such number of shares of our common stock equal to (x) the amount of the accrued and unpaid interest to be repaid, divided by (y) the greater of (i) the last closing bid price of a share of our common stock as reported on Nasdaq on the date of such election and (ii) the applicable Conversion Price. Commencing September 8, 2016, we have elected to accrue interest.

On March 7, 2017, we entered into a Securities Purchase Agreement (the Purchase Agreement) with certain of our existing investors pursuant to which we issued and sold a total of 8,000 units (the Units) for a purchase price of $1,000 per Unit, with each Unit consisting of (i) one share of our Series A Convertible Preferred Stock (Series A Preferred Stock) and (ii) a warrant to purchase up to a number of shares of our common stock equal to 100% of the conversion shares issuable on March 7, 2017 pursuant to the shares of Series A Preferred Stock purchased by each investor (the March 2017 Warrants) (collectively, the Series A Preferred Stock Offering). Each share of Series A Preferred Stock has an initial stated value of $1,000 and is convertible into shares of our common stock at a conversion price of $11.6355 per share of common stock, subject to

Table of Contents

adjustment for stock splits, stock dividends, stock combinations, recapitalizations or similar events. Holders of the Series A Preferred Stock are also entitled to receive cumulative dividends at a rate per share of 4% per annum (with such dividend rate increasing to 8% per annum on the five year anniversary of the original issuance of the Series A Preferred Stock), with such dividends compounded quarterly by increasing the stated value of the Series A Preferred Stock. Each March 2017 Warrant has an exercise price of $12.68865 per share, is exercisable six months after the date of issuance and expires five years from the date of issuance.

On December 7, 2017, we entered into an underwriting agreement (the HCW Underwriting Agreement) with H.C. Wainwright & Co., LLC (HCW), pursuant to which we issued and sold 1,542,832 shares of our common stock, pre-funded warrants to purchase an aggregate of 1,184,442 shares of our common stock (December 2017 Pre-Funded Warrants) and common stock purchase warrants to purchase up to an aggregate of 2,809,404 shares of our common stock (the December 2017 Common Warrants), which amount of common stock purchase warrants includes warrants to purchase up to 82,118 shares of our common stock pursuant to the partial exercise of HCW's option to purchase additional common stock purchase warrants . Each share of our common stock or December 2017 Pre-Funded Warrant, as applicable, was sold together with a December 2017 Common Warrant to purchase one share of our common stock at a combined effective price to the public of $3.85 per share and accompanying December 2017 Common Warrant (collectively, the December 2017 Offering). Each December 2017 Common Warrant has an exercise price of $3.85 per share, was exercisable upon the date of issuance and expires five years from the date of issuance.

As additional compensation, we issued warrants to HCW to purchase 87,274 shares of our common stock (the December 2017 Underwriter Warrants). Each December 2017 Underwriter Warrant has an exercise price of $4.8125 per share, was exercisable as of the date of the HCW Underwriting Agreement, and will expire five years after the date of the HCW Underwriting Agreement.

On May 29, 2018, we entered into securities purchase agreements (the May 2018 Purchase Agreements) with certain institutional and accredited investors, pursuant to which we sold 2,038,224 shares of our common stock (the May 2018 Registered Direct Offering). Concurrently with the May 2018 Registered Direct Offering, and pursuant to the May 2018 Purchase Agreements, in connection with a private placement (the May 2018 Private Placement), we sold warrants to purchase up to an aggregate of 1,528,668 shares of our common stock (the May 2018 Private Placement Warrants). Each May 2018 Private Placement Warrant has an exercise price of $2.86 per share, was exercisable upon the date of issuance and expires 5 and one-half years from the date of issuance.

As partial compensation for placement agent services by HCW in connection with the May 2018 Registered Direct Offering, we issued warrants to HCW to purchase 142,676 shares of our common stock (the May 2018 Placement Agent Warrants and, together with the May 2018 Private Placement Warrants, the May 2018 Warrants). Each May 2018 Placement Agent Warrant has an exercise price of $3.679 per share, was exercisable as of the date of issuance, and will expire five years after the date of the HCW Underwriting Agreement.

On July 2, 2018, we entered into securities purchase agreements (the July 2018 Purchase Agreements) with certain institutional and accredited investors, pursuant to which we sold 1,474,080 shares of our common stock (the July 2018 Registered Direct Offering). Concurrently with the July 2018 Registered Direct Offering, and pursuant to the July 2018 Purchase Agreements, in connection with a private placement (the July 2018 Private Placement and, together with the July 2018 Registered Direct Offering, the July 2018 Offering), we sold warrants to purchase up to an aggregate of 958,152 shares of our common stock (the July 2018 Private Placement Warrants). Each July 2018 Private Placement Warrant has an exercise price of $2.70 per share, was exercisable upon the date of issuance and expires 5 and one-half years from the date of issuance.

As partial compensation for placement agent services by HCW in connection with the July 2018 Registered Direct Offering, we issued warrants to HCW to purchase 103,186 shares of our common stock (the July 2018 Placement Agent Warrants and, together with the July 2018 Private Placement Warrants, the July 2018 Warrants). Each July 2018 Placement Agent Warrant has an exercise price of $3.464 per share, was exercisable as of the date of issuance, and will expire five years after the date of the HCW Underwriting Agreement.

Subject to adjustment upon certain corporate events, including stock dividends, stock splits and distributions of cash or other assets to stockholders:

- up to 1,056,068 shares of our common stock could be issuable by us in connection with the conversion of principal under the Notes; plus

Table of Contents

- up to 101,937 shares of our common stock could be issuable by us in satisfaction of our interest payment obligations under the Notes; plus

- up to1,205,840 shares of our common stock could be issuable by us in connection with the exercise of the 2016 Private Placement Warrants; plus

- up to 736,000 shares of our common stock could be issuable by us in connection with the conversion of the shares of Series A Preferred Stock; plus

- up to 687,468 shares of our common stock could be issuable by us in connection with the exercise of the March 2017 Warrants; plus

- up to 87,274 shares of our common stock could be issuable by us in connection with the exercise of the December 2017 Underwriter Warrants; plus

- up to 2,679,702 shares of our common stock could be issuable by us in connection with the exercise of the December 2017 Common Warrants; plus

- up to 1,528,668 shares of common stock could be issuable by us in connection with the exercise of the May 2018 Private Placement Warrants; plus

- up to 142,676 shares of common stock could be issuable by us in connection with the exercise of the May 2018 Placement Agent Warrants; plus

- up to 958,152 shares of common stock could be issuable by us in connection with the exercise of the July 2018 Private Placement Warrants; plus

- up to 103,186 shares of common stock could be issuable by us in connection with the exercise of the July 2018 Placement Agent Warrants.

The exercise of the 2016 Private Placement Warrants, the March 2017 Warrants, the December 2017 Pre-Funded Warrants, the December 2017 Underwriter Warrants, the December 2017 Common Warrants, the May 2018 Private Placement Warrants, the May 2018 Placement Agent Warrants, the July 2018 Private Placement Warrants or the July 2018 Placement Agent Warrants, or the conversion of the Notes or Series A Preferred Stock would cause substantial dilution to our existing stockholders.

If our stockholders' equity falls below $2.5 million, our common stock may be subject to delisting from Nasdaq

Nasdaq has the authority, pursuant to Nasdaq Listing Rule 5550(b)(1), to delist our common stock if our stockholders' equity falls below $2.5 million. As of December 31, 2018, our stockholders' equity was $9.5 million. If our stockholders equity is hereafter reduced below $2.5 million as a result of operating losses or for other reasons, we will fail to meet Nasdaq's stockholders' equity requirement. If that occurs, or if we are unable to demonstrate to Nasdaq's satisfaction that we will be able to sustain compliance with this requirement, Nasdaq may delist our common stock. In addition, even if we regain technical compliance with the stockholders' equity requirement, we will have to continue to meet other objective and subjective listing requirements to continue to be listed on the Nasdaq Capital Market, including the requirement that our common stock continues to trade above $1.00. For the year ended December 31, 2018, we incurred a net loss of $10.3 million and used $12.7 million in operating activities and had an accumulated deficit of $189.1 million as of December 31, 2018.

We are actively monitoring our stockholders' equity and will consider any and all options available to us to maintain compliance. There can be no assurance, however, that we will be able to maintain compliance and meet Nasdaq's minimum stockholders' equity requirements. The alternatives to trading on the Nasdaq Capital Market or another national securities exchange are generally considered to be less efficient and less broad-based than the national securities exchanges and the liquidity of our common stock will likely be reduced. In addition, if at any time we are not listed on the Nasdaq Capital Market (or similar national securities exchange), then each holder of our Notes will have the option to declare the Notes held by each holder immediately due and payable, which would drain our financial resources, have a material adverse effect on our financial condition and make it exceedingly difficult to continue as a going concern.

Table of Contents

If our common stock becomes subject to the penny stock rules, it would become more difficult to trade our shares.

The SEC has adopted rules that regulate broker-dealer practices in connection with transactions in penny stocks. Penny stocks are generally equity securities with a price of less than $5.00, other than securities registered on certain national securities exchanges or authorized for quotation on certain automated quotation systems, provided that current price and volume information with respect to transactions in such securities is provided by the exchange or system. If we do not retain our listing on the Nasdaq Capital Market and if the price of our common stock is less than $5.00, our common stock may be deemed a penny stock. The penny stock rules require a broker-dealer, before a transaction in a penny stock not otherwise exempt from those rules, to deliver a standardized risk disclosure document containing specified information. In addition, the penny stock rules require that before effecting any transaction in a penny stock not otherwise exempt from those rules, a broker-dealer must make a special written determination that the penny stock is a suitable investment for the purchaser and receive (i) the purchaser's written acknowledgment of the receipt of a risk disclosure statement; (ii) a written agreement to transactions involving penny stocks; and (iii) a signed and dated copy of a written suitability statement. These disclosure requirements may have the effect of reducing the trading activity in the secondary market for our common stock, and therefore stockholders may have difficulty selling their shares.

We have a limited operating history, which may make it difficult for you to evaluate the success of our business to date and to assess our future viability.

We have a limited operating history and our primary business activities consist of research, pre-clinical development and conducting clinical trials, pursuing our collaborations with Intrexon and previously commercializing LAVIV (azficel-T) which had previously been approved by the FDA in June 2011 for the improvement of the appearance of moderate to severe nasolabial fold wrinkles in adults. As such, our historical financial data is of limited value in estimating future operating expenses. Our budgeted expense levels are based in part on our expectations concerning the costs of our research, pre-clinical development, clinical trials and our collaborations with Intrexon, which depend on the success of such activities, and our ability to effectively and efficiently conduct such research, pre-clinical development, clinical trials and our expectations related to our efforts to achieve FDA approval with respect to our product candidates. Our limited operating history and clinical trial experience make these costs difficult to forecast accurately. We may be unable to adjust our operations in a timely manner to compensate for any unexpected increase in costs. Further, our fixed manufacturing costs and operating expenses may increase significantly as we expand our operations. Accordingly, a significant increase in costs could have an immediate and material adverse effect on our business, results of operations and financial condition.

We may acquire other assets or businesses, or form collaborations or make investments in other companies or technologies that could harm our operating results, dilute our stockholders' ownership, incur debt or cause us to incur significant expense.

As part of our business strategy, we may pursue acquisitions of assets or businesses, or strategic alliances and collaborations, to expand our existing technologies and operations. We may not identify or complete these transactions in a timely manner, on a cost-effective basis, or at all, and we may not realize the anticipated benefits of any such transaction, any of which could have a detrimental effect on our financial condition, results of operations and cash flows. We may not be able to find suitable acquisition candidates, and if we make any acquisitions, we may not be able to integrate these acquisitions successfully into our existing business and we may incur debt or assume unknown or contingent liabilities in connection therewith. Integration of an acquired company or assets may also disrupt ongoing operations, require the hiring of additional personnel and the implementation of additional internal systems and infrastructure, especially the acquisition of commercial assets, and require management resources that would otherwise focus on developing our existing business. We may not be able to find suitable collaboration partners or identify other investment opportunities, and we may experience losses related to any such investments.

To finance any acquisitions or collaborations, we may choose to issue debt or shares of our common stock as consideration. Any such issuance of shares would dilute the ownership of our stockholders. If the price of our common stock is low or volatile, we may not be able to acquire other assets or companies or fund a transaction using

our stock as consideration. Alternatively, it may be necessary for us to raise additional capital for acquisitions through public or private financings. Additional capital may not be available on terms that are favorable to us, or at all. In addition, the Notes issued in the 2016 Private Placement restrict or limit our ability to incur or assume additional indebtedness.

Table of Contents

Risks Related to Clinical Development, Regulatory Approval and Commercialization of Our Product Candidates

Our product candidates are based on novel technology, which makes it difficult to predict the time and cost of product development and subsequently obtaining regulatory approval. At the moment, only a small number of gene therapy products have been approved in the United States and the European Union.

Our product candidates, are based on novel technology. Our future success depends on the successful development of this therapeutic approach. There can be no assurance that any development problems we experience in the future related to our product candidates will not cause significant delays or unanticipated costs, or that such development problems can be solved. We may also experience delays in developing a sustainable, reproducible and manufacturing process or transferring that process to commercial partners, which may prevent us from completing our clinical trials or commercializing our product candidates on a timely or profitable basis, if at all.

In addition, the clinical trial requirements of the FDA, the European Medicines Agency (the EMA), and other regulatory agencies and the criteria these regulators use to determine the safety and efficacy of a product candidate vary substantially according to the type, complexity, novelty and intended use and market of the potential products. The regulatory approval process for gene therapy product candidates such as ours can be more expensive and take longer than for other, better known or more extensively studied pharmaceutical or biologic product candidates. At the moment, only a small number of gene therapy products have been approved in the Western world, which makes it difficult to determine how long it will take or how much it will cost to obtain regulatory approvals for our product candidates in the United States, the European Union or other jurisdictions. Approvals by the EMA may not be indicative of what the FDA may require for approval and vice versa.

Regulatory requirements governing gene and cell therapy products have evolved and may continue to change in the future. For example, the FDA has established the Office of Tissues and Advanced Therapies within its Center for Biologics Evaluation and Research (CBER) to consolidate the review of gene therapy and related products, and established the Cellular, Tissue and Gene Therapies Advisory Committee to advise CBER on its review. In addition, the Recombinant DNA Advisory Committee (RAC) of the U.S. National Institutes of Health (NIH), reviews human gene transfer protocols when an oversight body, such as an Institutional Review Board (IRB), has determined that the protocol would significantly benefit from RAC review and when the protocol meets certain criteria. The NIH Director may also select a protocol unilaterally for RAC review if it presents significant scientific, societal, or ethical concerns. Although the FDA decides whether individual gene therapy protocols may proceed, the RAC review process can impede the initiation of a clinical trial, even if the FDA has reviewed the clinical trial and approved its initiation. Furthermore, all institutions and clinical trial sites in the United States that are subject to the NIH Guidelines for Research Involving Recombinant or Synthetic Nucleic Acid Molecules (NIH Guidelines), must follow those NIH Guidelines and all RAC recommendations, or risk losing NIH funding for such research or needing NIH pre-approval before conducting such research. Institutions subject to the NIH Guidelines include all institutions that receive any funding from NIH for research involving recombinant or synthetic nucleic acid molecules, as well as institutions subject to federal or state regulations, local ordinances, or agency guidelines that require compliance with the NIH Guidelines, and institutions receiving support from federal agencies or private funders who condition such support on compliance with the NIH Guidelines. In addition, the FDA can put an IND on clinical hold for numerous reasons, such as if the information in such IND is not sufficient to assess the risks in pediatric patients. Before a clinical trial can begin at any institution, that institution’s IRB, and its Institutional Biosafety Committee will have to review the proposed clinical trial to assess the safety of the trial. Moreover, because of the relative novelty of gene therapy, SAEs, or other concerns observed in clinical trials of gene therapy product candidates conducted by other sponsors may cause the FDA or other regulatory bodies to initiate a clinical hold on our clinical trials or otherwise to change the requirements for approval of any of our product candidates.

These regulatory review agencies, committees and advisory groups and any new requirements and guidelines they promulgate may lengthen the regulatory review process, require us to perform additional or larger studies, increase our

development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of these product candidates or lead to significant post-approval studies, limitations or restrictions. As we advance our product candidates, we may be required to consult with these regulatory and advisory groups and comply with applicable requirements and guidelines. If we fail to do so or to do so effectively, we may be required to delay or discontinue development of our product candidates. Delay or failure to obtain, or unexpected costs in obtaining, the regulatory approval necessary to bring a potential product to market could decrease our ability to generate sufficient product revenue to maintain our business.

Initial results from a pre-clinical study or clinical trial do not ensure that the study or trial will be successful and success in pre-clinical studies and early stage clinical trials does not ensure success in later-stage clinical trials.

Although we have seen positive results in pre-clinical studies of FCX-007 and FCX-013 and interim data from the Phase 1 portion of the Phase 1/2 clinical trial of FCX-007, results from pre-clinical studies and early clinical trials often do not

Table of Contents

accurately predict final pivotal clinical trial results. In addition, data from one pivotal clinical trial may not be predictive of the results of other pivotal clinical trials for the same product candidate, even if the trial designs are the same or similar. Data obtained from pre-clinical studies and clinical trials are susceptible to varying interpretations, which may delay, limit or prevent regulatory approval. Adverse side effects may be observed in clinical trials that delay, limit or prevent the regulatory approval, and even after a product candidate has received marketing approval, the emergence of adverse side effects in more widespread clinical practice may cause the product's regulatory approval to be limited or even rescinded. Additional trials necessary for approval may not be undertaken or may ultimately fail to establish the safety and efficacy of our product candidates.

In addition, while the clinical trials of our product candidates are designed based on the available relevant information, in view of the uncertainties inherent in drug development, such clinical trials may not be designed with a focus on indications, patient populations, dosing regimens, safety or efficacy parameters or other variables that will provide the necessary safety and efficacy data to support regulatory approval to commercialize the product. In addition, the methods we select to assess particular safety or efficacy parameters may not yield statistically significant results regarding our product candidates' effects on patients. Even if we believe the data collected from clinical trials of our product candidates are promising, these data may not be sufficient to support approval by the FDA or foreign regulatory authorities. Pre-clinical and clinical data can be interpreted in different ways. Accordingly, the FDA or foreign regulatory authorities could interpret these data in different ways from us, which could delay, limit or prevent regulatory approval.

In previous clinical trials conducted by sponsors other than us involving viral vectors for gene therapy, some patients experienced SAEs, including the development of leukemia due to vector-related insertional oncogenesis. If our vectors demonstrate a similar effect, we may be required to halt or delay further clinical development of our product candidates.

A significant risk in any gene therapy product based on viral vectors is that the vector will insert in or near cancer-causing oncogenes leading to uncontrolled clonal proliferation of mature cancer cells in the patient. For example, in 2003, 20 patients treated for X-linked severe combined immunodeficiency in two gene therapy trials conducted by sponsors other than us using a murine, or mouse-derived, gamma-retroviral vector showed correction of the disease, but the trials were terminated after five patients developed leukemia (four of whom were subsequently cured). The cause of these adverse events was shown to be insertional oncogenesis, which is the process whereby the corrected gene inserts in or near a gene that is important in a critical cellular process like growth or division, and this insertion results in the development of a cancer (often leukemia). Using molecular diagnostic techniques, it was determined that clones from these patients showed retrovirus insertion in proximity to the promoter of the LMO2 proto-oncogene. Earlier generation retroviruses like the one used in these two trials have been shown to preferentially integrate in regulatory regions of genes that control cell growth.

These well-publicized adverse events led to the development of new viral vectors, such as lentiviral vectors like the ones we utilize for FCX-007 and FCX-013, with potentially improved safety profiles, as well as the requirement of enhanced safety monitoring in gene therapy clinical trials, including routine performance of vector copy number analysis on all production lots to monitor the number of insertion events per cell. Notwithstanding the potential safety improvements of lentiviral vectors, the risk of insertional oncogenesis remains a significant concern for gene therapy and we cannot assure that it will not occur in any of our clinical trials. There is also the potential risk of delayed adverse events following exposure to gene therapy products due to persistent biological activity of the genetic material or other components of products used to carry the genetic material. The FDA has stated that lentiviral vectors possess characteristics that may pose risks of delayed adverse events. If any such adverse events occur, further advancement of our clinical trials could be halted or delayed, which would have a material adverse effect on our business and operations.

We may find it difficult to enroll patients in our clinical trials, which could delay or prevent clinical trials of our product candidates.

Identifying and qualifying patients to participate in clinical trials of our product candidates is critical to our success. The timing of our clinical trials depends in part on the speed at which we can recruit patients to participate in testing our product candidates. We have experienced delays in some of our clinical trials, and we may experience similar delays in the future. If patients are unwilling to participate in our clinical trials because of negative publicity from adverse events in cell and gene therapies or for other reasons, including competitive clinical trials for similar patient populations, the timeline for recruiting patients, conducting clinical trials and obtaining regulatory approval of potential products may be delayed. These delays could result in increased costs, delays in advancing our product candidates, delays in testing the effectiveness of our technology or termination of the clinical trials altogether.

We may not be able to identify, recruit and enroll a sufficient number of patients, or those with required or desired characteristics to achieve diversity in a clinical trial, or complete our clinical trials in a timely manner. Patient enrollment is affected by a variety factors including, among others:

Table of Contents

- severity of the disease under investigation;
- design of the study protocol;
- prevalence of the disease/size of the patient population;
- eligibility criteria for the clinical trial in question;
- perceived risks and benefits of the product candidate under study;
- proximity and availability of clinical trial sites for prospective patients;
- availability of competing therapies and clinical trials;
- efforts to facilitate timely enrollment in clinical trials;
- patient referral practices of physicians; and
- ability to monitor patients adequately during and after treatment.

Our current product candidates are being developed to treat rare diseases with limited patient pools from which to draw for clinical trials and the process of finding and diagnosing patients may prove costly. We have estimated that there are approximately 1,100 to 2,500 U.S. patients with RDEB and approximately 90,000 patients in the US considered to have moderate to severe localized scleroderma. We may not be able to initiate or continue clinical trials if we cannot enroll a sufficient number of eligible patients to participate in the clinical trials required by the FDA or other regulatory agencies. If we have difficulty enrolling a sufficient number of patients to conduct our clinical trials as planned, we may need to delay, limit or terminate ongoing or planned clinical trials, any of which would have an adverse effect on our business.

If physicians do not follow our established protocols, the efficacy and safety of our product candidates may be adversely affected.

We depend on physicians and other healthcare professionals to follow our established protocols as to the administration and the handling of our product candidates in connection with our clinical trials, and we will continue to depend on physicians and other healthcare professionals to follow such protocols if and when our product candidates are commercialized. The treatment protocol requires each physician to verify the patient's name and date of birth with the patient and the patient records immediately prior to injection. In the past, more than one patient's cells have been delivered to a physician, or the wrong patient's cells have been delivered to a physician. While it is the physician's obligation to follow the treatment protocol and assure that the patient is treated with the correct cells, if physicians and other healthcare professionals do not follow our protocols, the efficacy and safety of our product candidates may be adversely affected.

Clinical trials may fail to demonstrate the safety or efficacy of our product candidates, which could prevent or significantly delay regulatory approval of our product candidates and harm our business.

Prior to receiving approval to commercialize any of our product candidates, we must demonstrate with substantial evidence from well-controlled clinical trials, and to the satisfaction of the FDA and other regulatory authorities in the United States and abroad, if applicable, that such product candidate is both safe and effective. We will need to demonstrate such product candidate's efficacy and monitor its safety throughout the development process. If our current or future clinical trials are unsuccessful, regulatory approval of our product candidates could be delayed or prevented and our business could be harmed.

All of our product candidates are subject to the risks of failure inherent in drug development. The results of early-stage clinical trials of our product candidates do not necessarily predict the results of later-stage clinical trials. Product candidates in later-stage clinical trials may fail to demonstrate desired safety and efficacy traits despite having successfully progressed through initial clinical testing. Even if we believe the data collected from clinical trials of our product candidates is promising, this data may not be sufficient to support approval by the FDA or any other U.S. or foreign regulatory approval. The FDA may also reject any of our completed clinical trials as inadequate to support

approval if the trial design does not include specific safety monitoring measures. Pre-clinical and clinical data can be interpreted in different ways. Accordingly, FDA officials could reach different conclusions in assessing such data than we do which could delay, limit or prevent regulatory approval. In addition, the FDA, other regulatory authorities, our IRB or we may suspend or terminate clinical trials at any time.

Obtaining FDA and other regulatory approvals is complex, time consuming and expensive, and the outcomes are uncertain.

The process of obtaining FDA and other regulatory approvals is time consuming, expensive and difficult. Clinical trials are required to establish the safety and efficacy of product candidates. Applications to market product candidates must be

Table of Contents

submitted to the FDA and the FDA must review and approve them before product candidates may be marketed, and subsequent clinical trials, manufacturing, and the marketing of products, once approved, are subject to strict regulatory compliance. The commencement and completion of clinical trials for any of our product candidates could be delayed or prevented by a variety of factors, including:

- delays in obtaining regulatory approvals or in an IND going into effect to commence a study or trial;
- delays in identifying and reaching agreement on acceptable terms with prospective clinical trial sites;
- delays or failures in obtaining approval of our clinical trial protocol from an IRB to conduct a clinical trial at a prospective study site;
- delays in the enrollment of patients;
- manufacturing difficulties;
- failure of our clinical trials and clinical investigators to comply with FDA's GCP requirements;
- failure of our third-party contract research organizations, clinical site organizations or other clinical trial managers, to satisfy their contractual duties, comply with regulations or meet expected deadlines;
- lack of efficacy during clinical trials; or
- unforeseen safety issues.

We do not know whether our clinical trials will need to be restructured or will be completed on schedule, if at all, or whether they will provide data necessary to support regulatory approval. Significant delays in clinical trials will impede our ability to commercialize our product candidates and generate revenue, and could significantly increase our development costs.

In addition, we utilize bovine-sourced materials to manufacture our product candidates. It is possible that future FDA regulations may require us to change the source of the bovine-sourced materials we use in our product candidates or to cease using bovine-sourced materials. If we are required to use alternative materials in our product candidates, and in the event that such alternative materials are available to us, or if we choose to change the materials used in our product candidates in the future, we would need to validate the new manufacturing process and run comparability trials with any reformulated product candidate, which could delay future clinical trials and the submission for regulatory approval of our product candidates and negatively impact the development and potential commercialization of our product candidates.

If we fail to obtain the necessary regulatory approvals, or if such approvals are limited, we will not be able to commercialize our product candidates at all or to the extent we expected, and we will not generate any product revenues, or the product revenues we generate may be substantially less than expected.

Even if we comply with all FDA pre-approval regulatory requirements, the FDA may determine that our product candidates are not safe or effective, and we may never obtain regulatory approval for such product candidates. If we fail to obtain regulatory approval for some or all of our product candidates, we will have fewer commercial products, if any, and correspondingly lower product revenues, if any. Even if our product candidates receive regulatory approval, such approval may involve limitations on the indications and conditions of use or marketing claims for our products. Further, later discovery of previously unknown problems or AEs could result in additional regulatory restrictions, including addition of warnings or other statements on the product label or product withdrawal.

In jurisdictions outside the United States, we must receive marketing authorizations from the appropriate regulatory authorities before commercializing our product candidates. Regulatory approval processes outside the United States generally include requirements and risks similar to those associated with FDA approval, although the requirements and risks in certain jurisdictions may vary in certain important respects and in some instances, may exceed those associated with FDA approval.

Our failure to comply with extensive governmental regulation may significantly affect our operating results.

Even if we obtain regulatory approval for some or all of our product candidates, we will continue to be subject to extensive ongoing requirements by the FDA, as well as by applicable foreign, national, state and local agencies. These regulations will impact many aspects of our operations, including testing, research and development, manufacturing, safety,

Table of Contents

efficacy, labeling, storage, quality control, AE reporting, import and export, record keeping, approval, distribution, advertising and promotion of our future products. We must also submit new or supplemental applications and obtain FDA approval for certain changes to an approved product, product labeling or manufacturing process. Application holders must also submit advertising and other promotional material to the FDA and report on ongoing clinical trials. The FDA enforces post-marketing regulatory requirements, including cGMP requirements, through periodic unannounced inspections. We do not know whether we or our contractors will pass any future FDA inspections. Failure to pass an inspection could disrupt, delay or shut down our manufacturing operations. Failure to comply with applicable regulatory requirements could result in, among other things:

- administrative or judicial enforcement actions;
- changes to advertising;
- failure to obtain regulatory approvals for our product candidates;
- revocation or suspension of regulatory approvals of products;
- product seizures or recalls;
- court-ordered injunctions;
- import detentions;
- delay, interruption or suspension of product manufacturing, distribution, marketing and sales; or
- civil or criminal sanctions.

The discovery of previously unknown problems with any of our future approved products may result in restrictions on such products, including withdrawal from the market. In addition, the FDA may revisit and change its prior determinations with regard to the safety or efficacy of our future approved products. If the FDA's position changes, we may be required to change our labeling or cease to manufacture and market our future approved products. Even prior to any formal regulatory action, we could voluntarily decide to cease the distribution and sale or recall any of our future approved products if concerns about their safety or efficacy develop.

In their regulation of advertising and promotion, the FDA may issue correspondence alleging that some advertising or promotional practices are false, misleading or deceptive. The FDA is authorized to impose a wide array of sanctions on companies for such advertising and promotion practices, which could result in, for example:

- incurring substantial expenses, including fines, penalties, legal fees and costs to comply with the FDA's requirements;
- making changes to the methods of marketing and selling products; or
- taking FDA mandated corrective action, which may include placing advertisements or sending letters to physicians rescinding previous advertisements or promotions.

Improper promotional activities may also lead to investigations by federal or state prosecutors, and result in criminal and civil penalties. If we become subject to any of the above requirements, it could be damaging to our reputation and restrict our ability to sell or market our future approved products, and our business condition could be adversely affected. We may also incur significant expenses in defending ourselves.

Physicians may prescribe pharmaceutical or biologic products for uses that are not described in a product's labeling or differ from those tested by us and approved by the FDA. While such "off-label" uses are common and the FDA does not regulate physicians' choice of treatments, the FDA does restrict a manufacturer's communications about unapproved uses of its products. Companies cannot promote FDA-approved pharmaceutical or biologic products for unapproved uses, but under certain limited circumstances they may disseminate to practitioners articles published in peer-reviewed journals. To the extent allowed by the FDA, we may disseminate peer-reviewed articles on our future approved products to practitioners. If, however, our activities fail to comply with the FDA's regulations or guidelines, we may be subject to warnings from or enforcement action by the FDA or other regulatory or law enforcement authorities.

Any sales, marketing, and scientific/educational grant programs we may conduct in the future, must also comply with applicable requirements of various federal and state laws and regulations, including the federal Anti-Kickback Statute,

the federal civil False Claims Act, HIPAA's anti-fraud provisions, the federal Physician Payment Sunshine Act (Open Payments Program), and similar state fraud and abuse laws and regulations. Additional information about the scope of these requirements is offered under "Other U.S. Regulatory Requirements" in the Government Regulatory section above.

Table of Contents

Depending on the circumstances, failure to meet post-approval requirements can result in criminal prosecution, fines or other penalties, injunctions, recall or seizure of products, total or partial suspension of production, denial or withdrawal of pre-marketing product approvals, or refusal to allow us to enter into supply contracts, including government contracts. Any government investigation of alleged violations of law could require us to expend significant time and resources in response, and could generate negative publicity.

We are subject to significant regulation with respect to the manufacturing of our product candidates.

Components of a finished therapeutic product approved for commercial sale or used in clinical trials must be manufactured in accordance with cGMP. These regulations govern manufacturing processes and procedures and the implementation and operation of quality systems to control and assure the quality of investigational products and products approved for sale. Our facilities and quality systems and the facilities and quality systems of some or all of our third party manufacturers and suppliers, if any, must pass inspection for compliance with the applicable regulations as a condition of FDA approval of our products. In addition, the FDA may, at any time, audit or inspect our manufacturing facility or the facilities of our third party manufacturers, if any, or our associated quality systems, for compliance with the regulations applicable to the activities being conducted. If any such inspection or audit identifies a failure to comply with applicable regulations or if a violation of our product specifications or applicable regulation occurs independent of such an inspection or audit, we or the FDA may require remedial measures that may be costly and/or time consuming for us or a third party to implement and that may include the temporary or permanent suspension of a clinical trial or commercial sales, recalls, market withdrawals, seizures or the temporary or permanent closure of a facility. Any such remedial measures imposed upon us or third parties with whom we contract could materially harm our business.

If we fail to obtain or maintain orphan drug exclusivity for any of our product candidates, our competitors may sell products to treat the same conditions and our operations will be adversely impacted.

As part of our business strategy, we have obtained FDA orphan drug designation for FCX-007 and FCX-013. Under the Orphan Drug Act, the FDA may designate a product as an orphan drug if, among other things, it is intended to treat a rare disease or condition, defined as a patient population of fewer than 200,000 in the U.S. The first product to obtain FDA approval for a designated orphan drug for a given rare disease receives marketing exclusivity for that drug for the orphan-designated condition for a period of seven years. Orphan drug exclusive marketing rights may be lost if the FDA later determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the drug or biologic.

Because the extent and scope of patent protection for some of our product candidates is limited, orphan drug designation is especially important for our product candidates that are eligible for it. For eligible product candidates, we plan to rely, in part, on the exclusivity period under the Orphan Drug Act to maintain a competitive position. If we do not obtain orphan drug exclusivity for our product candidates that do not have broad patent protection, our competitors may then sell the same drug or biologic to treat the same condition which could adversely affect our operations.

Even though we have obtained orphan drug designation for FCX-007 and FCX-013 and even if we obtain such designation for other potential product candidates in the future, due to the uncertainties associated with developing biopharmaceutical products, we may not be the first to obtain regulatory approval for any particular orphan indication, which means that we may not obtain orphan drug exclusivity and could also potentially be blocked from approval until the first product’s orphan drug exclusivity period expires. Further, even if we obtain orphan drug exclusivity for a product, that exclusivity may not effectively protect the product from competition because different drugs can be approved for the same condition. Even after an orphan drug is approved and granted orphan drug exclusivity, the FDA can subsequently approve the same drug or biologic for the same condition if the FDA concludes that the later drug or

biologic is safer, more effective or makes a major contribution to patient care. Orphan drug designation does not shorten the regulatory review and approval process, nor does it provide any additional opportunities for review and guidance from the FDA during the review and approval process.

Even if we were to obtain approval for FCX-007 or FCX-013 with rare pediatric disease designation, the Rare Pediatric Disease PRV Program may no longer be in effect at the time of such approval.

FCX-007 has received rare pediatric disease designation from the FDA for the treatment of RDEB and FCX-013 has received rare pediatric disease designation from the FDA for the treatment of localized scleroderma. The FDA defines a “rare pediatric disease” as a serious or life-threatening disease in which the serious or life-threatening manifestations primarily affect individuals under the age of 18 years old, and that is a rare disease or condition as defined in the Orphan Drug Act (e.g., that affects fewer than 200,000 individuals in the U.S.). Under the FDA’s Rare Pediatric Disease PRV program, upon the approval

Table of Contents

of a NDA or BLA for the treatment of a rare pediatric disease, the sponsor of such application would be eligible for a Rare Pediatric Disease PRV that can be used to obtain priority review for a subsequent NDA or BLA. The PRV may be sold or transferred an unlimited number of times. Congress has extended the PRV program until 2020, but this program has been subject to criticism, including by the FDA, thus it is possible that even if we obtain approval for FCX-007 and FCX-013 and meet the current criteria for a rare pediatric disease PRV, the program may no longer be in effect at the time of approval.

We are largely dependent on the future commercial success of our product candidates.

Our ability to generate revenues and become profitable will depend in large part on the future commercial success of our product candidates. If any product that we commercialize in the future does not gain an adequate level of acceptance among physicians, patients and third parties, we may not generate significant product revenues or become profitable. Market acceptance by physicians, patients and third party payors of the products we may commercialize will depend on a number of factors, some of which are beyond our control, including:

- Their efficacy, safety and other potential advantages in relation to alternative treatments;
- Their relative convenience and ease of administration;
- The availability of adequate coverage or reimbursement by third parties, such as insurance companies and other healthcare payors, and by government healthcare programs, including Medicare and Medicaid;
- The prevalence and severity of adverse events;
- Their cost of treatment in relation to alternative treatments, including generic or biosimilar products;
- The extent and strength of our third party manufacturer and supplier support;
- The extent and strength of marketing and distribution support;
- The limitations or warnings contained in a product's FDA approved labeling; and
- Distribution and use restrictions imposed by the FDA or that are part of a REMS or voluntary risk management plan.

For example, even if our products have been approved by the FDA, physicians and patients may not immediately be receptive to them and may be slow to adopt them. In addition, even though we believe our product candidates have significant advantages to other treatment options, because no head-to-head trials comparing our product candidates to competing products will have been conducted, the prescribing information approved by the FDA would not contain claims that our product is safer or more effective than competitive products. Accordingly, we may experience limitations in promoting any comparative advantages our products may have. If our products do not achieve an adequate level of acceptance among physicians, patients and third party payors, we may not generate meaningful revenues and we may not become profitable.

Negative public opinion and increased regulatory scrutiny of gene therapies may damage public perception of our product candidates or adversely affect our ability to conduct our business or obtain regulatory approvals for our product candidates.

Public perception may be influenced by claims that gene therapies are unsafe, and gene therapies may not gain the acceptance of the public or the medical community. In particular, our success will depend upon appropriate physicians prescribing treatments that involve the use of our product candidates in lieu of, or in addition to, existing treatments they are already familiar with and for which greater clinical data may be available. More restrictive government regulations or negative public opinion would have a negative effect on our business or financial condition and may delay or impair the development and commercialization of our product candidates or demand for any products we may develop. Adverse events in our clinical trials, even if not ultimately attributable to our product candidates, and the resulting publicity could result in increased governmental regulation, unfavorable public perception, potential regulatory delays in the testing or approval of our potential product candidates, stricter labeling requirements for those product candidates that are approved and a decrease in demand for any such product candidates.

Future sales of our products are subject to adequate coverage, pricing and reimbursement from third-party payors, which are subject to increasing and intense pressure from political, social, competitive and other sources. Our inability

to obtain and maintain adequate coverage, pricing or reimbursement, could have an adverse effect on our business. Future sales of our product candidates, should they receive regulatory approval and be commercialized, are dependent, in large part, on the availability and extent of coverage, pricing and reimbursement from government health administration authorities, private health insurers and other organizations. When a new pharmaceutical or biologic product is approved, the availability of government and private reimbursement for that product may be uncertain, as is the pricing and amount for which

Table of Contents

that product will be reimbursed. The manner and level at which reimbursement is provided for services related to our product candidates (e.g., for administration of our products to patients) is also important. Inadequate reimbursement for such services may lead to physician resistance and adversely affect our ability to market or sell our products.

Pricing and reimbursement for our products and services related to our products may be adversely affected by a number of factors, including:

- changes in federal, state or foreign government regulations or private third-party payors' reimbursement policies;
- pressure by employers on private health insurance plans to reduce costs; and
- consolidation and increasing assertiveness of payors, including managed care organizations, health insurers, pharmacy benefit managers, government health administration authorities, private health insurers and other organizations, seeking price discounts or rebates in connection with the placement of our products on their formularies and, in some cases, the imposition of restrictions on access or coverage of particular drugs or biologics pricing determined based on perceived value.

Our failure to maintain adequate coverage, pricing, or reimbursement for our products and services related to our products would have an adverse effect on our business, revenues and results of operation, could curtail or eliminate our ability to adequately fund research and development programs for the discovery and commercialization of new product candidates, and could cause a decline in our stock price.

Drug pricing and other health care costs are under significant scrutiny in the U.S. and are subject to intense political and societal pressures which we anticipate will continue and escalate on a global basis. As a result, our business and reputation may be harmed, our stock price may be adversely impacted and experience periods of volatility, and our results of operations may be adversely impacted.

If the market opportunities for our product candidates are smaller than we believe they are, our results of operations may be adversely affected and our business may suffer.

We focus our research and product development on treatments of diseases affecting the skin and connective tissue. Our projections of both the number of people who have these diseases, as well as the subset of people with these diseases who have the potential to benefit from treatment with our product candidates, are based on estimates. These estimates may prove to be incorrect and new studies or clinical trials may change the estimated incidence or prevalence of these diseases. The number of patients in the U.S. and elsewhere may turn out to be lower than expected, may not be otherwise amenable to treatment with our products, or new patients may become increasingly difficult to identify or gain access to, all of which would adversely affect our results of operations and our business.

If any of our approved products were to become the subject of problems related to their efficacy, safety, or otherwise, our business would be seriously harmed.

Any of our product candidates that may be approved by the FDA will be subject to continual review by the FDA, and we cannot assure you that newly discovered or developed safety issues will not arise. For all of our product candidates, the FDA has required us to pay special attention to potential skin cancer and hypersensitivity reactions at the site of injection and, while we have seen no issues to date, we cannot rule out that issues may arise in the future. With the use of any newly marketed drug by a wider patient population, serious AEs may occur from time to time that initially do not appear to relate to the drug itself. Any safety issues could cause us to suspend or cease marketing of our approved products, cause us to modify how we market our approved products, subject us to substantial liabilities, and adversely affect our financial condition and business.

Our product candidates for which we intend to seek approval as biological products may face competition sooner than expected.

With the enactment of the BPCIA, abbreviated pathways for approval of biosimilar and interchangeable biological products were created. The BPCIA establishes legal authority for the FDA to review and approve biosimilars for marketing, as well as biosimilars that have been designated as “interchangeable” with a previously approved biologic, or reference product. Under the BPCIA, an application for a biosimilar product cannot be approved by the FDA until 12 years after the reference product was approved under a full BLA. This period of regulatory exclusivity runs concurrently with, but is independent of, periods of patent protection for the reference product.

Table of Contents

We believe that any of our product candidates approved as a biological product under a full BLA should qualify for a 12-year period of exclusivity. However:

- the United States Congress could amend the BPCIA to significantly shorten this exclusivity period as has been previously proposed; and

- a potential competitor could seek and obtain approval of its own BLA during our exclusivity period instead of seeking approval of a biosimilar version.

The BPCIA is complex and its provisions continue to be interpreted and implemented by the FDA and U.S. courts. As a result, the ultimate impact, implementation and implications of the BPCIA are subject to uncertainty and could compromise the future commercial prospects for our biological products. Moreover, it is not yet clear the extent to which a biosimilar, once approved, may be substituted for any one of our reference products in a way that is similar to traditional generic substitution for pharmaceutical products; this will depend on a number of marketplace and regulatory factors that are still developing at both the federal and state levels of government.

We may be liable for product liability claims not covered by insurance.

Physicians, patients and clinical trial participants who have used our products in the past or who use them in the future may bring product liability claims against us. While we have taken, and continue to take, what we believe are appropriate precautions, we may be unable to avoid significant liability exposure. We currently keep in force product liability insurance, although such insurance may not be adequate to fully cover any potential claims or may lapse in accordance with its terms prior to the assertion of claims. We may be unable to obtain product liability insurance in the future, or we may be unable to do so on acceptable terms. Any insurance we obtain or have obtained in the past may not provide adequate coverage against any asserted claims. In addition, regardless of merit or eventual outcome, product liability claims may result in:

- diversion of management’s time and attention;
- expenditure of large amounts of cash on legal fees, expenses and payment of damages;
- decreased demand for our products or any of our future products and services; or
- injury to our reputation.

If we are the subject of any future product liability claims, our business could be adversely affected, and if these claims are in excess of insurance coverage, if any, that we may possess, our financial position will suffer.

Risks Related to Our Dependence on Third Parties

We will incur additional expenses in connection with our exclusive channel collaboration agreements with Intrexon. Pursuant to our exclusive channel collaboration agreements with Intrexon, we are responsible for future research, development and commercialization expenses of product candidates developed under such collaborations, including FCX-007, FCX-013 and our gene therapy program for arthritis and related conditions, the effect of which we expect will increase the level of our overall research and development expenses going forward. Although all manufacturing, pre-clinical studies and human clinical trials are expensive and difficult to design and implement, costs associated with the manufacturing, research and development of gene therapy product candidates are generally greater in comparison to small molecule product candidates. We have added personnel and expect to add additional personnel, either directly or through consulting arrangements, to support our exclusive channel collaborations with Intrexon. Because development activities are determined pursuant to a joint steering committee comprised of Intrexon and ourselves and we have limited experience, future development costs associated with our product candidates may be difficult to anticipate and exceed our expectations. Our actual cash requirements may vary materially from our current expectations for a number of other factors that may include, but are not limited to, unanticipated technical challenges, changes in the focus and direction of our development activities or adjustments necessitated by changes in the

competitive landscape in which we operate. If we are unable to continue to financially support such collaborations due to our own working capital constraints, we may be forced to discontinue the collaborations or delay our activities.

Table of Contents

We may not be able to retain the exclusive rights licensed to us by Intrexon to develop and commercialize our product candidates.

Pursuant to our exclusive channel collaboration agreements, we are using Intrexon's technology in connection with all of our product candidates. The collaboration agreements grant us a license to use patents and other intellectual property of Intrexon in connection with the research, development, and commercialization of collaboration products within "Fields" that we set forth above in the "Item 1. Business - Intrexon Collaboration".

The exclusive channel collaboration agreements may be terminated by Intrexon if we fail to exercise diligent efforts in developing products through the collaborations or if we elect not to pursue the development of a therapy in a "Field" identified by Intrexon that is a "Superior Therapy" as defined in the collaboration agreements. Upon such termination, the product candidates covered by the applicable exclusive channel collaboration agreement in active and ongoing Phase 2 or 3 clinical trials or later stage development through the exclusive channel collaboration agreement shall be entitled to be continued by us with a continuation of the related royalties for such product candidates, and all rights to products covered by the exclusive channel collaboration agreement still in an earlier stage of development shall revert to Intrexon.

There can be no assurance that we will be able to successfully perform under the exclusive channel collaboration agreements and if any of the agreements are terminated it may prevent us from achieving our business objectives and our business may be harmed.

Any manufacturing difficulties, disruptions or delays could adversely affect our ability to conduct our clinical trials.

Manufacturing biologic products is difficult, complex and highly regulated. During 2016, we began to manufacture the pre-clinical supply of our FCX-013 product candidate in our facility in Exton, PA. We lease and operate our own manufacturing facility located in Exton, Pennsylvania. We have historically used this facility to manufacture our non-genetically modified products and during 2016 began using this facility for pre-clinical manufacturing of our gene therapy product candidate, FCX-013. We designated our Exton, PA cGMP manufacturing facility in Exton, PA as the production site for FCX-007 in the fourth quarter of 2017, and are currently producing drug product for clinical development of FCX-007 and FCX-013 at that facility. We also plan to produce drug product for commercial sales of FCX-007 and FCX-013, if approved, at this facility. Our ability to adequately and timely manufacture and supply our product candidates is dependent on the operation of our sole facility which may be impacted by, among other things:

- availability, performance, or contamination of raw materials and components used in the manufacturing process, particularly those for which we have no other source or supplier;
- capacity of our facility and the facility of any of our suppliers;
- the performance of information technology systems;
- compliance with regulatory requirements;
- inclement weather and natural disasters;
- changes in forecasts of future demand for product components;
- timing and actual number of production runs for product components;
- potential facility contamination by microorganisms or viruses;
- updating of manufacturing specifications; and
- product quality success rates and yields.

If the efficient manufacture and supply of our product candidates is interrupted, we may experience delayed shipments or supply constraints, which may materially impact our ongoing and future pre-clinical studies and clinical trials.

Our manufacturing processes must undergo a potentially lengthy FDA approval process, as well as other regulatory approval processes, and are subject to continued review by the FDA and other regulatory authorities. It is a multi-year process

Table of Contents

to build and license a new manufacturing facility and it can take significant time to qualify and license a contract manufacturer, if needed.

If regulatory authorities determine that we or certain of our third-party service providers have violated regulations or if they restrict, suspend or revoke our prior approvals, they could prohibit us from manufacturing our products or conducting clinical trials or selling our marketed products until we or the affected third-party service providers comply, or indefinitely. Because our third-party service providers are subject to the FDA and, potentially, in the future, foreign regulatory authorities, alternative qualified third-party service providers may not be available on a timely basis or at all. If we or our third-party service providers cease or interrupt production or if our third-party service providers fail to supply materials, products or services to us, we may experience delayed shipments, and supply constraints for our products.

We receive vectors from our contract manufacturers, but the majority of our research, development and manufacturing operations depend on one facility for all of our product candidates. If this facility is destroyed or is out of operation for a substantial period of time, our business may be adversely impacted.

We currently conduct our research, development and manufacturing operations related to our product candidates in our facility located in Exton, Pennsylvania. Previously we outsourced certain manufacturing of our genetically-modified product candidate FCX-007, to a contract manufacturer with a facility located in Mountain View, California.

If regulatory, manufacturing or other problems require us to discontinue production at our Exton, PA facility, we will not be able to have supplies for our pre-clinical studies and clinical trials, which would adversely impact our business. If the facility or the equipment in it is significantly damaged or destroyed by fire, flood, power loss or similar events, we may not be able to quickly or inexpensively replace our facility. In the event of a temporary or protracted loss of either facility or equipment, we might not be able to transfer manufacturing to a third party. Even if we could transfer manufacturing to a third party, the shift would likely be expensive and time-consuming, particularly since the new facility would need to comply with necessary regulatory requirements.

Risks Related to Our Intellectual Property

If we or our licensors are unable to protect our intellectual property rights or if our intellectual property rights are inadequate for our technologies and product candidates, our competitive position could be harmed.

Our commercial success will depend in large part on our, and our licensors, ability to obtain and maintain patent and other intellectual property protection in the U.S. and other countries with respect to our proprietary technology and our product candidates. We rely on trade secret, patent, copyright and trademark laws, and confidentiality, licensing and other agreements with employees and third parties, all of which offer only limited protection. We seek to protect our proprietary position by filing and prosecuting patent applications in the U.S. and abroad related to our novel technologies and product candidates that are important to our business.

The patent positions of biopharmaceutical companies generally are highly uncertain, involve complex legal and factual questions and have in recent years been the subject of much litigation. As a result, the issuance, scope, validity, enforceability and commercial value of our patents, including those patent rights licensed to us by third parties, are highly uncertain. The steps we or our licensors have taken to protect our proprietary rights may not be adequate to preclude misappropriation of our proprietary information or infringement of our intellectual property rights, both inside and outside the U.S. Further, the examination process may require us or our licensors to narrow the claims for our pending patent applications, which may limit the scope of patent protection that may be obtained if these applications issue. The rights already granted under any of our currently issued patents or those licensed to us and

those that may be granted under future issued patents may not provide us with the proprietary protection or competitive advantages we are seeking. If we or our licensors are unable to obtain and maintain patent protection for our technology and products, or if the scope of the patent protection obtained is not sufficient, our competitors could develop and commercialize technology and products similar or superior to ours, and our ability to successfully commercialize our technology and products may be adversely affected. It is also possible that we or our licensors will fail to identify patentable aspects of inventions made in the course of our development and commercialization activities before it is too late to obtain patent protection on them.

With respect to patent rights, we do not know whether any of the pending patent applications for any of our therapies will result in the issuance of patents that protect our technology or products, or if any of our or our licensors' issued patents will effectively prevent others from commercializing competitive technologies and product candidates. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the U.S. and other jurisdictions are typically not published until 18 months after filing or in some cases not at all, until they are issued as a patent. Therefore, we

Table of Contents

cannot be certain that we or our licensors were the first to make the inventions claimed in our owned or licensed patents or pending patent applications, or that we or our licensors were the first to file for patent protection of such inventions.

Our issued patents, those that may be issued in the future or those licensed or acquired by us, may be challenged, invalidated or circumvented, and the rights granted under any issued patent may not provide us with proprietary protection or competitive advantages against competitors with similar technology. In particular, we do not know if competitors will be able to design variations on our treatment methods to circumvent our current and anticipated patent claims. Furthermore, competitors may independently develop similar technologies or duplicate any technology developed by us.

Our pending applications cannot be enforced against third parties practicing the technology claimed in such applications unless and until a patent issues from such applications. Because the issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, issued patents that we own or have licensed from third parties may be challenged in the courts or patent offices in the U.S. and abroad. Such challenges may result in the loss of patent protection, the narrowing of claims in such patents or the invalidity or unenforceability of such patents, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection for our technology and products. Protecting against the unauthorized use of our or our licensor's patented technology, trademarks and other intellectual property rights is expensive, difficult and may in some cases not be possible. In some cases, it may be difficult or impossible to detect third-party infringement or misappropriation of our intellectual property rights, even in relation to issued patent claims, and proving any such infringement may be even more difficult.

Third parties may initiate legal proceedings alleging that we are infringing their intellectual property rights, the outcome of which would be uncertain and could harm our business.

Our commercial success depends upon our ability to develop, manufacture, and if approved, market and sell our product candidates and to use our related proprietary technologies. We may become party to, or threatened with, future adversarial proceedings or litigation regarding intellectual property rights with respect to our product candidates, including interference, post grant review, inter partes review or derivation proceedings before the U.S. Patent and Trademark Office (USPTO). Third parties may assert infringement claims against us based on existing patents or patents that may be granted in the future. If we are found to infringe a third party's intellectual property rights, we could be required to obtain a license from such third party in order to be able to commercialize any of our product candidates that obtain regulatory approval. However, we may not be able to obtain any required license on commercially reasonable terms or at all. Under certain circumstances, we could be forced, including by court order, to cease commercializing our future approved products and then expend time and funding to redesign such products so that such products do not infringe others' patents while still allowing us to compete in the market with a substantially similar product. In addition, in any such proceeding or litigation, we could be found liable for monetary damages. A finding of infringement could prevent us from commercializing any of our product candidates that obtain regulatory approval or force us to cease some of our business operations, which could materially harm our business. Any claims by third parties that we have misappropriated their confidential information or trade secrets could have a similar negative impact on our business. In addition, our involvement in any of these proceedings may cause us to incur substantial costs and result in diversion of management and technical personnel. Furthermore, parties making claims against us may be able to obtain injunctive or other equitable relief that could effectively block our ability to develop, commercialize and sell products, and could result in the award of substantial damages against us.

We believe that use of our product candidates in clinical trials falls within the scope of the exemptions provided by 35 U.S.C. Section 271(e) in the U.S., which exempts from patent infringement liability activities reasonably related to the development and submission of information to the FDA. As our product candidates progress toward regulatory approval and commercialization, the possibility of a patent infringement claim against us increases. We attempt to ensure that our product candidates and the methods we employ to manufacture them, as well as the methods for their

use we intend to promote, do not infringe other parties' patents and other proprietary rights. There can be no assurance they do not, however, and competitors or other parties may assert that we infringe their proprietary rights in any event.

We may not be able to protect our intellectual property rights throughout the world.

Filing, prosecuting and defending patents on all of our product candidates throughout the world would be prohibitively expensive, and our or our licensors' intellectual property rights in some countries outside the U.S. can be less extensive than those in the U.S. In addition, the laws and practices of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the U.S. Consequently, we and our licensors may not be able to prevent third parties from practicing our and our licensors' inventions in all countries outside the U.S., or from selling or importing products made using our and our licensors' inventions in and into the U.S. or other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products, and may export otherwise infringing

Table of Contents

products to territories where we or our licensors have patent protection, but where enforcement is not as strong as that in the U.S. These products may compete with our products in jurisdictions where we do not have any issued patents and our patent claims or other intellectual property rights may not be effective or sufficient to prevent them from so competing.

Many companies have encountered significant problems in protecting and defending intellectual property rights in certain foreign jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents and other intellectual property protection, particularly those relating to biopharmaceuticals, which could make it difficult for us to stop the infringement of our or our licensor's patents or marketing of competing products in violation of our proprietary rights generally in those countries. Proceedings to enforce our patent rights in foreign jurisdictions could result in substantial cost and divert our efforts and attention from other aspects of our business, could put our and our licensors' patents at risk of being invalidated or interpreted narrowly and our and our licensors' patent applications at risk of not issuing and could provoke third parties to assert claims against us or our licensors. We or our licensors may not prevail in any lawsuits that we or our licensors initiate and the damages or other remedies awarded, if any, may not be commercially meaningful.

The laws of certain foreign countries may not protect our rights to the same extent as the laws of the U.S., and these foreign laws may also be subject to change. For example, methods of treatment and manufacturing processes may not be patentable in certain jurisdictions, and the requirements for patentability may differ in certain countries, particularly developing countries. Furthermore, competitors may challenge the scope, validity or enforceability of our or our licensors' patents, requiring us or our licensors to engage in complex, lengthy and costly litigation or other proceedings. Biopharmaceutical companies may develop, seek approval for, and launch biosimilar versions of our products. Many countries, including European Union countries, India, Japan and China, have compulsory licensing laws under which a patent owner may be compelled under certain circumstances to grant licenses to third parties. In those countries, we and our licensors may have limited remedies if patents are infringed or if we or our licensors are compelled to grant a license to a third party, which could materially diminish the value of those patents. This could limit our potential revenue opportunities. Accordingly, our and our licensors' efforts to enforce intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we own or license.

Patent terms may be inadequate to protect our competitive position on our product candidates for an adequate amount of time.

Given the amount of time required for the development, testing and regulatory review of our product candidates, patents protecting such product candidates might expire before or shortly after such product candidates are commercialized. We expect to seek extensions of patent terms in the U.S. and, if available, in other countries where we are prosecuting patents. In the U.S., the Drug Price Competition and Patent Term Restoration Act of 1984 permits a patent term extension of up to five years beyond the normal expiration of the patent, which is limited to the approved indication (or any additional indications approved during the period of extension). However, the applicable authorities, including the FDA and the USPTO in the U.S., and any equivalent regulatory authority in other countries, may not agree with our assessment of whether such extensions are available, and may refuse to grant extensions to our patents, or may grant more limited extensions than we request. If this occurs, our competitors may be able to take advantage of our investment in development and clinical trials by referencing our clinical and pre-clinical data and launch their product earlier than might otherwise be the case.

Changes in patent law, including recent patent reform legislation, could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents.
As is the case with other biopharmaceutical companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the biopharmaceutical industry involve technological and legal

complexity, and obtaining and enforcing pharmaceutical patents is costly, time-consuming, and inherently uncertain. Changes in either the patent laws or interpretation of the patent laws in the U.S. and other countries may diminish the value of our patents or narrow the scope of our patent protection. For example, the U.S. Supreme Court has ruled on several patent cases in recent years, either narrowing the scope of patent protection available in certain circumstances or weakening the rights of patent owners in certain situations. In addition to increasing uncertainty with regard to our and our licensors' ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on decisions by Congress, the federal courts, and the USPTO, the laws and regulations governing patents could change in unpredictable ways that would weaken our and our licensors' ability to obtain new patents or to enforce existing patents and patents we and our licensors may obtain in the future. Recent patent reform legislation could increase the uncertainties and costs surrounding the prosecution of our and our licensors' patent applications and the enforcement or defense of our or our licensors' issued patents.

Table of Contents

In September 2011, the Leahy-Smith America Invents Act (the Leahy-Smith Act), was signed into law. The Leahy-Smith Act includes a number of significant changes to U.S. patent law. These include provisions that affect the way patent applications will be prosecuted and may also affect patent litigation. In particular, under the Leahy-Smith Act, the U.S. transitioned in March 2013 to a “first to file” system in which the first inventor to file a patent application will be entitled to the patent. Third parties are allowed to submit prior art before the issuance of a patent by the USPTO and may become involved in opposition, derivation, reexamination, post grant review, inter-partes review or interference proceedings challenging our patent rights or the patent rights of our licensors. An adverse determination in any such submission, proceeding or litigation could reduce the scope of, or invalidate, our or our licensors’ patent rights, which could adversely affect our competitive position. The Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents and those licensed to us.

Obtaining and maintaining our patent protection depends on compliance with various procedural, document submissions, fee payment and other requirements imposed by governmental patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.

Periodic maintenance fees on any issued patent are due to be paid to the USPTO and foreign patent agencies in several stages over the lifetime of the patent. The USPTO and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. While an inadvertent lapse can in many cases be cured by payment of a late fee or by other means in accordance with the applicable rules, there are situations in which noncompliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. Non-compliance events that could result in abandonment or lapse of a patent or patent application include, but are not limited to, failure to respond to official actions within prescribed time limits, non-payment of fees and failure to properly legalize and submit formal documents. If we or our licensors fail to maintain the patents and patent applications covering our product candidates, our competitive position would be adversely affected.

If we are unable to protect the confidentiality of our trade secrets, our business and competitive position would be harmed.

To protect our proprietary know-how and our inventions for which patents may be unobtainable or difficult to obtain, and to maintain our competitive position, we rely on trade secret protection and confidentiality agreements. To this end, it is our general policy to require our employees, consultants, advisors, and contractors to enter into agreements that prohibit the disclosure of confidential information and, where applicable, require disclosure and assignment to us of the ideas, developments, discoveries, and inventions important to our business. These agreements may not provide adequate protection for our trade secrets, know-how or other proprietary information in the event of any unauthorized use or disclosure or the lawful development by others of such information. Moreover, we may not be able to obtain adequate remedies for any breaches of these agreements. Our trade secrets may also be obtained by third parties by other means, such as breaches of our physical or computer security systems. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive and time-consuming, and the outcome is unpredictable. In addition, some courts inside and outside the United States are less willing or unwilling to protect trade secrets. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent them, or those to whom they communicate it, from using that technology or information to compete with us. If any of our trade secrets, know-how or other proprietary information is disclosed, the value of our trade secrets, know-how and other proprietary rights would be significantly impaired and our business and competitive position would suffer.

We may become involved in lawsuits to protect or enforce our intellectual property, which could be expensive, time consuming and unsuccessful and have a material adverse effect on the success of our business.

Competitors may infringe our patents or misappropriate or otherwise violate our intellectual property rights. To counter infringement or unauthorized use, litigation may be necessary in the future to enforce or defend our intellectual property rights, to protect our trade secrets or to determine the validity and scope of our own intellectual property rights or the proprietary rights of others. Also, third parties may initiate legal proceedings against us or our licensors to challenge the validity or scope of intellectual property rights we own or control. These proceedings can be expensive and time consuming. Many of our current and potential competitors have the ability to dedicate substantially greater resources to defend their intellectual property rights than we can. Accordingly, despite our efforts, we may not be able to prevent third parties from infringing upon or misappropriating our intellectual property. Litigation could result in substantial costs and diversion of management resources, which could harm our business and financial results. In addition, in an infringement proceeding, a court may decide that a patent owned by or licensed to us is invalid or unenforceable, or may refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the technology in question. An adverse result in any litigation proceeding could put one or more of our patents at risk of being invalidated, held unenforceable or interpreted

Table of Contents

narrowly. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. There could also be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a material adverse effect on the price of shares of our common stock.

We may be subject to claims by third parties asserting that our licensors, employees or we have misappropriated their intellectual property, or claiming ownership of what we regard as our own intellectual property.

Many of our employees and our licensors' employees, including our senior management, were previously employed at universities or at other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that our employees do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that we or these employees have used or disclosed intellectual property, including trade secrets or other proprietary information, of any such third party. Litigation may be necessary to defend against such claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel or sustain damages. Such intellectual property rights could be awarded to a third party, and we could be required to obtain a license from such third party to commercialize our technology or products. Such a license may not be available on commercially reasonable terms or at all. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management.

Intellectual property rights do not necessarily address all potential threats to our competitive advantage.

The degree of future protection afforded by our intellectual property rights is uncertain because intellectual property rights have limitations, and may not adequately protect our business, or permit us to maintain our competitive advantage. The following examples are illustrative:

- others may be able to make biologics that are the same as or similar to our product candidates, but that are not covered by the claims of the patents that we own or have exclusively licensed;
- we or our licensors or any strategic partners might not have been the first to make the inventions covered by the issued patents or pending patent applications that we own or have exclusively licensed;
- we or our licensors might not have been the first to file patent applications covering certain of our inventions;
- others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing our intellectual property rights;
- it is possible that our pending patent applications will not lead to issued patents;issued patents that we own or have exclusively licensed may not provide us with any competitive advantages, or may be held invalid or unenforceable as a result of legal challenges;
- our competitors might conduct research and development activities in the U.S. and other countries that provide a safe harbor from patent infringement claims for certain research and development activities, as well as in countries where we do not have patent rights and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- we may not develop additional proprietary technologies that are patentable; and
- the patents of others may have an adverse effect on our business.

Risks Related to Business Operations

We are dependent on our executives and other key professionals and the loss of any of these individuals could harm our business.

We are dependent on the efforts of our executives and other key scientific, manufacturing and quality personnel. The loss of any of these individuals, or our inability to recruit and train additional key personnel in a timely manner, could materially and adversely affect our business and our future prospects. A loss of one or more of our current executives or other key professionals could severely and negatively impact our operations. All of our employees, including our chief executive

Table of Contents

officer, are employed "at-will," and any of them may elect to pursue other opportunities at any time. We have no present intention of obtaining key man life insurance on any of our executive officers or key professionals.

We may need to attract, train and retain additional experienced executives and other key professionals in the future.

In the future, we may need to seek additional executives and other key professionals. There is a high demand for experienced executive, scientific, manufacturing and quality personnel in our industry. We do not know whether we will be able to attract, train and retain such experienced personnel in the future, which could have a material adverse effect on our business, financial condition and results of operations.

Our business may be adversely affected by current and potential future healthcare reforms.

In the U.S., federal and state legislatures, health agencies and third-party payors continue to focus on containing the cost of health care. Legislative and regulatory proposals and enactments to reform health care insurance programs could significantly influence the manner in which our product candidates, if approved, are prescribed and purchased. For example, the Affordable Care Act has changed the way health care is paid for by both governmental and private insurers, including increased rebates owed by manufacturers under the Medicaid Drug Rebate Program, annual fees and taxes on manufacturers of certain branded prescription drugs, the requirement that manufacturers participate in a discount program for certain outpatient drugs under Medicare Part D and the expansion of the number of hospitals eligible for discounts under Section 340B of the Public Health Service Act. In addition, there have been efforts by the Trump Administration to repeal or replace certain aspects of the Affordable Care Act and to alter the implementation of the Affordable Care Act and related laws. For example, the Tax Cuts and Jobs Act enacted on December 22, 2017, eliminated the tax-based shared responsibility payment for individuals who fail to maintain minimum essential coverage under section 5000A of the Code, commonly referred to as the "individual mandate," effective January 1, 2019. Further, the Bipartisan Budget Act of 2018, among other things, amended the Medicare statute to reduce the coverage gap in most Medicare drug plans, commonly known as the "donut hole," by raising the required manufacturer point-of-sale discount from 50% to 70% off the negotiated price effective as of January 1, 2019. Further legislative changes, regulatory changes, and judicial challenges related to the Affordable Care Act remain possible.

There is also significant economic pressure on state budgets that may result in states increasingly seeking to achieve budget savings through mechanisms that limit coverage or payment for certain drugs. In recent years, some states have considered legislation and ballot initiatives that would control the prices of drugs, including laws to allow importation of pharmaceutical products from lower cost jurisdictions outside the U.S. and laws intended to impose price controls on state drug purchases. State Medicaid programs are increasingly requesting manufacturers to pay supplemental rebates and requiring prior authorization by the state program for use of any drug for which supplemental rebates are not being paid. Government efforts to reduce Medicaid expenses may lead to increased use of managed care organizations by Medicaid programs. This may result in managed care organizations influencing prescription decisions for a larger segment of the population and a corresponding constraint on prices and reimbursement for our product candidates, if approved. In addition, under the Affordable Care Act, as states implement their health care marketplaces or operate under the federal exchange, the impact on drug manufacturers will depend in part on the formulary and benefit design decisions made by insurance sponsors or plans participating in these programs. It is possible that we may need to provide discounts or rebates to such plans in order to maintain favorable formulary access for our future product candidates, if approved, which could have an adverse impact on our sales and results of operations.

If we fail to comply with federal and state healthcare laws, including fraud and abuse and health information privacy and security laws, we could face substantial penalties and our business, results of operations, financial condition and prospects could be adversely affected.

As a biopharmaceutical company, even though we do not bill directly to Medicare, Medicaid or other third-party payors, certain federal and state healthcare laws and regulations pertaining to fraud and abuse and patients' rights are and will be applicable to our business. For example, we could be subject to healthcare fraud and abuse and patient privacy regulation by both the federal government and the states in which we conduct our business. The laws that may affect our ability to operate include, among others:

- The federal Anti-Kickback Statute, which constrains our business activities, including our marketing practices, educational programs, pricing policies, and relationships with healthcare providers or other entities, by prohibiting, among other things, soliciting, receiving, offering or paying remuneration, directly or indirectly, to induce, or in return for, either the referral of an individual or the purchase or recommendation of an item or service reimbursable under a federal healthcare program, such as the Medicare and Medicaid programs;

Table of Contents

- Federal civil false claims laws and civil monetary penalty laws, which prohibit, individuals or entities from, among other things, knowingly presenting, or causing to be presented, claims for payment of government funds, or other third-party payors that are false or fraudulent. Criminal prosecution is also possible for making or presenting a false or fictitious or fraudulent claim to the federal government;

- HIPAA's anti-fraud provisions, which prohibit, among other things, executing a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters;

- HIPAA's privacy and security provisions, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009 (HITECH), and its implementing regulations, which imposes certain requirements relating to the privacy, security and transmission of individually identifiable health information;

- The federal Physician Payment Sunshine Act, being implemented as the Open Payments Program, which requires manufacturers of drugs, devices, biologics, and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program (with certain exceptions) to report annually to CMS information related to direct or indirect payments and other transfers of value to physicians and teaching hospitals, as well as ownership and investment interests held in the company by physicians and their immediate family members; and

- Analogous state and local laws and regulations, such as state anti-kickback and false claims laws, which may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers; state laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government or otherwise restrict payments that may be made to healthcare providers; state laws that restrict the ability of manufacturers to offer co-pay support to patients for certain prescription drugs; state that require drug manufacturers to report information related to clinical trials, or information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures; state laws and local ordinances that require identification or licensing of sales representatives.

Additional information about the scope of these requirements is offered under "Other U.S. Regulatory Requirements" in the Government Regulatory section above. Because of the breadth of these laws and the narrowness of the statutory exceptions and safe harbors available, it is possible that some of our business activities could be subject to challenge under one or more of such laws. For example, the federal Anti-Kickback Statute is subject to evolving interpretations and has been applied by government enforcement officials to a number of common business arrangements in the pharmaceutical industry. The federal government has enforced the Anti-Kickback Statute to reach large settlements with pharmaceutical manufacturers based on allegedly sham consultant arrangements with physicians and other arrangements that are common in our industry. The government can establish a violation of the Anti-Kickback Statute without proving that a person or entity had actual knowledge of the law or specific intent to violate it. There are a number of statutory exceptions and regulatory safe harbors protecting certain common activities from prosecution or other regulatory sanctions, but the exceptions and safe harbors are drawn narrowly. Failure to meet all of the requirements of a particular statutory exception or regulatory safe harbor does not make the conduct per se illegal under the Anti-Kickback Statute, but the legality of the arrangement will be evaluated on a case-by- case basis based on the totality of the facts and circumstances. In addition, a claim to a federal health care program that includes items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the federal False Claims Act. In addition, settlements with DOJ or other law enforcement agencies have forced healthcare companies to agree to additional compliance and reporting requirements as part of a consent decree or corporate integrity agreement.

To the extent that any of our product candidates is ultimately sold in a foreign country, we may be subject to similar foreign laws and regulations.

If we or our operations are found to be in violation of any of the laws described above or any other governmental regulations that apply to us, we may be subject to penalties, including administrative, civil and criminal penalties, damages, fines, exclusion from participation in United States federal or state health care programs, such as Medicare

and Medicaid, and the curtailment or restructuring of our operations any of which could materially adversely affect our ability to operate our business and our financial results. Although compliance programs can mitigate the risk of investigation and prosecution for violations of these laws, the risks cannot be entirely eliminated. Any action against us for violation of these laws, even if we successfully defend against it, could cause us to incur significant legal expenses and divert our management's attention from the operation of our business. Moreover, achieving and sustaining compliance with applicable federal and state privacy, security and fraud laws may prove costly.

Table of Contents

If we fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could have a material adverse effect on the success of our business.

We are subject to numerous environmental, health and safety laws and regulations, including those governing manufacturing and laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. Our operations involve the use of hazardous and flammable materials, including chemicals and biological materials. Our operations also produce hazardous waste products. We generally contract with third parties for the disposal of these materials and wastes. We cannot eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting from our use of hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties.

Although we maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials or other work-related injuries, this insurance may not provide adequate coverage against potential liabilities. In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. These current or future laws and regulations may impair our research, development or production efforts. Failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions.

Our business and operations would suffer in the event of computer system failures.

Despite the implementation of security measures, our internal computer systems, and those of our contract research organizations, contract manufacturing organization, and other third parties on which we rely, are vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. System failures, accidents or security breaches could cause interruptions in our operations or the unauthorized transfer of our proprietary information, and could result in a material disruption of our research, pre-clinical and clinical activities and business operations, in addition to possibly requiring substantial expenditures of resources to remedy. If such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our development programs. For example, the loss of clinical trial data from completed or ongoing clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach was to result in a loss of or damage to our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability and the further development of our product candidates could be delayed.

Our ability to use net operating loss carryforwards to reduce future tax payments may be limited or restricted.

We have generated significant net operating loss carryforwards (NOLs) as a result of our incurrence of losses and our conduct of research activities since inception. We generally are able to carry NOLs forward to reduce our tax liability in future years. However, our ability to utilize the NOLs is subject to the rules of Sections 382 and 383 of the Code. Those sections generally restrict the use of NOLs after an "ownership change." An ownership change occurs if, among other things, the stockholders (or specified groups of stockholders) who own or have owned, directly or indirectly, 5% or more of a corporation's common stock or are otherwise treated as 5% stockholders under Section 382 of the Code and the United States Treasury Department regulations promulgated thereunder increase their aggregate percentage ownership of that corporation's stock by more than 50 percentage points over the lowest percentage of the stock owned by these stockholders over the applicable testing period. In the event of an ownership change, Section 382 imposes an annual limitation on the amount of taxable income a corporation may offset with NOL carry forwards and Section 383 imposes an annual limitation on the amount of tax a corporation may offset with carry forwards. Any unused annual limitation may be carried over to later years until the applicable expiration date for the respective NOL carry forwards.

We have completed several financings since our inception which we believe have resulted in “ownership changes” within the meaning of Section 382. We may also experience ownership changes in the future as a result of additional financings and subsequent shifts in our stock ownership. As a result, our NOLs may be subject to limitations and we may be required to pay taxes earlier and in larger amounts than would be the case if our NOLs were freely usable.

Table of Contents

Risks Related to Ownership of our Common Stock

The trading price of the shares of our common stock has been highly volatile, and purchasers of our common stock could incur substantial losses.

Our stock began trading on NYSE MKT on May 17, 2013 and then on the Nasdaq Capital Market on August 29, 2014. Between May 17, 2013 and December 31, 2018, our common stock has traded between $1.45 and $114.00. Our stock price could be subject to wide fluctuations in response to a variety of factors, which include:

- whether our clinical trials can be conducted within the timeframe that we expect and whether such trials will yield positive results;
- whether our collaborations with Intrexon can be advanced with positive results within the timeframe and budget that we expect;
- changes in laws or regulations applicable to our products or product candidates, including but not limited to clinical trial requirements for approvals;
- unanticipated serious safety concerns related to the use of our product candidates;
- a decision to initiate a clinical trial, not to initiate a clinical trial or to terminate an existing clinical trial;
- our ability to increase our manufacturing capacity and reduce our manufacturing costs through the improvement of our manufacturing process, our ability to validate any such improvements with the relevant regulatory agencies and our ability to accomplish the foregoing on a timely basis;
- adverse regulatory decisions;
- the introduction of new products or technologies offered by us or our competitors;
- negative public opinion or perception of cell and gene therapies;
- the inability to effectively manage our growth;
- actual or anticipated variations in quarterly operating results;
- the failure to meet or exceed the estimates and projections of the investment community;
- the perception of the biopharmaceutical industry by the public, legislatures, regulators and the investment community;
- the overall performance of the U.S. equity capital markets and general political and economic conditions;
- announcements of significant acquisitions, strategic partnerships, joint ventures, collaborations or capital commitments by us or our competitors;
- disputes or other developments relating to proprietary rights, including patents, litigation matters and our ability to obtain patent protection for our technologies;
- additions or departures of key personnel;

- the trading volume of our common stock; and

- other events or factors, many of which are beyond our control.

In addition, the stock market in general, and the stock of biotechnology companies in particular, have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of these companies. Broad market and industry factors may negatively affect the market price of our common stock, regardless of our actual operating performance.

Table of Contents

Randal J. Kirk and certain of his affiliates (including Intrexon) own a substantial percentage of our common stock and will be able to exert significant influence over matters subject to stockholder approval.

As of March 20, 2019, Randal J. Kirk and certain of his affiliates (including Intrexon, our collaboration partner on our gene therapy programs) beneficially owned approximately 1.7 million shares, or approximately 17%, of our common stock, excluding common stock underlying the Notes and Private Placement Warrants issued in connection with the 2016 Private Placement, the Series A Preferred Stock, the March 2017 Warrants and the December 2017 Common Warrants. If Randal J. Kirk and certain affiliates exercised the convertible securities or warrants acquired in the September 2016 Private Placement, the Series A Preferred Stock Offering and the December 2017 Offering, they would receive, in the aggregate, (i) approximately 450,000 shares of our common stock pursuant to exercise of the Private Placement Warrants, (ii) approximately 400,000 shares of common stock underlying $6,762,500 outstanding principal amount of Notes, (iii) approximately 38,000 shares of common stock underlying accrued interest on the Notes, (iv) approximately 277,000 shares of common stock upon conversion of the Series A Preferred Stock (v) approximately 260,000 shares of common stock pursuant to the exercise of the March 2017 Warrants and (vi) approximately 545,000 shares of common stock pursuant to the exercise of the December 2017 Common Warrants, resulting in the beneficial ownership of approximately 31% of our common stock.

Mr. Kirk and his affiliates may have interests that conflict with our other stockholders and, if acting together, have the ability to significantly influence the outcome of matters submitted to our stockholders for approval, including the election and removal of directors and any merger, consolidation or sale of all or substantially all of our assets. This concentration of ownership could delay or prevent any acquisition of our company on terms that other stockholders may desire.

Additionally, two of our directors, Julian Kirk (who is the son of Randal J. Kirk) and Marcus Smith, are employees of Third Security, LLC, which is an affiliate of Randal J. Kirk.

Our operating results may fluctuate significantly in the future, which may cause our results to fall below the expectations of securities analysts, stockholders and investors.

Our operating results may fluctuate significantly in the future as a result of a variety of factors, many of which are outside of our control. These factors include, but are not limited to:

- the timing, implementation and cost of our research, pre-clinical studies and clinical trials;
- expenses in connection with our exclusive channel collaboration agreements with Intrexon;
- the timely and successful implementation of improved manufacturing processes;
- our ability to attract and retain personnel with the necessary strategic, technical and creative skills required for effective operations;
- the amount and timing of expenditures by practitioners and their patients;
- introduction of new technologies;
- product liability litigation, class action and derivative action litigation, or other litigation;
- the amount and timing of capital expenditures and other costs relating to the expansion of our operations;
- the state of the debt and/or equity capital markets at the time of any proposed offering we choose to initiate;

- our ability to successfully integrate new acquisitions into our operations;

- government regulation and legal developments regarding our product candidates in the United States and in the foreign countries in which we may operate in the future; and

- general economic conditions.

Table of Contents

As a strategic response to changes in the competitive environment, we may from time to time make pricing, service, technology or marketing decisions or business or technology acquisitions that could have a material adverse effect on our operating results. Due to any of these factors, our operating results may fall below the expectations of securities analysts, stockholders and investors in any future period, which may cause our stock price to decline.

Future sales of our common stock may depress our stock price.

The market price of our common stock could decline as a result of sales of substantial amounts of our common stock in the public market, or as a result of the perception that these sales could occur, which could occur if we issue a large number of shares of common stock (or securities convertible into our common stock) in connection with a future financing, as our common stock is trading at low levels. These factors could make it more difficult for us to raise funds through future offerings of common stock or other equity securities. In addition to our common stock outstanding, as of December 31, 2018, we had warrants and stock options outstanding that were exercisable for a total of 7,679,678 shares of our common stock.

Holders of our outstanding preferred shares have dividend, liquidation and other rights that are senior to the rights of the holders of our common shares.

Upon our liquidation, dissolution or winding up, the holders of the Series A Preferred Stock are entitled to receive out of our assets, whether capital or surplus, an amount equal to such holder’s then stated value for each share of Series A Preferred Stock before any distribution to the holders of the common stock, any class or series of preferred stock and all other common stock equivalents other than those securities which are explicitly senior or pari passu to the Series A Preferred Stock in redemption, distribution of assets upon a liquidation or dividends. If there are insufficient assets to pay in full such amounts, then the available assets will be ratably distributed to the holders of the Series A Preferred Stock in accordance with the respective amounts that would be payable on such shares if all amounts payable thereon were paid in full. This will reduce the remaining amount of our assets, if any, available to distribute to holders of our common stock.

We have not declared any dividends on our common stock to date, and we have no intention of declaring dividends in the foreseeable future.

The decision to pay cash dividends on our common stock rests with our Board and will depend on our earnings, unencumbered cash, capital requirements and financial condition. We do not anticipate declaring any dividends in the foreseeable future, as we intend to use any excess cash to fund our operations. In addition, the Notes and Series A Preferred Stock each restrict our ability to pay cash dividends on our equity securities. Investors in our common stock should not expect to receive dividend income on their investment, and investors will be dependent on the appreciation of our common stock to earn a return on their investment.

Provisions in our charter documents could prevent or delay stockholders’ attempts to replace or remove current members of our Board.

Our charter documents provide for staggered terms for the members of our Board. Our Board is divided into three staggered classes, and each director serves a term of three years. At stockholders’ meetings, only those directors comprising one of the three classes will have completed their term and be subject to re-election or replacement.

In addition, our Board is authorized to issue “blank check” preferred stock, with designations, rights and preferences as they may determine. Accordingly, our Board has in the past and may in the future, without stockholder approval, issue shares of preferred stock with dividend, liquidation, conversion, voting or other rights that could adversely affect the voting power or other rights of the holders of our common stock. This type of preferred stock could also be issued to

discourage, delay or prevent a change in our control.

The use of a staggered Board and the ability to issue “blank check” preferred stock are traditional anti-takeover measures. These provisions in our charter documents make it difficult for a majority stockholder to gain control of the Board and of our company. These provisions may be beneficial to our management and our Board in a hostile tender offer and may have an adverse impact on stockholders who may want to participate in such a tender offer, or who may want to replace some or all of the members of our Board.

Table of Contents

Provisions in our bylaws provide for indemnification of officers and directors, which could require us to direct funds away from our business and the development of our product candidates.

Our bylaws provide for the indemnification of our officers and directors. We have in the past and may in the future be required to advance costs incurred by an officer or director and to pay judgments, fines and expenses incurred by an officer or director, including reasonable attorneys' fees, as a result of actions or proceedings in which our officers and directors are involved by reason of being or having been an officer or director of our company. Funds paid in satisfaction of judgments, fines and expenses may be funds we need for the operation of our business and the development of our product candidates, thereby affecting our ability to attain profitability.

An active market for our common stock may not be sustained.

In the past, we have had a limited, volatile and sporadic public trading market for our common stock. Although our common stock is listed on the Nasdaq Capital Market, an active trading market for our common stock may not be sustained, especially given the large percentage of our common stock held by our affiliates. If an active market for our common stock is not sustained, it may be difficult for our stockholders to sell shares without depressing the market price for our common stock.

If securities or industry analysts do not publish research or publish inaccurate or unfavorable research about our business, our stock price and trading volume could decline.

The trading market for our common stock depends in part on the research and reports that securities or industry analysts publish about us or our business. We do not have any control over these analysts. If one or more of the analysts who cover us downgrade our stock or publish inaccurate or unfavorable research about our business, our stock price would likely decline. If one or more of these analysts cease coverage of our company or fail to publish reports on us regularly, demand for our stock could decrease, which might cause our stock price and trading volume to decline.

Item 1B. Unresolved Staff Comments

Not applicable.

Item 2. Properties

Our corporate office and manufacturing facility are located at 405 Eagleview Boulevard, Exton, Pennsylvania. This location consists of approximately 17,500 square feet of manufacturing and laboratory space and 69,000 square feet of office space, which we lease pursuant to a lease agreement that expires on March 31, 2023. We believe this facility is suitable for our current needs.

Item 3. Legal Proceedings

We are not a party to any pending legal proceedings.

Item 4. Mine Safety Disclosure

Not applicable.

Table of Contents

Part II

Item 5. Market for Registrant's Common Equity, Related Stockholder Matters and Issuer Purchases of Equity Securities

Market Information

Our common stock is traded on the Nasdaq Capital Market under the symbol "FCSC."

Holders of Record

As of March 20, 2019, there were 9,758,332 shares of our common stock outstanding. There were approximately 33 holders of record at March 20, 2019. Because many of our shares are held by brokers and other institutions on behalf of stockholders, we are unable to estimate the total number of stockholders represented by these record holders.

Securities Authorized for Issuance under Equity Compensation Plans

Information regarding securities authorized for issuance under equity compensation plans is incorporated by reference into the information in Part III, Item 12 of this Form 10-K.

Recent Sales of Unregistered Securities

On December 7, 2018, we entered into a securities purchase agreement (the December 2018 Purchase Agreement) with two accredited investors, EBRP and EBMRF (December 2018 Private Placement Purchasers), pursuant to which we issued and sold to the December 2018 Private Placement Purchasers an aggregate of 443,350 shares of our common stock at a per share price of $2.03, which represented the consolidated closing bid price of our common stock, as reported by the Nasdaq Capital Market, on the business day immediately preceding the execution of the December 2018 Purchase Agreement. We refer to the sale of these shares of common stock as the December 2018 Private Placement. We received approximately $900,000 in gross proceeds from the December 2018 Private Placement. The offer, sale, and issuance of the shares were made in a private placement transaction exempt from registration pursuant to Rule 506 of Regulation D and Section 4(a)(2) of the Securities Act of 1933, as amended. The shares of our common stock sold to the December 2018 Private Placement Purchasers were subsequently registered for resale on a registration statement on Form S-3 (file number 333-229307), which was declared effective by the Securities and Exchange Commission on February 12, 2019.

Purchases of Equity Securities by the Issuer and Affiliated Purchasers

We did not repurchase any of our equity securities during the year ended December 31, 2018.

Item 6. Selected Financial Data

We are a smaller reporting company as defined by Rule 12b-2 of the Securities Exchange Act of 1934 and are not required to provide the information under this item.

Table of Contents

Item 7. Management's Discussion and Analysis of Financial Condition and Results of Operations

The following discussion and analysis of our financial condition and results of operations should be read in conjunction with our audited consolidated financial statements and the related notes included in Part IV of this Form 10-K.

Overview

We are an autologous cell and gene therapy company focused on translating personalized biologics into medical breakthroughs for diseases affecting the skin and connective tissue. Our distinctive approach to personalized biologics is based on our proprietary autologous fibroblast technology. Fibroblasts are the most common cell in skin and connective tissue and are responsible for synthesizing extracellular matrix proteins, including collagen and other growth factors, that provide structure and support. Because fibroblasts naturally reside in the localized environment of the skin and connective tissue, they represent an ideal delivery vehicle for proteins targeted to these areas. We target the underlying cause of disease by using fibroblast cells from a patient's skin and genetically modifying them to create localized therapies that are compatible with the unique biology of the patient (i.e., which are autologous).

We are focused on discovering and developing localized therapies for diseases affecting the skin and connective tissue, where there are high unmet needs, to improve the lives of patients and their families. In that regard, we commit significant resources to our research and development programs. Currently, all of our research and development operations and focus are on gaining regulatory approvals to commercialize our product candidates in the United States; however, we may seek to expand into international markets in the future.

Development Programs

Our current pipeline consists of the following product candidates, which we are developing in collaboration with Intrexon:

FCX-007 is our clinical-stage, gene therapy product candidate for the treatment of RDEB, a congenital and progressive orphan skin disease caused by the deficiency of COL7. FCX-007 is a genetically-modified autologous fibroblast that encodes the gene for COL7 for localized treatment of RDEB and is being developed in collaboration with Intrexon. By genetically modifying autologous fibroblasts ex vivo to produce COL7, culturing them and then treating wounds locally via injection, FCX-007 offers the potential to address the underlying cause of the disease by providing high levels of COL7 directly to the affected areas, thereby avoiding systemic treatment.

FCX-007 is currently in a Phase 1/2 clinical trial. In January 2018, Fibrocell obtained allowance from the FDA to initiate enrollment of pediatric patients in the Phase 2 portion of its Phase 1/2 clinical trial of FCX-007, based on evidence of safety and potential benefit of FCX-007 in adult patients dosed in the Phase 1 portion of the clinical trial. In May 2018, we reported on interim adult data and provided a Phase 1 trial update which included presenting at the 7th International Investigative Dermatology meeting on May 19, 2018.

We completed the targeted enrollment of six patients ages seven and older in the Phase 2 portion of the Phase 1/2 clinical trial for FCX-007, and have over-enrolled by one patient for a total of seven patients. The Phase 2 population consists of one adult and six pediatric patients. In March 2019, we reported additional positive safety and wound healing data for our ongoing Phase 1/2 trial.

In October 2018, we completed a Type C meeting with the FDA to discuss the design of a Phase 3 clinical trial protocol for FCX-007. The FDA provided guidance on various clinical trial design aspects and CMC requirements of the proposed Phase 3 clinical trial. In November 2018, we received the official minutes from the FDA for the Type C meeting. Based on FDA's feedback, we prepared a Phase 3 clinical trial protocol for FCX-007 and filed it as part of the briefing package for the Type B meeting in March 2019. We completed a Type B end-of-Phase 2 face-to-face meeting with the FDA in March 2019 to discuss the design of a Phase 3 clinical trial for FCX-007 to support a BLA filing. In

the Type B meeting, the FDA provided guidance on various design aspects of our proposed Phase 3 clinical trial, named DEFI-RDEB. We plan to submit a revised clinical trial protocol and statistical analysis plan based upon the FDA's feedback and requested CMC information to the IND application. We plan to continue the remaining follow-up visits with all Phase 1/2 patients, but do not intend to dose additional patients as part of the trial. Furthermore, we plan to initiate a Phase 3 clinical trial for FCX-007 in the second quarter of 2019.

We have designated our existing, cGMP cell therapy manufacturing facility in Exton, PA as the production site for FCX-007 after incorporation into our IND application. FCX-007 drug product dosed in the fourth quarter of 2017 was produced and distributed from our Exton, PA facility. This multi-product, gene therapy manufacturing facility will be used for the

Table of Contents

remaining clinical and, if approved, future commercial manufacture of FCX-007, as we have sufficient cGMP vector supply to complete our clinical trials and existing manufacturing capacity to serve the U.S. market for RDEB.

FCX-007 has received Orphan Drug Designation for the treatment of DEB, including RDEB, Rare Pediatric Disease Designation for the treatment of RDEB and Fast Track Designation for the treatment of RDEB from the FDA.

In addition, our second clinical stage gene therapy candidate, FCX-013 is in development for the treatment of moderate to severe localized scleroderma, which manifests as excess production of extracellular matrix, specifically collagen, resulting in thickening of the skin and connective tissue. FCX-013 is designed to be injected under the skin at the location of the fibrotic lesions where the genetically-modified fibroblast cells will produce matrix metalloproteinase 1 (MMP-1) to break down excess collagen accumulation. We previously completed a proof-of-concept study and pre-clinical dose-ranging study for FCX-013. In December 2017, we completed a GLP toxicology/biodistribution study. We submitted an IND application for FCX-013 to the FDA in January 2018, and in March 2018, the FDA allowed the IND to progress to clinical trials. We initiated the first investigator site for clinical enrollment for an open label, single arm Phase 1/2 clinical trial. We are currently enrolling the Phase 1 portion of the Phase 1/2 clinical trial for FCX-013, and expect to complete enrollment of Phase 1 adult patients in the third quarter of 2019. We plan to manufacture FCX-013 at our Exton, PA cGMP manufacturing facility.

FCX-013 has received Orphan Drug Designation from the FDA for the treatment of localized scleroderma and Rare Pediatric Disease Designation and in September 2018, Fast Track Designation for moderate to severe localized scleroderma.

Gene Therapy Research Program for Arthritis and Related Conditions

We expanded our collaboration with Intrexon to pursue the research, development and commercialization of products for the treatment of chronic inflammation and degenerative diseases of human joints through intra-articular or other local administration of genetically modified fibroblasts. We are currently in the research phase for a gene therapy to treat arthritis and related conditions under this collaboration. Our goal is to deliver a protein therapy locally to the joint to provide sustained efficacy while avoiding key side effects typically associated with systemic therapy.

See “Item 1—Business” within Part I of this Form 10-K for additional details regarding our development programs, research programs, and collaboration agreements.

Table of Contents

Critical Accounting Policies

The following discussion and analysis of financial condition and results of operations are based upon our Consolidated Financial Statements, which have been prepared in conformity with U.S. generally accepted accounting principles (GAAP). Preparing financial statements requires us to make estimates and assumptions that affect the reported amounts of assets, liabilities, revenues and expenses. Estimates are based on our historical operations, our future business plans and projected financial results, the terms of existing contracts, our observance of trends in the industry, information provided by our customers and information available from other outside sources, as appropriate. These estimates and assumptions are affected by the application of our accounting policies. Critical accounting policies and practices are both important to the portrayal of a company's financial condition and results of operations, and require management's most difficult, subjective or complex judgments, often as a result of the need to make estimates about the effects of matters that are inherently uncertain. Actual results could differ from such estimates due to changes in economic factors or other conditions that are outside the control of management. A summary of our significant accounting policies is more fully described in Note 3 of the Consolidated Financial Statements contained in this Form 10-K.

Warrant Liability

The Company accounts for stock warrants as either equity instruments, derivative liabilities, or liabilities in accordance with ASC 480, Distinguishing Liabilities from Equity (ASC 480), depending on the specific terms of the warrant agreement. Stock warrants are accounted for as a derivative in accordance with ASC 815, Derivatives and Hedging (ASC 815) if the stock warrants contain terms that could potentially require "net cash settlement" and therefore, do not meet the scope exception for treatment as a derivative. Warrant instruments that could potentially require "net cash settlement" in the absence of express language precluding such settlement are initially classified as derivative liabilities at their estimated fair values, regardless of the likelihood that such instruments will ever be settled in cash. The Company will continue to classify the fair value of the warrants that contain "net cash settlement" as a liability until the warrants are exercised, expire or are amended in a way that would no longer require these warrants to be classified as a liability. Warrants that the Company may be required to redeem through payment of cash or other assets outside its control are classified as liabilities pursuant to ASC 480 and are initially and subsequently measured at their estimated fair values. For additional discussion on warrants, see Note 7.

Debt Issued With Warrants

The Company considers guidance within ASC 470-20, Debt (ASC 470), ASC 480, and ASC 815 when accounting for the issuance of convertible debt with detachable warrants. As described above under the caption "Warrant Liability", the Company classifies stock warrants as either equity instruments, derivative liabilities, or liabilities depending on the specific terms of the warrant agreement. In circumstances in which debt is issued with liability-classified warrants, the proceeds from the issuance of convertible debt are first allocated to the warrants at their full estimated fair value and established as both a liability and a debt discount. The remaining proceeds, as further reduced by discounts created by the bifurcation of embedded derivatives and beneficial conversion features, are allocated to the debt. The Company accounts for debt as liabilities measured at amortized cost and amortizes the resulting debt discount from the allocation of proceeds, to interest expense using the effective interest method over the expected term of the debt instrument pursuant to ASC 835, Interest (ASC 835).

Embedded Derivatives. The Company considers whether there are any embedded features in debt instruments that require bifurcation and separate accounting as derivative financial instruments pursuant to ASC 815. Embedded derivatives are initially and subsequently measured at fair value. See Note 6 for additional discussion on the embedded derivatives associated with the Company's convertible notes.

Beneficial Conversion Feature. If the amount allocated to the convertible debt results in an effective per share conversion price less than the fair value of the Company's common stock on the commitment date, the intrinsic value of this beneficial conversion feature is recorded as a discount to the convertible debt with a corresponding increase to additional paid in capital. The beneficial conversion feature discount is equal to the difference between the effective conversion price and the fair value of the Company's common stock at the commitment date, unless limited by the remaining proceeds allocated to the debt. See Note 6 for additional discussion on the beneficial conversion feature associated with the Company's convertible notes.

Debt Issuance Costs. The Company follows the guidance under Accounting Standards Update (ASU) 2015-03, Interest - Imputation of Interest (Subtopic 835-30): Simplifying the Presentation of Debt Issuance Costs (ASU 2015-03) for accounting for debt issuance costs. The Company allocates debt issuance costs between the debt and the warrants on the same basis as proceeds were allocated. The Company expenses issuance costs allocated to the warrants and presents the issuance costs allocated to the debt as a direct reduction from the carrying amount of the debt liability in the balance sheet. However, if

Table of Contents

debt issuance costs exceed the carrying amount of the debt, issuance costs are recorded to additional paid-in capital as a reduction of the beneficial conversion feature. As of December 31, 2018, the Company's debt issuance costs are presented in additional paid-in capital as a reduction of the beneficial conversion feature and are being amortized to interest expense (despite their classification in additional paid-in capital) using the effective interest rate method over the expected term of the debt pursuant to ASC 835.

Results of Operations
Comparison of Years Ending December 31, 2018 and 2017

Research and Development Expenses

For each of our research and development programs, we incur both direct and indirect expenses. We track direct research and development expenses by program, which include third party costs such as contract research, consulting and pre-clinical development costs and clinical trial and manufacturing costs. We do not allocate indirect research and development expenses, which may include regulatory, laboratory (equipment and supplies), personnel, facility, process development and other overhead costs (including depreciation and amortization), to specific programs, as these expenses are to be deployed across all of our product candidates. We expect research and development costs to continue to be significant for the foreseeable future as a result of our pre-clinical studies and clinical trials, as well as our ongoing collaborations with Intrexon.

Direct research and development costs, by major program, and indirect research and development costs, by major component, were as follows:

	Year Ended December 31,		2018 vs 2017 Change		
($ in thousands)	2018	2017	$	%	
Direct costs:					
FCX-007	870	4,350	(3,480)	(80.0)%	(1)
FCX-013	471	3,117	(2,646)	(84.9)%	(2)
Other	(32)	72	(104)	(144.4)%	(3)
Total direct costs	1,309	7,539	(6,230)	(82.6)%	
Indirect costs:					
Regulatory costs	63	91	(28)	(30.8)%	(4)
Compensation and related expenses	2,003	2,031	(28)	(1.4)%	(5)
Process development	—	7	(7)	(100.0)%	(6)
Other indirect R&D costs	2,609	2,564	45	1.8 %	(7)
Total indirect costs	4,675	4,693	(18)	(0.4)%	
Total research and development expenses	$5,984	$12,232	$(6,248)	(51.1)%	

(1) Costs for our FCX-007 program decreased approximately $3.5 million, or 80.0%, for the year ended December 31, 2018 compared to 2017 due primarily to decreased costs from (1) our clinical partner Intrexon, as the Phase 1 portion of the FCX-007 clinical trial was substantially completed at the end of 2017; (2) movement in-house of the manufacturing of the drug product used in the Phase 1/2 clinical trial of FCX-007 previously contracted to a third party manufacturer and (3) a decrease of approximately $0.5 million in an estimate of costs to settle a dispute with one of Intrexon's vendors, for which a settlement was agreed to and was paid by us in August 2018.

Through December 31, 2018, we have incurred approximately $25.6 million in direct research and development costs related to this program, life-to-date, which include non-cash expenses of $6.9 million in stock issuance costs associated with the 2012 ECC with Intrexon. Other costs include product and assay development, key opinion leader development, pre-clinical studies and manufacturing, the design of the Phase 1/2 clinical trial protocol and recruiting patients, clinical product manufacturing, statistical analyses, report generation and future clinical trial costs.

(2) Costs for our FCX-013 program for the year ended December 31, 2018 decreased approximately $2.6 million, or 84.9% compared to 2017 due primarily to decreased costs from our clinical partner Intrexon of approximately $2.3 million, as substantially all of the costs of the pre-clinical phase of this program were incurred at the end of 2017.

Table of Contents

Through December 31, 2018, we have incurred approximately $14.3 million in direct research and development costs related to this program, life-to-date, which include non-cash expenses of $6.4 million in stock issuance costs with the 2012 ECC with Intrexon. Other costs include product and assay development, key opinion leader development, National Institutes of Health Recombinant DNA Advisory Committee (NIH RAC) meeting preparation expenses, and the design and execution of clinical trials.

(3) Costs for our other programs decreased approximately $0.1 million or 144.4%, for the year ended December 31, 2018 compared to 2017. The azficel-T for chronic dysphonia program was discontinued at June 30, 2016 and the costs recorded since then relate to specific close out activities of the program.

(4) Regulatory costs were not significant for both years ended December 31, 2018 and 2017.

(5) Compensation and related expenses were approximately $2.0 million for the years ended December 31, 2018 and 2017.

(6) Process development costs were zero in the year ended December 31, 2018 and were not significant in the year ended December 31, 2017.

(7) Other indirect R&D costs were approximately $2.6 million for the years ended December 31, 2018 and 2017.

Selling, General and Administrative Expenses

Selling, general and administrative expenses were comprised of the following:

	Year Ended December 31,		2018 vs 2017 Change		
($ in thousands)	2018	2017	$	%	
Compensation and related expenses	$1,717	$1,764	(47)	(2.7)%	(1)
Professional fees	1,574	2,103	(529)	(25.2)%	(2)
Facilities and related expenses and other	3,114	2,882	232	8.0 %	(3)
Total selling, general and administrative expenses	$6,405	$6,749	$(344)	(5.1)%	

(1) Compensation and related expenses were approximately $1.7 million for the years ending December 31, 2018 and 2017.

(2) Professional fees decreased approximately $0.5 million, or 25.2%, for the year ended December 31, 2018 compared to 2017. This decrease is attributable primarily to lower levels of legal, accounting and consulting fees.

(3) Facilities and related expenses increased approximately $0.2 million for the year ended December 31, 2018 versus the year ended December 31, 2017. This increase was due primarily to approximately $0.2 million of income recognized in the 2017 period, as a result of the release of certain reserves included in accrued expenses as of December 31, 2016.

Warrant Revaluation Income

During the years ended December 31, 2018 and 2017, we recorded non-cash income of approximately $0.9 million and $4.9 million, respectively, for warrant revaluation income in our Consolidated Statements of Operations. Due to the nature and inputs of the model used to assess the fair value of our outstanding warrants, it is not abnormal to experience significant fluctuations from year to year. These fluctuations were due to a variety of factors including changes in our stock price, changes in the remaining contractual life of the warrants, and changes in management's estimated probability of certain events occurring that would impact the warrants. Warrant revaluation income for 2018 and 2017 was driven primarily by decreases in both our stock price and the remaining contractual term of the warrants.

Derivative Revaluation Expense

During the years ended December 31, 2018 and 2017, we recorded non-cash derivative revaluation income (expense) of approximately $1.7 million and ($1.4 million), respectively, for derivative liability revaluation charges in our

Consolidated Statement of Operations related to a compound bifurcated derivative initially recorded in September 2016 in connection with the 2016 Private Placement. Derivative valuation income for the year ended December 31, 2018, was primarily the result of an estimate change in the timing and probability of a change of control occurring, that would affect the convertible notes issued in

Table of Contents

September 2016. See Note 6 in the accompanying Notes to the Consolidated Financial Statements contained in Part IV of this Form 10-K for further details.

Interest Expense

During the years ended December 31, 2018 and 2017, we recorded interest expense of approximately $0.8 million, in each of the year end periods, in our Consolidated Statement of Operations related to the Notes that we issued in the 2016 Private Placement which bear interest at 4% per annum. See Note 6 in the accompanying Notes to the Consolidated Financial Statements contained in Part IV of this Form 10-K for further details.

Net Loss

Net loss decreased approximately $5.9 million to $10.3 million for the year ended December 31, 2018, as compared to $16.2 million for the year ended December 31, 2017. The decrease was due primarily to an overall net decrease in operating expenses of approximately $6.6 million, as more fully described at the component level above, increased derivative revaluation income of $3.1 million, all partially offset by a decrease in warrant revaluation income of approximately $4.0 million.

Table of Contents

Financial Condition, Liquidity and Capital Resources

Financial Condition

We have experienced losses since our inception. As of December 31, 2018, we had an accumulated deficit of approximately $189.1 million. The process of developing and commercializing our product candidates requires significant research and development efforts and clinical trial work, as well as significant manufacturing and process development. These activities, together with our selling, general and administrative expenses, are expected to continue to result in significant operating losses for the foreseeable future. Additionally, to fund our operations, we issued convertible promissory notes in an aggregate amount of approximately $18.1 million, which bear interest at 4% per annum, in connection with the 2016 Private Placement as more fully described under the heading “Contractual Obligations” below and in Note 6 in the accompanying Notes to the Consolidated Financial Statements contained in Part IV of this Form 10-K.

Our financial condition is summarized below as of the following dates:

	As of December 31,		Change	
($ in thousands)	2018	2017	$	%
Cash and cash equivalents	$14,430	$17,417	$(2,987)	(17.1)%
Working capital:				
Total current assets	$14,535	$17,902	$(3,367)	(18.8)%
Less: Total current liabilities	2,172	4,425	(2,253)	(50.9)%
Net working capital	$12,363	$13,477	$(1,114)	(8.3)%
Convertible notes payable (gross principal)	$18,003	$18,003	$—	— %

Liquidity and Capital Resources

Our principal sources of liquidity are cash and cash equivalents of approximately $14.4 million as of December 31, 2018. As of December 31, 2018, we had net working capital of approximately $12.4 million which decreased approximately $1.1 million, or 8.3%, from December 31, 2017. We believe that our existing cash and cash equivalents, will be sufficient to fund our operations into the fourth quarter of 2019; however, changing circumstances may cause us to consume capital faster than we currently anticipate, and we may need to spend more money than currently expected because of such circumstances. We will require additional capital to fund operations beyond that point and prior to our business achieving significant net cash from operations. Our future capital requirements may be substantial, and will depend on many factors, including, but not limited to:

- the cost of clinical activities and outcomes related to the clinical trials for FCX-007 and FCX-013;
- the cost of additional pre-clinical studies and clinical trials in order to obtain regulatory approvals for our product candidates;
- the cost of regulatory submissions, as well as the preparation, initiation and execution of clinical trials in potential new clinical indications; and
- the cost of filing, surveillance around, prosecuting, defending and enforcing patent claims.

To meet our capital needs, we consider multiple alternatives, including but not limited to equity financings, debt financings, corporate collaborations, partnerships and other strategic transactions and funding opportunities. However, there is no assurance that we will be able to complete any such transaction or obtain the additional required capital on acceptable terms or otherwise. Furthermore, the covenants under our convertible notes limit our ability to obtain additional debt financing. If we raise additional funds by issuing equity securities, our stockholders will experience

dilution. Debt financing, if available, will result in increased fixed payment obligations and may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends. Any debt or equity financing that we complete may contain terms, such as liquidation and other preferences, which are not favorable to us or our stockholders. If we raise additional funds through collaboration or partnership arrangements with third parties, it may be necessary to relinquish valuable rights to our technologies, future revenue streams or product candidates or to grant licenses on terms that may not be favorable to us.

Table of Contents

If we are unable to raise additional capital in sufficient amounts or on terms acceptable to us, we will need to curtail and reduce our operations and costs and modify our business strategy which may require us to, among other things:

- significantly delay, scale back or discontinue the development or commercialization of one or more of our product candidates or one or more of our other research and development initiatives;
- seek collaborators for one or more of our current or future product candidates at an earlier stage than otherwise would be desirable or on terms that are less favorable than might otherwise be available; or
- sell or license on unfavorable terms our rights to technologies or product candidates that we otherwise would seek to develop or commercialize ourselves.

Additionally, failure to obtain the necessary capital in a timely manner could require us to seek bankruptcy protection or result in our breach or default under agreements on which our business relies or pursuant to which we obtain valuable rights which could result in, among other things, the potential acceleration of payments thereunder or the termination of such agreements.

These factors raise substantial doubt about our ability to continue as a going concern. Consequently, the audit report prepared by our independent registered public accounting firm relating to our Consolidated Financial Statements for the year ended December 31, 2018 includes a paragraph related to the substantial doubt about our ability to continue as a going concern.

2017 Series A Preferred Stock Offering

On March 8, 2017, we completed the 2017 Series A Preferred Stock Offering pursuant to which we sold the Units for a purchase price of $1,000 per Unit, with each Unit consisting of (i) one share of our Series A Preferred Stock, with an initial stated value of $1,000 that is convertible into shares of our common stock with a conversion price of $11.6355 and (ii) a warrant to purchase up to a number of shares of common stock equal to 100% of the conversion shares issuable on March 7, 2017 pursuant to the shares of Series A Preferred Stock purchased by each investor for the sale of $8.0 million to certain of our existing investors, including certain related parties such as Intrexon. After deducting offering expenses, net proceeds from the Series A Preferred Stock Offering, excluding the proceeds, if any, from the exercise of the warrants, was approximately $7.6 million.

2017 Common Stock and Warrant Offering

On December 11, 2017, we completed the December 2017 Offering pursuant to which we sold 1,542,832 shares of our common stock, pre-funded warrants to purchase an aggregate of 1,184,442 shares of our common stock and common warrants to purchase up to an aggregate of 2,809,404 shares of our common stock for $10.5 million. After deducting offering expenses, net proceeds from the December 2017 Offering excluding the proceeds, if any, from the exercise of the warrants, was approximately $9.3 million.

May 2018 Registered Direct Offering and Private Placement

On May 31, 2018, we completed the May 2018 Registered Direct Offering and the May 2018 Private Placement pursuant to which we sold 2,038,224 shares of our common stock and common warrants to purchase up to an aggregate of 1,528,668 shares of our common stock for approximately $6.0 million. After deducting offering expenses, net proceeds from the May 2018 Registered Direct Offering and the May 2018 Private Placement was approximately $5.3 million.

July 2018 Registered Direct Offering and Private Placement

On July 5, 2018, we completed the July 2018 Registered Direct Offering and the July 2018 Private Placement pursuant to which we sold 1,474,080 shares of our common stock and common warrants to purchase up to an aggregate of 958,152 shares of our common stock for approximately $4.0 million. After deducting offering expenses, net proceeds from the July 2018 Registered Direct Offering and the July 2018 Private Placement was approximately $3.6 million.

December 2018 Private Placement

On December 11, 2018, we completed the December 2018 Private Placement pursuant to which we sold 443,350 shares of our common stock for approximately $0.9 million. After deducting offering expenses, net proceeds from the December 2018 Private Placement was approximately $0.8 million.

Table of Contents

Also, see Risks Related to Our Financial Position and Need for Additional Capital included within Part I, Item 1A, "Risk Factors" of this Form 10-K.

Cash Flows

The following table summarizes our cash flow activity:

	Year Ended December 31,		2018 vs 2017 Change	
($ in thousands)	2018	2017	$	%
Net cash flows provided by (used in):				
Operating activities	$(12,717)	$(17,037)	$4,320	(25.4)%
Investing activities	$(164)	$(433)	$269	(62.1)%
Financing activities	$9,894	$17,372	$(7,478)	(43.0)%

Operating Activities. Cash used in operating activities during the year ended December 31, 2018 was approximately $12.7 million, a decrease of approximately $4.3 million over the year ended December 31, 2017. This decrease was due primarily to the reduction in net loss for the year ended December 31, 2018 as compared to the year ended December 31, 2017.

Investing Activities. Cash used in investing activities during the year ended December 31, 2018, decreased by approximately $0.3 million over the year ended December 31, 2017. The amount in both periods related primarily to the purchases of equipment and leasehold improvements.

Financing Activities. Cash provided by financing activities during the year ended December 31, 2018 was approximately $9.9 million, a decrease of approximately $7.5 million as compared to the year ended December 31, 2017. The decrease was due primarily to net proceeds from the three offerings in 2018, raising approximately $9.8 million, as compared to the 2017 Series A Preferred Stock Offering and the December 2017 Offering raising approximately $17.4 million. See additional information regarding the offerings in Note 8, Equity.

Off-Balance Sheet Transactions

We do not engage in material off-balance sheet transactions.

Contractual Obligations

The following table summarizes our contractual obligations and commercial commitments as of December 31, 2018 and the effects such obligations are expected to have on our liquidity and cash flows in future periods:

	Payments due by period						
($ in thousands)	Total	2019	2020	2021	2022	2023	2024 and thereafter
Operating lease obligations (1)	$6,197	$1,416	$1,471	$1,471	$1,471	$368	$ —
Debt obligations (2)	21,968	—	—	21,968	—	—	—
Total (3)	$28,165	$1,416	$1,471	$23,439	$1,471	$368	$ —

(1) Operating lease obligations are stated based on the amended lease agreement for our office, warehouse and laboratory facility executed in February 2012.

(2) Obligations under the Notes issued in connection with the 2016 Private Placement which includes principal and accrued interest through September 7, 2021, based on stated fixed rates, as we have elected to accrue interest. The Notes have a maturity date of the earlier of (i) September 7, 2026 and (ii) one-hundred and eighty (180) days after the date on which our product candidate, FCX-007, is approved by the FDA for the treatment of RDEB. However,

(3) each Note holder has the right to require us to repay all or any portion of the unpaid principal and accrued interest from time to time on or after September 7, 2021. See details under the sub-heading “2016 Private Placement” below. This table does not include (a) any milestone payments which may become payable to third parties under license agreements as the timing and likelihood of such payments are not known, (b) any royalty payments to third parties as the amounts of such payments, timing and/or the likelihood of such payments are not known, and (c) contracts that are entered into in the ordinary course of business which are not material in the aggregate in any period presented above.

Table of Contents

2016 Private Placement

In September 2016, we issued an aggregate of approximately $18.1 million in principal of Notes and accompanying Private Placement Warrants to purchase an aggregate of 1,205,840 shares of common stock in a private placement to the Investors, including certain related parties (including Intrexon) which were issued an aggregate of approximately $6.8 million in principal of Notes and accompanying Private Placement Warrants to purchase an aggregate of 450,835 shares of our common stock.

The Notes bear interest at four percent (4%) per annum. Interest is earned daily and compounded quarterly and, at our election at the beginning of each quarter, shall accrue or be paid in cash. If we elect to have interest accrue, such interest will not be added to the principal amount of the Notes but such interest shall be subject to additional interest at the rate of four percent (4%) per annum, compounded quarterly, and shall be due and payable upon the earliest of the conversion of the Notes, exercise of the Put Right, exercise of the Prepayment Right or the Maturity Date (in each case, as defined below). Additionally, if we elect for interest to accrue, then (i) we may elect to repay any such accrued and unpaid interest in cash at any time and from time to time and (ii) each Investor may elect to have us repay any such accrued and unpaid interest by delivering such number of shares of common stock equal to (x) the amount of the accrued and unpaid interest to be repaid, divided by (y) the greater of (i) the last closing bid price of a share of common stock as reported on Nasdaq on the date of such election and (ii) the applicable Conversion Price.

All unpaid principal of each Investor's Note is convertible, at any time and from time to time, at the option of such Investor into shares of common stock at the conversion price, ($17.04375 to $18.39375) and accrued interest is convertible at the greater of (x) the conversion price or (y) the last closing bid price of a share of common stock as reported on the Nasdaq Capital Market at the time of such Investor's execution of the Purchase Agreement, plus $0.12625.

The Notes have a maturity date of the earlier of (i) September 7, 2026 and (ii) one-hundred and eighty (180) days after the date on which our product candidate, FCX-007, is approved by the FDA for the treatment of RDEB (the Maturity Date). Each individual Note holder has the right to require us to repay all or any portion of the unpaid principal and accrued and unpaid interest from time to time on or after September 7, 2021 (the Put Right). Such Put Right must be exercised by such Note holder by delivering written notice to us no later than one-hundred and eighty (180) days prior to such exercise date of such Put Right. In addition, upon consummation of a specified change of control transaction or the occurrence of certain events of default, as defined in the Notes, each Note holder may elect to accelerate the repayment of all unpaid principal and accrued interest under such holder's Note. If an Investor does not elect to have us prepay its Note upon such change of control transaction, then we may prepay the Notes, in an amount equal to one hundred one percent (101%) of the outstanding principal due under the Notes (together with accrued and unpaid interest due thereon) (the Prepayment Right). Additionally, upon the occurrence of certain events of default, as defined in the Notes, each Investor may elect to accelerate the repayment of all unpaid principal and accrued interest under each Note and the Notes provide for automatic redemption upon the occurrence of certain bankruptcy related events of default, as defined in the Notes.

Collaborations with Related Party

We are party to two separate exclusive channel collaboration agreements with Intrexon, a related party, pursuant to which we became Intrexon's exclusive channel collaborator in the research, development and commercialization of certain products as defined in the respective agreements. In connection with these exclusive channel collaboration agreements, we engage Intrexon for support services for the research and development of product candidates covered under the respective agreements and reimburses Intrexon for its cost for time and materials for such services.

For the years ended December 31, 2018 and 2017, we incurred expenses of approximately $0.5 million and $5.2 million, respectively, for goods and services received from Intrexon. These December 31, 2018 and December 31, 2017 amounts do not include approximately $0.5 million in costs we estimated to settle a dispute with one of Intrexon's vendors in 2017, and for which there was a corresponding reduction in 2018. As of December 31, 2018 and 2017, we had outstanding payables with Intrexon of $0.1 million and $2.3 million, respectively.

For additional details, see information within Part I, Item 1—Business, under the heading "Intrexon Collaborations" and Note 12, Related Party Transactions, to the Consolidated Financial Statements, included in Part IV of this Form 10-K.

Table of Contents

Recently Issued Accounting Pronouncements

See Note 3, Summary of Significant Accounting Policies, in the Notes to the Consolidated Financial Statements included in Part IV of this Form 10-K for discussion on recently issued accounting pronouncements.

Item 7A. Quantitative and Qualitative Disclosure about Market Risk

Not applicable.

Item 8. Financial Statements and Supplementary Data

The information required by Item 8 including the financial statements and notes thereto, and report of the independent registered public accounting firm thereon, are included in this Form 10-K as set forth in the “Index to Consolidated Financial Statements” on page F1.

Item 9. Changes in and Disagreements with Accountants on Accounting and Financial Disclosure.

Not applicable.

Item 9A. Controls and Procedures

Evaluation of Disclosure Controls and Procedures

Our management, including our principal executive officer and principal financial officer, has evaluated 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 Securities Exchange Act of 1934, as amended (the Exchange Act)), as of the end of the period covered by this Form 10-K. Based upon that evaluation, our Chief Executive Officer (our principal executive officer and principal financial officer), concluded that, as of December 31, 2018, our disclosure controls and procedures were effective to provide reasonable assurance that (a) the information required to be disclosed by us 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 (b) such information is accumulated and communicated to our management, including our Chief Executive Officer (our principal executive officer and principal financial officer), as appropriate to allow timely decisions regarding required disclosure.

In designing and evaluating our disclosure controls and procedures, our management recognized that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving the desired control objectives, and our management necessarily was required to apply its judgment in evaluating the cost-benefit relationship of possible controls and procedures.

Management’s Report on Internal Control over Financial Reporting

Management is responsible for establishing and maintaining adequate internal control over financial reporting, as such term is defined in Exchange Act Rules 13a-15(f) and 15d-15(f). Under the supervision and with the participation of our management, including our principal executive officer, who also serves as our principal financial officer, we conducted an evaluation of the effectiveness of our internal control over financial reporting, based on the framework in the Internal Control - Integrated Framework (2013) issued by the Committee of Sponsoring Organizations of the Treadway Commission (COSO 2013).

Our 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 in the United States of America. Our internal control over financial reporting includes those policies and procedures that (i) pertain to the maintenance of records that, in reasonable detail,

accurately and fairly reflect the transactions and dispositions of our assets; (ii) provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and our receipts and expenditures are being made only in accordance with authorizations of our management and directors; and (iii) provide

Table of Contents

reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use, or disposition of our assets that could have a material effect on the financial statements.

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.

Based on our evaluation under the framework in COSO 2013, our management concluded that our internal control over financial reporting was effective as of December 31, 2018.

This Form 10-K does not include an attestation report of our independent registered public accounting firm regarding internal control over financial reporting. Based upon the aggregate market value of our common stock held by non-affiliates as of the end of the second quarter of 2018, we are a non-accelerated filer and exempt from this requirement for the 2018 fiscal year. As a result, internal control over financial reporting was not subject to the attestation by our independent registered public accounting firm, given that we are a non-accelerated filer and are exempt from this requirement.

Changes in Internal Control over Financial Reporting

There were no changes in our internal control over financial reporting during the quarter ended December 31, 2018 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

Item 9B. Other Information

None.

Table of Contents

Part III

Item 10. Directors, Executive Officers and Corporate Governance

The information required by this item is incorporated by reference to our Proxy Statement for the 2019 Annual Meeting of Stockholders to be filed with the Securities and Exchange Commission within 120 days of the fiscal year ended December 31, 2018.

Our Board has adopted a written Code of Business Conduct and Ethics applicable to all officers, directors and employees, which is available on our website (www.fibrocell.com) under “Corporate Governance” within the “Investors” section. We intend to satisfy the disclosure requirement under Item 5.05 of Form 8-K regarding any amendment to, or waiver from, a provision of this Code and by posting such information on the website address and location specified above.

Item 11. Executive Compensation

The information required by this item is incorporated by reference to our Proxy Statement for the 2019 Annual Meeting of Stockholders to be filed with the Securities and Exchange Commission within 120 days of the fiscal year ended December 31, 2018.

Item 12. Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters

The information required by this item is incorporated by reference to our Proxy Statement for the 2019 Annual Meeting of Stockholders to be filed with the Securities and Exchange Commission within 120 days of the fiscal year ended December 31, 2018.

Item 13. Certain Relationships and Related Transactions, and Director Independence

The information required by this item is incorporated by reference to our Proxy Statement for the 2019 Annual Meeting of Stockholders to be filed with the Securities and Exchange Commission within 120 days of the fiscal year ended December 31, 2018.

Item 14. Principal Accountant Fees and Services

The information required by this item is incorporated by reference to our Proxy Statement for the 2019 Annual Meeting of Stockholders to be filed with the Securities and Exchange Commission within 120 days of the fiscal year ended December 31, 2018.

Table of Contents

Part IV
Item 15. Exhibits and Financial Statement Schedule

(a) (1) Consolidated Financial Statements.
The Consolidated Financial Statements are filed as part of this report. See the Index to the Consolidated Financial Statements on page F1.
(2) Consolidated Financial Statement Schedule.
Schedules are omitted because they are not applicable, or are not required, or because the information is included in the Consolidated Financial Statements and Notes thereto.

(3) The exhibits listed under Item 15(b), which are incorporated herein by reference, are filed or furnished as part of this report or are incorporated into this report by reference.
(b) Exhibits.

EXHIBIT NO.	IDENTIFICATION OF EXHIBIT
2.1	Debtors' First Amended Joint Plan of Reorganization dated July 30, 2009 and Disclosure Statement (incorporated by reference to as Exhibit 10.2 to our Quarterly Report on Form 10-Q for the quarter ended June 30, 2009, filed on August 12, 2009 and as Exhibit 99.1 to our Form 8-K, filed September 2, 2009)
3.1	Restated Certificate of Incorporation (incorporated by reference to Exhibit 3.1 to our Current Report on Form 8-K, filed December 13, 2012)
3.2	Certificate of Amendment of the Restated Certificate of Incorporation filed April 26, 2013 (incorporated by reference to Exhibit 3.1 to our Current Report on Form 8-K, filed April 29, 2013)
3.3	Certificate of Amendment to the Company's Restated Certificate of Incorporation, as amended, filed July 19, 2013 (incorporated by reference to Exhibit 3.1 to our Current Report on Form 8-K filed July 22, 2013)
3.4	Certificate of Amendment of the Restated Certificate of Incorporation filed July 12, 2016 (incorporated by reference to Exhibit 3.1 to our Quarterly Report on Form 10-Q for the quarter ended June 30, 2016, filed August 4, 2016)
3.5	Certificate of Amendment of the Restated Certificate of Incorporation of Fibrocell Science, Inc., as amended, dated March 10, 2017 (incorporated by reference to Exhibit 3.1 to our Current Report on Form 8-K, filed on March 10, 2017)
3.6	Certificate of Amendment of the Restated Certificate of Incorporation of Fibrocell Science, Inc., as amended, dated May 24, 2018 (incorporated by reference to Exhibit 3.1 to our Current Report on Form 8-K, filed on May 24, 2018)
3.7	Certificate of Designation of Preferences, Rights and Limitations of Series A Convertible Preferred Stock) incorporated by reference to Exhibit 3.1 to our Current Report on Form 8-K, filed on March 8, 2017)
3.8	Fourth Amended and Restated Bylaws (incorporated by reference to Exhibit 3.1 to our Quarterly Report on Form 10-Q for the quarter ended March 31, 2015, filed on May 8, 2015)
3.9	Amendment to Fourth Amended and Restated Bylaws (incorporated by reference to Exhibit 3.2 to our Quarterly Report on Form 10-Q for the quarter ended March 31, 2015, filed May 8, 2015)
4.1	Form of Common Stock Certificate (incorporated by reference to Exhibit 4.1 to our Quarterly Report on Form 10-Q for the quarter ended September 30, 2009, filed November 23, 2009)
4.2	Form of Convertible Promissory Note (incorporated by reference to Exhibit 4.1 to our Current Report on Form 8-K, filed September 8, 2016)
4.3	Form of Common Stock Purchase Warrant (incorporated by reference to Exhibit 4.2 to our Current Report on Form 8-K, filed September 8, 2016)
4.4	Form of Common Stock Purchase Warrant (incorporated by reference to Exhibit 4.1 to our Current Report on Form 8-K, filed on March 8, 2017)

Table of Contents

4.5	Form of Common Stock Purchase Warrant (incorporated by reference to Exhibit 4.2 to our Current Report on Form 8-K, filed December 11, 2017)
4.6	Form of Pre-Funded Common Stock Purchase Warrant (incorporated by reference to Exhibit 4.3 to our Current Report on Form 8-K, filed December 11, 2017)
4.7	Form of Underwriter's Common Stock Purchase Warrant issued in December 2017 offering (incorporated by reference to Exhibit 4.1 to our Current Report on Form 8-K, filed December 11, 2017)
4.8	Form of Warrant (incorporated by reference to Exhibit 4.1 to our Current Report on Form 8-K, filed May 31, 2018)
4.9	Form of Placement Agent Warrant (incorporated by reference to Exhibit 4.2 to our Current Report on Form 8-K, filed May 31, 2018)
4.10	Form of Warrant (incorporated by reference to Exhibit 4.1 to our Current Report on Form 8-K, filed July 5, 2018)
4.11	Form of Placement Agent Warrant (incorporated by reference to Exhibit 4.2 to our Current Report on Form 8-K, filed July 5, 2018)
10.1	Lease Agreement between Isolagen, Inc. and The Hankin Group dated April 7, 2005 (incorporated by reference to Exhibit 10.1 to our Current Report on Form 8-K, filed April 12, 2005)
10.2	Amendment to Lease Agreement between Fibrocell Science, Inc. and The Hankin Group dated February 17, 2012 (incorporated by reference to Exhibit 10.17 to our Annual Report on Form 10-K for the fiscal year ended December 31, 2011, filed March 30, 2012)
10.3	Registration Rights Agreement dated October 5, 2012 (incorporated by reference to Exhibit 10.2 to our Current Report on Form 8-K, filed October 9, 2012)
10.4	Stock Issuance Agreement dated October 5, 2012 between the Company and Intrexon Corporation (incorporated by reference to Exhibit 10.3 to our Current Report on Form 8-K, filed October 9, 2012)
10.5	Exclusive Channel Collaboration Agreement between Intrexon Corporation and Fibrocell Science, Inc. (incorporated by reference to Exhibit 10.21 to our Annual Report on Form 10-K for the fiscal year ended December 31, 2012, filed April 1, 2013)
10.6	First Amendment to Exclusive Channel Collaboration Agreement between the Company and Intrexon Corporation dated June 28, 2013 (incorporated by reference to Exhibit 10.1 to our Current Report on Form 8-K, filed July 1, 2013)
10.7	Supplemental Stock Issuance Agreement between the Company and Intrexon Corporation dated June 28, 2013 (incorporated by reference to Exhibit 10.2 to our Current Report on Form 8-K, filed July 1, 2013)
10.8	Second Amendment to Exclusive Channel Collaboration Agreement between the Company and Intrexon Corporation dated January 10, 2014 (incorporated by reference to Exhibit 10.1 to our Current Report on Form 8-K, filed January 13, 2014)
10.9	Supplemental Stock Issuance Agreement between the Company and Intrexon Corporation dated January 10, 2014 (incorporated by reference to Exhibit 10.2 to our Current Report on Form 8-K, filed January 13, 2014)
10.10	Letter Agreement to Exclusive Channel Collaboration Agreement, as amended, between Fibrocell Science, Inc. and Intrexon Corporation dated September 29, 2015 (incorporated by reference to Exhibit 10.2 to our Quarterly Report on Form 10-Q for the quarter ended September 30, 2015, filed November 5, 2015)
10.11t	Exclusive Channel Collaboration Agreement, dated December 31, 2015, between Fibrocell Science, Inc. and Intrexon Corporation (incorporated by reference to Exhibit 99.1 to our Current Report on Form 8-K, filed January 4, 2016)
10.12U	Fibrocell Science, Inc. 2009 Equity Incentive Plan, as amended and restated as of March 11, 2017 (incorporated by reference to Exhibit 10.1 to our Current Report on Form 8-K, filed June 20, 2014)
10.13	Form of Nonqualified Stock Option Agreement for Employee Grants under Fibrocell Science, Inc. 2009 Equity Incentive Plan (incorporated by reference to Exhibit 10.2 to our Quarterly Report on Form 10-Q for the quarter ended March 31, 2015, filed May 8, 2015)
10.14	Form of Nonqualified Stock Option Agreement for Director Grants under Fibrocell Science, Inc. 2009 Equity Incentive Plan (incorporated by reference to Exhibit 10.3 to our Quarterly Report on Form 10-Q for the

	quarter ended March 31, 2015, filed May 8, 2015)
10.15	Form of Incentive Stock Option Agreement for Employee Grants under Fibrocell Science, Inc. 2009 Equity Incentive Plan (incorporated by reference to Exhibit 10.4 to our Quarterly Report on Form 10-Q for the quarter ended March 31, 2015, filed May 8, 2015)
10.16U	Employment Agreement between the Company and John Maslowski dated September 14, 2015 (incorporated by reference to Exhibit 10.1 to our Current Report on Form 8-K, filed September 16, 2015)

Table of Contents

10.17U	Offer Letter by and between the Company and John M. Maslowski dated December 18, 2016 (incorporated by reference to Exhibit 10.2 to our Current Report on Form 8-K, filed December 19, 2016)
10.18	Agreement for the Purchase and Sale of Convertible Debt and Common Stock Warrants dated August 9, 2016 (incorporated by reference to Exhibit 10.1 to our Quarterly Report on Form 10-Q for the quarter ended September 30, 2016, filed on November 3, 2016)
10.19	Form of Registration Rights Agreement (incorporated by reference to Exhibit 10.1 to our Current Report on Form 8-K, filed on September 8, 2016)
10.20	Controlled Equity Offering Sales Agreement by and between the Company and Cantor Fitzgerald & Co. dated January 21, 2016 (incorporated by reference to Exhibit 10.1 of our Current Report on Form 8-K, filed January 21, 2016)
10.21	Form of Securities Purchase Agreement by and between the Company and other signatories thereto dated March 7, 2017 (incorporated by reference to Exhibit 10.1 to our Current Report on Form 8-K, filed on March 8, 2017
10.22	Form of Securities Purchase Agreement (incorporated by reference to Exhibit 10.1 to our Current Report on Form 8-K, filed May 31, 2018)
10.23	Form of Securities Purchase Agreement (incorporated by reference to Exhibit 10.1 to our Current Report on Form 8-K, filed July 5, 2018)
10.24	Common Stock Issuance Agreement, dated December 7, 2018, by and among the Company, EB Research Partnership, Inc. and Epidermolysis Medical Research Foundation (incorporated by reference to Exhibit 10.1 to our Registration Statement on Form S-3, filed on January 18, 2019)
*21	List of Subsidiaries
*23	Consent of PricewaterhouseCoopers LLP
*31	Certification of Principal Executive Officer and Principal Financial Officer pursuant to Rule 13a-14(a) and 15d-14(a), required under Section 302 of the Sarbanes-Oxley Act of 2002
*32	Certification of Principal Executive Officer and Principal Financial Officer pursuant to 18 U.S.C. Section 1350 as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
101.INS	XBRL Instance Document
101.SCH	XBRL Taxonomy Extension Schema Document
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document
101.LAB	XBRL Taxonomy Extension Label Linkbase Document
101.PRE	XBRL Taxonomy Extension Presentation Linkbase Document
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document

* Filed herewith.

U Indicates management contract or compensatory plan or arrangement.

t Confidential treatment has been granted as to certain portions of this exhibit pursuant to Rule 406 of the Securities Act of 1933, as amended, or Rule 24b-2 of the Securities Exchange Act of 1934, as amended.

Item 16. Form 10-K Summary

Not applicable.

Table of Contents

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.

FIBROCELL SCIENCE, INC.

By: /s/ John M. Maslowski
John M. Maslowski
President and Chief Executive Officer

Date: March 27, 2019

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 date indicated.

Signature	Title	Date
/s/ John M. Maslowski John M. Maslowski	President and Chief Executive Officer (Principal Executive Officer, Principal Financial Officer and Principal Accounting Officer)	March 27, 2019
/s/ Douglas J. Swirsky Douglas J. Swirsky	Chairman of the Board	March 27, 2019
/s/ Kelvin Moore Kelvin Moore	Director	March 27, 2019
/s/ Marc Mazur Marc Mazur	Director	March 27, 2019
/s/ Julian Kirk Julian Kirk	Director	March 27, 2019
/s/ Marcus Smith Marcus Smith	Director	March 27, 2019
/s/ Christine St.Clare Christine St.Clare	Director	March 27, 2019

Table of Contents

Fibrocell Science, Inc.
Index to Consolidated Financial Statements

Table of Contents

Report of Independent Registered Public Accounting Firm

To the Board of Directors and Stockholders of Fibrocell Science, Inc.

Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheets of Fibrocell Science, Inc. and its subsidiaries ("the Company") as of December 31, 2018 and 2017, and the related consolidated statements of operations, stockholders' equity and cash flows for the years then ended, including the related notes (collectively referred to as the "consolidated financial statements"). In our opinion, the consolidated financial statements present fairly, in all material respects, the financial position of the Company as of December 31, 2018 and 2017, and the results of its operations and its cash flows for the years then ended in conformity with accounting principles generally accepted in the United States of America.

Substantial Doubt About the Company's Ability to Continue as a Going Concern

The accompanying consolidated financial statements have been prepared assuming that the Company will continue as a going concern. As discussed in Note 1 to the consolidated financial statements, the Company has suffered recurring losses from operations, has an accumulated deficit and cash outflows from operating activities that raise substantial doubt about its ability to continue as a going concern. Management's plans in regard to these matters are also described in Note 1. The consolidated financial statements do not include any adjustments that might result from the outcome of this uncertainty.

Basis for Opinion

These consolidated financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's consolidated financial statements based on our audits. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) (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 of these consolidated financial statements 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 consolidated financial statements are free of material misstatement, whether due to error or fraud. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. As part of our audits we are required to obtain an understanding of internal control over financial reporting but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion.

Our audits included performing procedures to assess the risks of material misstatement of the consolidated 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 consolidated 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 consolidated financial statements. We believe that our audits provide a reasonable basis for our opinion.

/s/ PricewaterhouseCoopers LLP

Philadelphia, Pennsylvania
March 27, 2019

We have served as the Company's auditor since 2015.

Table of Contents

Fibrocell Science, Inc.
Consolidated Balance Sheets
($ in thousands, except share data)

	As of December 31,	
	2018	2017
Assets		
Current assets:		
Cash and cash equivalents	$14,430	$17,417
Prepaid expenses and other current assets	105	485
Total current assets	14,535	17,902
Property and equipment, net of accumulated depreciation of $2,311 and $1,919, respectively	1,222	1,470
Other assets	1	39
Total assets	$15,758	$19,411
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$452	$862
Related party payable	100	2,303
Accrued expenses	1,470	1,260
Deferred rent, current	150	—
Total current liabilities	2,172	4,425
Convertible promissory notes, net of debt discount of $18,003 and $18,003, respectively (see Note 6)	—	—
Accrued interest payable	1,738	967
Warrant liability	152	1,073
Derivative liability	1,474	3,136
Deferred rent	665	803
Total liabilities	6,201	10,404
Commitments and contingencies (Note 14)		
Stockholders' equity:		
Preferred stock, $0.001 par value; 5,000,000 shares authorized; 8,000 shares issued and outstanding as of December 31, 2018; 5,000,000 shares authorized, 8,000 shares issued and outstanding as of December 31, 2017; aggregate liquidation preference of $8,600 at December 31, 2018 and $8,264 at December 31, 2017	—	—
Common stock, $0.001 par value; 150,000,000 shares authorized, 9,758,332 shares issued and outstanding as of December 31, 2018; 150,000,000 shares authorized, 5,189,755 shares issued and outstanding as of December 31, 2017	10	5
Additional paid-in capital	198,627	187,805
Accumulated deficit	(189,080)	(178,803)
Total stockholders' equity	9,557	9,007
Total liabilities and stockholders' equity	$15,758	$19,411

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

F 3

Table of Contents

Fibrocell Science, Inc.
Consolidated Statements of Operations
($ in thousands, except share and per share data)

	Year Ended December 31,	
	2018	2017
Revenues:		
Total revenues	$—	$—
Operating expenses:		
Research and development expenses	6,018	6,512
Research and development expenses - related party	(34)	5,720
Selling, general and administrative expenses	6,405	6,749
Total operating expenses	12,389	18,981
Loss from operations	(12,389)	(18,981)
Other income (expense):		
Warrant revaluation income	921	4,920
Derivative revaluation income (expense)	1,662	(1,407)
Interest expense	(771)	(828)
Other income, net	300	56
Loss before income taxes	(10,277)	(16,240)
Income taxes	—	—
Net loss	$(10,277)	$(16,240)
Dividend paid in-kind to preferred stockholders	(336)	(264)
Deemed dividend on preferred stock (see Note 8)	(513)	(4,099)
Net loss attributable to common stockholders	$(11,126)	$(20,603)
Per Share Information:		
Net loss		
— Basic	$(1.45)	$(6.66)
— Diluted	$(1.45)	$(6.67)
Weighted average number of common shares outstanding		
— Basic	7,693,191	3,092,543
— Diluted	7,693,191	3,093,727

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

Table of Contents

Fibrocell Science, Inc.
Consolidated Statements of Stockholders' Equity
($ in thousands, except share data)

	Preferred Stock		Common Stock		Additional paid-in capital	Accumulated deficit	Total Equity
	Shares	Amount	Shares	Amount			
Balance, December 31, 2016	—	$ —	2,939,329	$ 15	$ 170,409	$ (162,563)	$ 7,861
Effect of the March 2017 and May 2018 reverse stock splits on common stock and additional paid in capital, beginning balance	—	—	—	(12)	12	—	—
Issuance of Series A convertible preferred stock with detachable warrants net of issuance costs of $377	8,000	—	—	—	7,623	—	7,623
Stock-based compensation expense	—	—	—	—	322	—	322
Exercise of liability-classified warrants	—	—	1,389	—	41	—	41
Conversion of promissory notes	—	—	4,984	—	95	—	95
Issuance of common stock with detachable warrants net of issuance costs $1,175	—	—	2,244,053	2	9,303	—	9,305
Net loss	—	—	—	—	—	(16,240)	(16,240)
Balance, December 31, 2017	8,000	$ —	5,189,755	$ 5	$ 187,805	$ (178,803)	$ 9,007
Conversion of pre-funded units	—	—	483,221	1	23	—	24
Stock-based compensation expense	—	—	—	—	526	—	526
May 2018 Registered Direct Offering, net of offering costs $676	—	—	2,038,224	2	5,322	—	5,324
July 2018 Registered Direct Offering, net of offering costs $494	—	—	1,474,080	1	3,590	—	3,591
December 2018 Private Placement, net of offering costs $37	—	—	443,350	1	862	—	863
Common warrants exercised	—	—	129,702	—	499	—	499
Net loss	—	—	—	—	—	(10,277)	(10,277)
Balance, December 31, 2018	8,000	$ —	9,758,332	$ 10	$ 198,627	$ (189,080)	$ 9,557

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

Table of Contents

Fibrocell Science, Inc.
Consolidated Statements of Cash Flows
($ in thousands)

	Year Ended December 31,	
	2018	2017
Cash flows from operating activities:		
Net loss	$ (10,277)	$ (16,240)
Adjustments to reconcile net loss to net cash used in operating activities:		
Stock-based compensation expense	526	322
Warrant liability revaluation income	(921)	(4,920)
Derivative liability revaluation expense (income)	(1,662)	1,407
Loss on disposal or impairment of property and equipment	—	40
Depreciation and amortization	392	384
Amortization of discount on convertible debt converted to common shares	—	86
Decrease (increase) in operating assets:		
Prepaid expenses and other current assets	380	28
Other assets	38	26
Increase (decrease) in operating liabilities:		
Accounts payable	(24)	69
Related party payable	(2,203)	1,361
Accrued expenses and deferred rent	263	(342)
Accrued interest payable	771	742
Net cash used in operating activities	(12,717)	(17,037)
Cash flows from investing activities:		
Purchase of property and equipment	(164)	(433)
	(164)	(433)

Net cash used in investing activities						
Cash flows from financing activities:						
Proceeds from private placement	900			7,623		
Proceeds from common stock offerings, (net of offering costs of $1,170)	8,915			9,749		
Proceeds from exercise of common warrants	499			—		
Proceeds from exercise of pre-funded warrants	24			—		
Payment of deferred offering costs	(444		)	—		
Net cash provided by financing activities	9,894			17,372		
Net decrease in cash and cash equivalents	(2,987		)	(98		)
Cash and cash equivalents, beginning of period	17,417			17,515		
Cash and cash equivalents, end of period	$	14,430		$	17,417	
Supplemental disclosures of cash flow information:						
Non-cash investing and financing activities:						
Property and equipment in accounts payable	$	9		$	29	
Offering costs in accounts payable and accrued expenses	$	37		$	444	
Reduction of warrant liability upon cashless exercise of warrants	$	—		$	41	
Reduction of accrued interest payable upon cashless exercise of promissory notes	$	—		$	3	
Reduction in derivative liability upon cashless exercise of promissory notes	$	—		$	6	

Cashless exercise of promissory notes	$	—	$	85
Dividend paid in-kind to preferred stockholders	$	336	$	264
Deemed dividend on preferred stock	$	513	$	4,099

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

Table of Contents
Fibrocell Science, Inc.
Notes to Consolidated Financial Statements

Note 1. Business and Organization

Organization

Fibrocell Science, Inc. (as used herein, “we,” “us,” “our,” “Fibrocell” or the “Company”) is the parent company of Fibrocell Technologies, Inc. (Fibrocell Tech). Fibrocell Tech is the parent company of Isolagen International, S.A., a company organized under the laws of Switzerland (Isolagen Switzerland). The Company’s international activities are currently immaterial.

Business Overview

Fibrocell is an autologous cell and gene therapy company translating personalized biologics into medical breakthroughs. The Company is focused on discovering and developing therapies for the localized treatment of diseases affecting the skin and connective tissue. All of the Company’s product candidates incorporate its proprietary autologous fibroblast technology. The Company’s research and development efforts focus on gaining regulatory approvals of its product candidates in the United States.

Liquidity and Financial Condition

The Company expects to continue to incur losses and will require additional capital to advance its product candidates through development to commercialization. For the year ended December 31, 2018, the Company incurred a net loss of approximately $10.3 million, had an accumulated deficit of $189.1 million and used approximately $12.7 million in cash for operations. As of December 31, 2018, the Company had cash and cash equivalents of approximately $14.4 million and working capital of approximately $12.4 million. The Company believes that its cash and cash equivalents at December 31, 2018, will be sufficient to fund operations into the fourth quarter of 2019. The Company will require additional capital to fund operations beyond that point. To meet its capital needs, the Company intends to raise additional capital through debt or equity financings, collaborations, partnerships or other strategic transactions. However, there can be no assurance that the Company will be able to complete any such transaction on acceptable terms or otherwise. The failure of the Company to obtain sufficient funds on acceptable terms when needed could have a material adverse effect on the Company’s business, results of operations and financial condition. These conditions raise substantial doubt about its ability to continue as a going concern. The accompanying consolidated financial statements do not include any adjustments that might result from the outcome of this uncertainty. Accordingly, the consolidated financial statements have been prepared on a basis that assumes the Company will continue as a going concern and which contemplates the realization of assets and satisfaction of liabilities and commitments in the ordinary course of business.

On January 23, 2018, the Company received notice (the Notice) from the Nasdaq Capital Market (Nasdaq) that it was not in compliance with Nasdaq Listing Rule 5550(a)(2), as the minimum bid price of the Company’s common stock had been below $1.00 per share for 30 consecutive business days. On May 24, 2018, the Company implemented a one-for-five reverse split of its issued and outstanding shares of the Company’s common stock (the 2018 Reverse Stock Split), as authorized at the annual meeting of stockholders on May 23, 2018. The 2018 Reverse Stock Split became effective on May 24, 2018 at 5:00 pm and the Company’s common stock began trading on Nasdaq on a post-split basis at the open of business on May 25, 2018. As a result of the 2018 Reverse Stock Split, every five shares of the Company’s issued and outstanding common stock were combined into one share of its common stock, except to the extent that the 2018 Reverse Stock Split resulted in any of the Company’s stockholders owning a fractional share, which was rounded up to the next highest whole share. In connection with the 2018 Reverse Stock Split, there was no

change in the nominal par value per share of $0.001. The 2018 Reverse Stock Split was effectuated in order to increase the per share trading price of the Company’s common stock to satisfy the $1.00 minimum bid price requirement for continued listing on Nasdaq. On June 11, 2018, the Company received written notice from Nasdaq notifying the Company that the closing bid price for the Company's common stock had been at $1.00 per share or greater for a minimum of ten consecutive business days and accordingly, the Company had regained compliance with Nasdaq Listing Rule 5550(a)(2). All share and per share amounts of common stock, options and warrants in the accompanying financial statements and related footnotes, have been restated for all periods to give retroactive effect to the 2018 Reverse Stock Split. Accordingly, the Condensed Consolidated Statement of Stockholders’ Equity reflects the impact of the 2018 Reverse Stock Split by reclassifying from “Common Stock” to “Additional paid in capital” an amount equal to the par value of the decreased shares resulting from the 2018 Reverse Stock Split.

Nasdaq has the authority, pursuant to Nasdaq Listing Rule 5550(b)(1), to delist the Company's common stock if its stockholders' equity falls below $2.5 million. As of December 31, 2018, the Company's stockholders' equity was $9.6 million. If the Company's stockholders equity is hereafter reduced below $2.5 million as a result of operating losses or for other reasons, the Company will fail to meet Nasdaq's stockholders' equity requirement. If that occurs, or if the Company is unable to demonstrate to Nasdaq's satisfaction that it will be able to sustain compliance with this requirement, Nasdaq may delist the Company's common stock. In addition, even if the Company regains technical compliance with the stockholders' equity requirement, the Company will have to continue to meet other objective and subjective listing requirements to continue to be listed on the Nasdaq Capital Market, including the requirement that our common stock continues to trade above $1.00.

The Company is actively monitoring its stockholders' equity and will consider any and all options available to it to maintain compliance. There can be no assurance, however, that the Company will be able to maintain compliance and meet Nasdaq's minimum stockholders' equity requirements.

Table of Contents
Fibrocell Science, Inc.
Notes to Consolidated Financial Statements

Note 2. Basis of Presentation

General

The accompanying Consolidated Financial Statements have been prepared in accordance with U.S. generally accepted accounting principles (GAAP) and include the accounts of Fibrocell and its wholly owned subsidiaries. The accompanying Consolidated Financial Statements should be read in conjunction with the Notes to the Consolidated Financial Statements.

All intercompany accounts and transactions have been eliminated in consolidation. The Company's foreign operations are immaterial and it has no unrealized gains or losses from the sale of investments. As a result, it does not have any items that would be classified as other comprehensive income in such a statement.

Reclassifications

On May 24, 2018, the Company implemented the 2018 Reverse Stock Split, as authorized at the annual meeting of stockholders on May 23, 2018. The 2018 Reverse Stock Split became effective on May 24, 2018 at 5:00 pm and the Company's common stock began trading on Nasdaq on a post-split basis at the open of business on May 25, 2018. As of a result of the 2018 Reverse Stock Split, every five shares of the Company's issued and outstanding common stock were combined into one share of its common stock, except to the extent that the 2018 Reverse Stock Split resulted in any of the Company's stockholders owning a fractional share, which was rounded up to the next highest whole share. In connection with the 2018 Reverse Stock Split, there was no change in the nominal par value per share of $0.001. The 2018 Reverse Stock Split was effectuated in order to increase the per share trading price of the Company's common stock to satisfy the $1.00 minimum bid price requirement for continued listing on Nasdaq. By letter dated June 11, 2018, The Nasdaq Capital Market Listing Qualification Department, confirmed that the Company's common stock was in compliance with listing requirements.
On March 10, 2017, the Company implemented a one-for-three reverse split of its issued and outstanding shares of common stock (the 2017 Reverse Stock Split and together with the 2018 Reverse Stock Split, the Reverse Stock Splits), as authorized at a special meeting of stockholders on March 1, 2017. The 2017 Reverse Stock Split became effective on March 10, 2017 at 5:00 pm and the Company's common stock began trading on Nasdaq on a post-split basis at the open of business on March 13, 2017. As a result of the 2017 Reverse Stock Split, every three shares of the Company's issued and outstanding common stock were combined into one share of its common stock, except to the extent that the 2017 Reverse Stock Split resulted in any of the Company's stockholders owning a fractional share, which was rounded up to the next highest whole share. In connection with the 2017 Reverse Stock Split, there was no change in the nominal par value per share of $0.001. The 2017 Reverse Stock Split was effectuated in order to increase the per share trading price of the Company's common stock to satisfy the $1.00 minimum bid price requirement for continued listing on Nasdaq. By letter dated March 27, 2017, The Nasdaq Capital Market Listing Qualification Department, confirmed that the Company's common stock was in compliance with listing requirements.
All share and per share amounts of common stock, options and warrants in the accompanying financial statements have been restated for all periods to give retroactive effect to the Reverse Stock Splits. Accordingly, the Consolidated Statement of Stockholders' Equity reflects the impact of the Reverse Stock Splits by reclassifying from "Common Stock" to "Additional paid-in capital" an amount equal to the par value of the decreased shares resulting from the Reverse Stock Splits.
Note 3. Summary of Significant Accounting Policies

Use of Estimates

The preparation of financial statements in conformity with GAAP requires management to make estimates, judgments, and assumptions that affect the reported amounts of assets, liabilities, equity, revenues and expenses, and related disclosure of contingencies in the accompanying Consolidated Financial Statements and Notes. In addition, management's assessment of the Company's ability to continue as a going concern involves the estimation of the amount and timing of future cash inflows and outflows. On an ongoing basis, the Company evaluates its estimates, judgments and methodologies. The Company bases its estimates on historical experience and on various other assumptions that are believed to be reasonable. Actual results may differ materially from those estimates.

Segment Information

The Company has determined that it operates in only one segment, as it only reports operational results on an aggregate basis to its chief operating decision maker, the Company's President and Chief Executive Officer. Additionally, all of the Company's research and development activities occur in, and assets are located in, the United States.

Cash and Cash Equivalents

The Company considers highly liquid investments with an original maturity of three months or less when purchased to be cash equivalents.

Concentration of Credit Risk

Financial instruments that potentially subject the Company to concentrations of credit risk are limited to the Company's cash and cash equivalents. As of December 31, 2018, the Company maintains its operating cash with one major U.S. domestic bank and the remainder of its cash and cash equivalents as a money market fund with one major global bank. Federal insurance coverage on operating cash amounted to $250,000 per depositor at each financial institution, and the Company's non-interest bearing cash balances may exceed federally insured limits. The terms of these deposits are on demand to minimize risk. The Company has not incurred losses related to these deposits.

Property and Equipment

Property and equipment is carried at acquisition cost less accumulated depreciation, subject to review for impairment whenever events or changes in circumstances indicate that the carrying amount of the asset may not be recoverable as described further under the heading "Impairment of Long-lived Assets" below. The cost of normal, recurring, or periodic repairs and maintenance activities related to property and equipment are expensed as incurred. The cost for planned major maintenance activities, including the related acquisition or construction of assets, is capitalized if the repair will result in future economic benefits.

Depreciation is computed on a straight-line basis over the estimated useful life of the respective assets, which are summarized as follows:

Property and equipment category	Useful life
Computer equipment and software	3 years
Laboratory equipment	6 years
Furniture and fixtures	10 years
Leasehold improvements	Lesser of remaining lease term or life of asset

When an asset is disposed of, the associated cost and accumulated depreciation is removed from the related accounts on the Company's Consolidated Balance Sheet with any resulting gain or loss included in the Company's Consolidated Statement of Operations.

Impairment of Long-Lived Assets

In accordance with Financial Accounting Standards Board (FASB) Accounting Standards Codification (ASC) 360-10-35, Impairment or Disposal of Long-Lived Assets, the Company reviews its long-lived assets and identifiable

finite-lived intangible assets for impairment whenever events or changes in circumstances indicate that the carrying amount of an asset may not be recoverable (i.e. impaired). Once an impairment is determined, the actual impairment recognized would be the difference between the carrying amount and the fair value (less any costs for disposal). The Company uses the following approaches to determine fair value: income, cost and/or market. Fair value using the income approach is determined primarily using a discounted cash flow model that uses the estimated cash flows associated with the asset or asset group under review, discounted at a rate commensurate with the risk involved. Fair value utilizing the cost approach is determined based on the replacement cost of the asset reduced for, among other things, depreciation and obsolescence. Fair value, utilizing the market approach, benchmarks the fair value against the carrying amount.

Warrant Liability

The Company accounts for stock warrants as either equity instruments, derivative liabilities, or liabilities in accordance with ASC 480, Distinguishing Liabilities from Equity (ASC 480), depending on the specific terms of the warrant agreement. Stock warrants are accounted for as a derivative in accordance with ASC 815, Derivatives and Hedging (ASC 815) if the stock warrants contain terms that could potentially require “net cash settlement” and therefore, do not meet the scope exception for treatment as a derivative. Warrant instruments that could potentially require “net cash settlement” in the absence of express language precluding such settlement are initially classified as derivative liabilities at their estimated fair values, regardless of the likelihood that such instruments will ever be settled in cash. The Company will continue to classify the fair value of the warrants that contain “net cash settlement” as a liability until the warrants are exercised, expire or are amended in a way that would no longer require these warrants to be classified as a liability. Warrants that the Company may be required to redeem through payment of cash or other assets outside its control are classified as liabilities pursuant to ASC 480 and are initially and subsequently measured at their estimated fair values. For additional discussion on warrants, see Note 7.

Debt Issued With Warrants

The Company considers guidance within ASC 470-20, Debt (ASC 470), ASC 480, and ASC 815 when accounting for the issuance of convertible debt with detachable warrants. As described above under the caption “Warrant Liability”, the Company classifies stock warrants as either equity instruments, derivative liabilities, or liabilities depending on the specific terms of the warrant agreement. In circumstances in which debt is issued with liability-classified warrants, the proceeds from the issuance of convertible debt are first allocated to the warrants at their full estimated fair value and established as both a liability and a debt discount. The remaining proceeds, as further reduced by discounts created by the bifurcation of embedded derivatives and beneficial conversion features, are allocated to the debt. The Company accounts for debt as liabilities measured at amortized cost and amortizes the resulting debt discount from the allocation of proceeds, to interest expense using the effective interest method over the expected term of the debt instrument pursuant to ASC 835, Interest (ASC 835).

Embedded Derivatives. The Company considers whether there are any embedded features in debt instruments that require bifurcation and separate accounting as derivative financial instruments pursuant to ASC 815. Embedded derivatives are initially and subsequently measured at fair value. See Note 6 for additional discussion on the embedded derivatives associated with the Company’s convertible notes.

Beneficial Conversion Feature. If the amount allocated to the convertible debt results in an effective per share conversion price less than the fair value of the Company’s common stock on the commitment date, the intrinsic value of this beneficial conversion feature is recorded as a discount to the convertible debt with a corresponding increase to additional paid in capital. The beneficial conversion feature discount is equal to the difference between the effective conversion price and the fair value of the Company’s common stock at the commitment date, unless limited by the remaining proceeds allocated to the debt. See Note 6 for additional discussion on the beneficial conversion feature associated with the Company’s convertible notes.

Debt Issuance Costs. The Company follows the guidance under Accounting Standards Update (ASU) 2015-03, Interest - Imputation of Interest (Subtopic 835-30): Simplifying the Presentation of Debt Issuance Costs (ASU 2015-03) for accounting for debt issuance costs. The Company allocates debt issuance costs between the debt and the warrants on the same basis as proceeds were allocated. The Company expenses issuance costs allocated to the

warrants and presents the issuance costs allocated to the debt as a direct reduction from the carrying amount of the debt liability in the balance sheet. However, if debt issuance costs exceed the carrying amount of the debt, issuance costs are recorded to additional paid-in capital as a reduction of the beneficial conversion feature. As of December 31, 2018, the Company's debt issuance costs are presented in additional paid-in capital as a reduction of the beneficial conversion feature and are being amortized to interest expense (despite their classification in additional paid-in capital) using the effective interest rate method over the expected term of the debt pursuant to ASC 835.

Research and Development Expenses

Research and development costs are expensed as incurred and include employee salaries and benefits, costs incurred with third party contractors to perform research, conduct clinical trials, develop and manufacture drug materials and delivery devices, and a portion of facilities costs. Research and development expenses also include costs to manufacture product for clinical trial use and to develop manufacturing, cell collection and logistical process improvements.

Clinical trial costs are a significant component of research and development expenses, often with third party service providers. Invoicing from third party contractors for services performed can lag several months. The Company accrues the costs of services rendered in connection with third party contractor activities based on its estimate of management fees, site management and monitoring costs and data management costs incurred in a given period.

Stock-Based Compensation

The Company follows ASC 718, Compensation – Stock Compensation (ASC 718), or ASC 505-50, Equity – Equity Based Payments to Non-Employees, where applicable. The Company accounts for stock-based awards to employees using the fair value based method to determine compensation for all arrangements where shares of stock or equity instruments are issued for compensation. In addition, the Company accounts for stock-based compensation to non-employees in accordance with the accounting guidance for equity instruments that are issued to entities or persons other than employees. The Company uses a Black-Scholes option-pricing model to determine the fair value of each option grant as of the date of grant for expense incurred. The Black-Scholes option pricing model requires inputs for risk-free interest rate, dividend yield, expected stock price volatility and expected term of the options. The value of the award vests based on the achievement of the requisite service period. The expense is recognized on a straight line basis. See Note 10 for additional details.

The Company considered the guidance in ASU 2016-09, Compensation - Stock Compensation (Topic 718): Improvements to Employee Share-Based Payment Accounting (ASU 2016-09), which allows an entity to elect as an accounting policy either to continue to estimate the total number of awards for which the requisite service period will not be rendered or to account for forfeitures when they occur. In connection with the adoption of this ASU, the Company made an accounting policy election to account for forfeitures as they occur.

Income Taxes

An asset and liability approach is used for financial accounting and reporting for income taxes. Deferred income taxes arise from temporary differences between income tax and financial reporting and principally relate to recognition of revenue and expenses in different periods for financial and tax accounting purposes and are measured using currently enacted tax rates and laws. In addition, a deferred tax asset can be generated by a net operating loss carryover. If it is more likely than not that some portion or all of a deferred tax asset will not be realized, a valuation allowance is recognized.

In the event the Company is charged interest or penalties related to income tax matters, the Company would record such interest as interest expense and would record such penalties as other expense in the Consolidated Statements of Operations. No such charges have been incurred by the Company. For each of the years ended December 31, 2018 and 2017, the Company had no uncertain tax positions. See Note 11 for additional details.

Loss Per Share Data

Basic loss per share is computed by dividing net loss for the period by the weighted average number of shares of common stock outstanding during that period. The diluted loss per share calculation gives effect to dilutive stock options, warrants, convertible notes and other potentially dilutive common stock equivalents outstanding during the period. Diluted loss per share is based on the if-converted method or the treasury stock method, as applicable, and includes the effect from the potential issuance of common stock, such as shares issuable pursuant to the conversion of

convertible notes and the exercise of stock options and warrants, assuming the exercise of all “in-the-money” common stock equivalents based on the average market price during the period. Common stock equivalents have been excluded where their inclusion would be anti-dilutive. See Note 13 for additional details.

Recently Issued Accounting Pronouncements

From time to time, new accounting pronouncements are issued by the Financial Accounting Standards Board (FASB) and rules are issued by the Securities and Exchange Commission (SEC) that we adopt as of the specified date. Unless otherwise noted, management does not believe that any other recently issued accounting pronouncements issued by the FASB or guidance issued by the SEC had, or is expected to have, a material impact on the Company’s present or future consolidated financial statements.

In June 2018, the FASB issued ASU 2018-07, “Compensation – Stock Compensation (Topic 718).” ASU 2018-07 simplifies the accounting for nonemployee share-based payment transactions. This ASU is effective for public entities for

interim and annual reporting periods beginning after December 15, 2018. The Company has evaluated the potential impact of this guidance and does not believe that it will have a material impact on the Company's financial statements.

In July 2017, the FASB issued ASU No. 2017-11, "Earnings Per Share (Topic 260), Distinguishing Liabilities from Equity (Topic 480) and Derivatives and Hedging (Topic 815): Part 1 - Accounting for Certain Financial Instruments with Down Round Features and Part 2 - Replacement of the Indefinite Deferral for Mandatorily Redeemable Financial Instruments of Certain Nonpublic Entities and Certain Mandatorily Redeemable Noncontrolling Interests with Scope Exception." Part 1 of ASU No. 2017-11 addresses the complexity of accounting for certain financial instruments with down round features. Down round features are features of certain equity-linked instruments (or embedded features) that result in the strike price being reduced on the basis of the pricing of future equity offerings. Current accounting guidance creates cost and complexity for entities that issue financial instruments (such as warrants and convertible instruments) with down round features that require fair value measurement of the entire instrument or conversion option. Part II of ASU No. 2017-11 addresses the difficulty of navigating Topic 480, Distinguishing Liabilities from Equity, because of the existence of extensive pending content in the FASB Accounting Standards Codification®. This pending content is the result of the indefinite deferral of accounting requirements about mandatorily redeemable financial instruments of certain nonpublic entities and certain mandatorily redeemable noncontrolling interests. For public business entities, the amendments in Part I of this update are effective for fiscal years, and interim periods within those fiscal years, beginning after December 15, 2018. The amendments in Part II of this update do not require any transition guidance because those amendments do not have an accounting effect. The Company currently does not have any outstanding financial instruments with down round provisions, and therefore the impact of the adoption of this standard on its Consolidated Financial Statements, will not be material.

In February 2016, the FASB issued ASU 2016-02, Leases (Topic 842). Under the new guidance, lessees (including lessees under both leases classified as finance leases, which are to be classified based on criteria similar to that applicable to capital leases under current guidance, and leases classified as operating leases) will recognize a right-to-use asset and a lease liability on the balance sheet, initially measured as the present value of lease payments under the lease. Under current guidance, operating leases are not recognized on the balance sheet. The standard is effective for fiscal years, and interim periods within those fiscal years, beginning after December 15, 2018. Early adoption is permitted. The new standard must be adopted using a modified retrospective transition approach for leases with an option to elect a package of practical expedients. Further, companies can elect to apply the transition approach either for leases existing at, or entered into after, the beginning of the earliest comparative period presented in the financial statements or those existing at, or entered into after, the adoption date.
The Company adopted the new standard effective January 1, 2019 and will not restate comparative periods. Presentation of leases within the consolidated statements of operations and consolidated statements of cash flows will be generally consistent with the current lease accounting guidance. The Company will elect the package of practical expedients permitted under the transition guidance and as such, the adoption of Topic 842 will not change the classification of any of its leases. The Company will elect to combine lease and non-lease components, elect not to record leases with an initial term of 12 months or less on the balance sheet and will recognize the associated lease payments in the consolidated statements of operations on a straight-line basis over the lease term. While the Company is currently assessing the full impact this ASU will have on its Consolidated Financial Statements, management believes the primary impact upon adoption will be the recognition, on a discounted basis, of its minimum commitments under the current non-cancellable operating lease, as amended, for its Exton, PA facility. The impact may result in the recording of right of use assets and lease obligations. The Company does not anticipate any other material impacts to its Consolidated Financial Statements.

F 13

Table of Contents
Fibrocell Science, Inc.
Notes to Consolidated Financial Statements

Note 4. Property and Equipment

Property and equipment consisted of the following as of:

	December 31,	
($ in thousands)	2018	2017
Laboratory equipment	$1,562	$1,514
Computer equipment and software	319	318
Furniture and fixtures	44	44
Leasehold improvements	1,449	1,412
Construction-in-process	159	101
Total property and equipment, gross	3,533	3,389
Less: Accumulated depreciation	(2,311)	(1,919)
Total property and equipment, net	$1,222	$1,470

Depreciation expense was approximately $0.4 million for each of the years ended December 31, 2018 and 2017.

Note 5. Accrued Expenses

Accrued expenses consisted of the following as of:

	December 31,	
($ in thousands)	2018	2017
Accrued professional fees	$281	$322
Accrued compensation	449	462
Accrued clinical trial expenses	525	342
Accrued other	215	134
Total accrued expenses	$1,470	$1,260

Table of Contents
Fibrocell Science, Inc.
Notes to Consolidated Financial Statements

Note 6. Convertible Notes

2016 Private Placement

In September 2016, the Company issued an aggregate of $18,087,500 in principal of convertible promissory notes (each, a Note and collectively, the Notes) and accompanying warrants to purchase an aggregate of 1,205,840 shares of common stock (each a Warrant and collectively, the Warrants) in a private placement (the 2016 Private Placement) to institutional and accredited investors (each an Investor and collectively, the Investors).

The Notes bear interest at four percent (4%) per annum. Interest is earned daily and compounded quarterly and, at the election of the Company at the beginning of each quarter, shall accrue or be paid in cash. If the Company elects to have interest accrue, such interest will not be added to the principal amount of the Notes but such interest shall be subject to additional interest at the rate of four percent (4%) per annum, compounded quarterly, and shall be due and payable upon the earliest of the conversion of the Notes, exercise of the Put Right, exercise of the Prepayment Right or the Maturity Date (in each case, as defined below). Additionally, if the Company elects for interest to accrue, then (i) the Company may elect to repay any such accrued and unpaid interest in cash at any time and from time to time and (ii) each Investor may elect to have the Company repay any such accrued and unpaid interest by delivering such number of shares of common stock equal to (x) the amount of the accrued and unpaid interest to be repaid, divided by (y) the greater of (i) the last closing bid price of a share of the Company's common stock as reported on Nasdaq on the date of such election and (ii) the Conversion Price (as defined below). As of December 31, 2018, the Company has elected to accrue interest.

All unpaid principal of each Investor's Note is convertible, at any time and from time to time, at the option of such Investor into shares of common stock at a range of $17.04375 to $18.39375 (the "conversion price"), and any unpaid interest at the greater of (x) the conversion price and (y) the last closing bid price of a share of common stock as reported on Nasdaq at the time of such Investor's execution of the Agreement for the Purchase and Sale of Convertible Debt and Common Stock Warrants plus $0.12625.

The Notes have a maturity date of the earlier of (i) September 7, 2026 and (ii) one-hundred and eighty (180) days after the date on which the Company's product candidate, FCX-007, is approved by the United States Food and Drug Administration for the treatment of recessive dystrophic epidermolysis bullosa (the Maturity Date). Each Investor has the right to require the Company to repay all or any portion of the unpaid principal and accrued and unpaid interest from time to time on or after September 7, 2021 (such right, a Put Right). Such Put Right must be exercised by such Investor by delivering written notice to the Company no later than one-hundred and eighty (180) days prior to such exercise date of such Put Right. In addition, upon consummation of a specified change of control transaction, each Investor may elect to accelerate the repayment of all unpaid principal and accrued interest under such Investor's Note. If an Investor does not elect to have the Company prepay its Note upon such change of control transaction, then the Company may prepay the Notes, in an amount equal to one hundred one percent (101%) of the outstanding principal due under the Notes (together with accrued and unpaid interest due thereon) (the Prepayment Right). Additionally, upon the occurrence of certain Events of Default, as defined in the Notes, each Investor may elect to accelerate the repayment of all unpaid principal and accrued interest under each Note and the Notes provide for automatic redemption upon the occurrence of certain bankruptcy related Events of Default, as defined in the Notes.

Accounting for Convertible Notes and Embedded Derivatives

The Company accounts for debt as liabilities measured at amortized cost and amortizes the resulting debt discount from allocation of proceeds to interest expense using the effective interest method over the expected term of the Notes

pursuant to ASC 835, Interest (ASC 835).

See Note 3 for discussion of the Company's policies for accounting for debt with detachable warrants. In connection with the issuance of the Notes and Warrants, the Company recorded a debt discount of approximately $18.1 million based on an allocation of proceeds to the Warrants of approximately $9.6 million, an allocation to bifurcated derivatives (which consist of a contingent put option upon a change of control or acceleration upon event of default (the Contingent Put Option) and a contingent call option upon a change of control (the Contingent Call Option) included in the Notes) of approximately $1.3 million, and a beneficial conversion feature of approximately $7.2 million, before issuance costs, based on the difference between the fair value of the underlying common stock at the commitment date of each Note transaction and the effective conversion price of the Notes, as limited by the proceeds allocated to the Notes.

Table of Contents
Fibrocell Science, Inc.
Notes to Consolidated Financial Statements
Note 6. Convertible Notes (continued)

Convertible promissory notes outstanding were as follows:

($ in thousands)	December 31, 2018	2017
Convertible promissory notes	$18,003	$18,003
Debt discount - warrants	(9,598)	(9,598)
Debt discount - compound bifurcated derivatives	(1,267)	(1,267)
Debt discount - beneficial conversion feature	(7,138)	(7,138)
Convertible promissory notes, net	$—	$—

The debt discount and issuance costs are amortized using the effective interest method over five years, the expected term of the Notes. Amortization of the debt discounts included in interest expense in the Consolidated Statement of Operations for the year ended December 31, 2018 and December 31, 2017 was approximately $0 and $0.1 million. Based on an effective yield of approximately 1157% resulting from the Notes being initially recorded at a full discount, the Company will not recognize any material amounts of amortization until years 2020 and 2021. The amortization of debt discount recorded in the year ended December 31, 2017 was related to the conversion of notes into shares.

Assumptions Used in Determining Fair Value of Compound Bifurcated Derivative

The Company utilizes a binomial lattice model to value its bifurcated derivatives included in the Notes. ASC 815 does not permit an issuer to account separately for individual derivative terms and features embedded in hybrid financial instruments that require bifurcation and liability classification as derivative financial instruments. Rather, such terms and features must be combined together and fair valued as a single, compound embedded derivative. The Company selected a binomial lattice model to value the compound embedded derivative because it believes this technique is reflective of all significant assumptions that market participants would likely consider in negotiating the transfer of the Notes. Such assumptions include, among other inputs:

Volatility. The Company estimates stock price volatility based on the Company’s historical stock price performance over a period of time that matches the volume-weighted average expected remaining life of the Notes.

Risk-free interest rate. The risk-free interest rate is based on the U.S. Treasury zero-coupon yield curve in effect at the valuation date commensurate with the expected remaining life assumption.

Expected remaining life. The expected life of the Notes is assumed to be equivalent to their remaining contractual term.

Dividend rate. The dividend rate is based on the historical rate, which the Company anticipates will remain at zero.

Scenarios. The probability of complex features of the compound bifurcated derivative being triggered is subjective (no observable inputs or available market data) and based on internal and external information known to management at the valuation date. Such assumptions include, among other inputs, probabilities related to a change of control and when it might occur as well as probabilities related to a default under the provisions of the Notes and when it might occur.

Changes to the key assumptions or to the scenarios used in the valuation model, including the probability of key events, such as a change of control transaction, and the credit spread could have a material impact to the overall valuation of the compound bifurcated derivative liability. A 5% change to the probability of a change of control event occurring in 2019 and 2020 would impact the derivative fair value by approximately $0.3 million and $0.1 million, respectively. A 5% change in the estimated credit spread would impact the derivative fair value by approximately $0.1 million. The sensitivity examples provided are included for illustrative purposes only and do not reflect the changes in these assumptions used by the Company. Changes in excess of those illustrated may occur in any period.

Table of Contents
Fibrocell Science, Inc.
Notes to Consolidated Financial Statements
Note 6. Convertible Notes (continued)

The estimated fair value of the compound bifurcated derivative is determined using Level 2 and Level 3 inputs. Significant inputs and assumptions used in the binomial lattice model for the derivative liability are as follows:

($ in thousands except per share data)	December 31, 2018	December 31, 2017
Calculated aggregate value	$1,474	$3,136
Closing price per share of common stock	$1.50	$3.20
Contractual remaining term	7 years, 8 months	8 years, 8 months
Contractual interest rate	4.0 %	4.0 %
Volume-weighted average conversion rate	$17.04667	$17.04667
Risk-free interest rate (term structure)	2.44% - 2.69%	1.28% - 2.40%
Dividend yield	—	—
Credit Rating	CC	CC
Credit Spread	31.77 %	36.98 %
Volatility	87.5 %	99.0 %

The foregoing compound bifurcated derivative was recorded at its estimated fair value at the date of issuance, with subsequent changes in estimated fair value recorded in derivative revaluation expense in the Company's Consolidated Statement of Operations. The change in estimated fair value of the Company's derivative liability for the years ended December 31, 2018 and December 31, 2017, resulted in non-cash income (expense) of approximately $1.7 million and $(1.4) million respectively.

Table of Contents
Fibrocell Science, Inc.
Notes to Consolidated Financial Statements

Note 7. Warrants

The Company accounts for common stock warrants as equity instruments, derivative liabilities, or liabilities, depending on the specific terms of the warrant agreement. See Note 3 for further details on accounting policies related to the Company's stock warrants.

In connection with various financing transactions, the Company has issued warrants to purchase the Company's common stock. In July 2018, in connection with a private placement (the July 2018 Private Placement), the Company issued unregistered warrants to purchase 958,152 shares of its common stock. Each common stock purchase warrant has an exercise price of $2.70 per share, was exercisable upon the date of issuance and expires five and one-half years from the date of the issuance. In addition, the Company also issued unregistered warrants to purchase up to an aggregate of 103,186 shares of its common stock to the designees of H.C. Wainwright & Co., LLC (HCW), as partial compensation for placement agent services by HCW in connection with the Company's registered direct public offering in July 2018 (the July 2018 Registered Direct Public Offering), and the July 2018 Private Placement. Such unregistered warrants have an initial exercise price of $3.464 per share are immediately exercisable and expire on July 3, 2023.

In May 2018, in connection with a private placement (the May 2018 Private Placement), the Company issued unregistered warrants to purchase 1,528,668 shares of its common stock. Each common stock purchase warrant has an exercise price of $2.86 per share, was exercisable upon the date of the issuance and expires five and one-half years from the date of the issuance. The Company also issued unregistered warrants to purchase up to an aggregate of 142,676 shares of its common stock to the designees of HCW, as partial compensation for placement agent services by HCW in connection with the Company's registered direct public offering in May 2018 (the May 2018 Registered Direct Public Offering), and the May 2018 Private Placement. Such unregistered warrants have an initial exercise price of $3.679 per share are immediately exercisable and expire on May 30, 2023.

In July 2018, the Company filed a registration statement on Form S-1 (the Resale Registration Statement) registering the resale of shares of the Company's common stock underlying warrants issued in the May 2018 Private Placement and the July 2018 Private Placement. The Resale Registration Statement was declared effective by the SEC on August 8, 2018.

In December 2017, the Company issued (i) pre-funded warrants to purchase an aggregate of 1,184,422 shares of the Company's common stock and (ii) common stock purchase warrants to purchase up to an aggregate of 2,809,404 shares of the Company's common stock including warrants to purchase up to 82,118 shares, issued pursuant to the partial exercise of the underwriters option to purchase additional common stock purchase warrants. Each pre-funded warrant was sold together with a common stock purchase warrant to purchase one share of the Company's common stock at a combined effective price of $3.85 per share and accompanying warrant. Each common stock purchase warrant has an exercise price of $3.85 per share, was exercisable upon the date of issuance and expires five years from the date of issuance. As additional compensation, the Company issued warrants to the underwriter to purchase 87,274 shares of the Company's common stock. Each such warrant has an exercise price of $4.8125 per share, and was exercisable as of the date of the underwriting agreement, and will expire five years after the date of the underwriting agreement, all as more fully described in Note 8.

In March 2017, the Company issued warrants to purchase 687,468 shares of its common stock in connection with the Company's public offering of convertible preferred stock and warrants (each a Series A Warrant and collectively, the Series A Warrants), more fully described in Note 8. Each warrant has an exercise price of $12.69, was exercisable six months after the date of issuance and will expire five years from the date of issuance.

In September 2016, the Company issued warrants to purchase 1,205,840 shares of its common stock for an exercise price of $22.50 per share to investors in connection with a private placement of convertible debt securities as more fully discussed in Note 6. The warrants are exercisable at any time beginning six months after issuance through five years after issuance. The Company classified these warrants as liabilities based on the guidance in ASC 480, as the warrants contain a provision that could result in the Company's redemption of the warrants outside its control for cash equal to the value of the warrants calculated using a Black-Scholes option pricing model.

Table of Contents
Fibrocell Science, Inc.
Notes to Consolidated Financial Statements
Note 7. Warrants (continued)

The following table summarizes the Company's outstanding warrants to purchase common stock as of:

	Number of Warrants			
	December 31, 2018	December 31, 2017	Exercise Price	Expiration Dates
Liability-classified Warrants				
Issued with June 2012 Convertible Notes	—	75,040	$37.50	Jun 2018
Issued in Series E Preferred Stock offering	—	104,676	$112.50	Dec 2018
Issued with September 2016 Convertible Notes	1,205,840	1,205,840	$22.50	Sept 2021
	1,205,840	1,385,556		
Equity-classified Warrants				
Issued in 2017 Series A Preferred Stock Offering	687,468	687,468	$12.69	Mar 2022
Issued in 2017 Common Stock Offering - common warrants	2,679,702	2,809,404	$3.85	Dec 2022
Issued in 2017 Common Stock Offering - underwriter warrants	87,274	87,274	$4.8125	Dec 2022
Issued in 2017 Common Stock Offering - pre-funded warrants	—	483,221	$0.05	No expiration
Issued in May 2018 Private Placement - common warrants	1,528,668	—	$2.86	Nov 2023
Issued in May 2018 Registered Direct Offering - underwriter warrants	142,676	—	$3.679	May 2023
Issued in July 2018 Private Placement - common warrants	958,152	—	$2.70	Jan 2024
Issued in July 2018 Registered Direct Offering - underwriter warrants	103,186	—	$3.464	Jul 2023
	6,187,126	4,067,367		
Total outstanding warrants	7,392,966	5,452,923		

The table below is a summary of the Company's warrant activity for the year ended December 31, 2018.

	Number of warrants			Weighted-average exercise price
	Liability-classified	Equity-classified	Total	
Outstanding at December 31, 2017	1,385,556	4,067,367	5,452,923	$ 11.32
Granted	—	2,732,682	2,732,682	2.87
Exercised	—	(612,923)	(612,923)	0.85
Expired	(179,716)	—	(179,716)	81.18
Outstanding at December 31, 2018	1,205,840	6,187,126	7,392,966	$ 7.36

Accounting for Liability-classified Warrants

The foregoing warrants are recorded as liabilities at their estimated fair value at the date of issuance, with subsequent changes in estimated fair value recorded in warrant revaluation income in the Company's Consolidated Statement of Operations in each subsequent period. The change in estimated fair value of the Company's warrant liability for the years ended December 31, 2018 and 2017 resulted in non-cash income of $0.9 million and $4.9 million, respectively. Additionally, the warrants are classified as either current or non-current on the Company's Consolidated Balance Sheet based on their contractual expiration date. The Company utilizes the Monte Carlo simulation valuation method to value its liability-classified warrants.

F 19

Table of Contents
Fibrocell Science, Inc.
Notes to Consolidated Financial Statements
Note 7. Warrants (continued)

Assumptions Used in Determining Fair Value of Warrants

The estimated fair value of warrants is determined using Level 2 and Level 3 inputs which is further discussed in Note 9. Inherent in the Monte Carlo simulation valuation method are the following assumptions:

Volatility. The Company estimates stock price volatility based on the Company's historical stock price performance over a period of time that matches the volume-weighted average expected remaining life of the warrants.

Risk-free interest rate. The risk-free interest rate is based on the U.S. Treasury zero-coupon yield curve in effect at the valuation date commensurate with the expected remaining life assumption.

Expected remaining life. The expected life of the warrants is assumed to be equivalent to their remaining contractual term.

Dividend rate. The dividend rate is based on the historical rate, which the Company anticipates will remain at zero.

Scenarios. The probability of complex features of the warrants being triggered is subjective (no observable inputs or available market data) and based on internal and external information known to management at the valuation date. Such assumptions include, among other inputs, probabilities related to a change of control and when it might occur as well as probabilities related to a default under the provisions of the Notes and when it might occur.

Changes to the key assumptions or to the scenarios used in the valuation model, including the probability of key events, such as a change of control transaction, could have a material impact to the overall valuation of the warrant liability.

The following table summarizes the calculated aggregate fair values, along with the assumptions utilized in each calculation:

($ in thousands, except per share data)	December 31, 2018	December 31, 2017
Calculated aggregate value	$ 152	$ 1,073
Weighted average exercise price per share	$ 22.50	$ 30.10
Closing price per share of common stock	$ 1.50	$ 3.20
Volatility	94.1 %	92.2 %
Weighted average remaining expected life	2 years, 8 months	3 years, 4 months
Risk-free interest rate	2.45 %	2.00 %
Dividend yield	—	—

Note 8. Equity

Preferred Stock

The Company is authorized to issue 5,000,000 shares of preferred stock, at a par value of $0.001 per share, in one or more series and to fix the rights, preferences, privileges, and restrictions thereof. These rights, preferences and privileges could include dividend rights, conversion rights, voting rights, terms of redemption, liquidation preferences, sinking fund terms and the number of shares constituting any series or the designation of such series, any or all of

which may be greater than the rights of common stock. The issuance of the Company's preferred stock could adversely affect the voting power of holders of common stock and the likelihood that such holders will receive dividend payments and payments upon liquidation. In addition, the issuance of preferred stock could have the effect of delaying, deferring or preventing a change of control of the Company or other corporate action.

Series A Convertible Preferred Stock

In March 2017, the Company's Board of Directors (the Board) authorized the issuance of 8,000 shares of preferred stock designated as Series A Convertible Preferred Stock (the Series A Preferred Stock). The rights, preferences and privileges

of the Series A Preferred Stock is set forth in the Certificate of Designation of Preferences, Rights and Limitations of Series A Convertible Preferred Stock dated March 7, 2017 (Certificate of Designation).

On March 7, 2017, the Company entered into a securities purchase agreement with certain of its existing accredited investors pursuant to which the Company agreed to sell a total of 8,000 units (the Units) for a purchase price of $1,000 per Unit, with each Unit consisting of (i) one share of the Company's Series A Preferred Stock, with an initial stated value of $1,000 and is convertible into 85 shares of the Company's common stock with a conversion price of $11.6355 and (ii) a warrant to purchase up to the number of shares of common stock equal to 100% of the conversion shares issuable on March 7, 2017 pursuant to the shares of Series A Preferred Stock purchased by each investor (collectively, the 2017 Series A Preferred Stock Offering). The 2017 Series A Preferred Stock Offering closed on March 8, 2017 and resulted in gross proceeds of $8.0 million, before deducting offering costs.

The proceeds from the 2017 Series A Preferred Stock Offering (including offering costs) were allocated between the Series A Warrants and Series A Preferred Stock issued in the transaction based upon their respective fair values using the relative fair value (proportional) method. The fair value of the Series A Preferred Stock issued was calculated as the sum of (i) the value of the Series A Preferred Stock as if it had been converted into common stock on the issuance date and (ii) the value of a perpetual annuity paying a 4% dividend rate in conversion shares for five years and 8% thereafter. In connection with the valuation, the following assumptions were used: risk free interest rate of 3.15%, credit spread of 31.27% and a market yield of 34.42%. The application of the relative fair value method resulted in an allocation of gross proceeds to the Series A Preferred Stock of approximately $1.3 million, net of discounts of $3.0 million attributed to the warrants (See Note 7) and $3.7 million from a beneficial conversion feature. The discount attributed to the beneficial conversion feature was immediately amortized as the Series A Preferred Stock has no stated redemption date and is convertible at the issuance date. For the years ended December 31, 2018 and 2017, the Company recognized approximately $0.5 million and $4.1 million, respectively, of amortization of the discount on the Series A Preferred Stock as deemed dividends charged to additional paid-in capital (in the absence of retained earnings). The value of the beneficial conversion feature is calculated as the difference between the effective conversion price of the Series A Preferred Stock and the fair market value of the common stock into which the Series A Preferred Stock are convertible at the commitment date.

The discount attributed to the warrants is being accreted using the effective interest method and charged as a deemed dividend to additional paid-capital (in the absence of retained earnings), over the five-year period of the Series A Preferred Stock in which the stated dividend rate is 4%. For the years ended December 31, 2018 and 2017, the Company recognized approximately $0.5 million and $0.3 million, respectively, in deemed dividends due to the accretion of the warrant discount.

The 2017 Series A Preferred Stock Offering securities purchase agreement contains customary representations, warranties, and agreements by the Company. The securities purchase agreement also contains customary prohibitions on certain Company payments, the incurrence of certain senior and pari passu debt, certain affiliate transactions and the incurrence of certain liens.

Holders of the Series A Preferred Stock are entitled to receive cumulative dividends at a rate per share of 4% per annum (with such dividend rate increasing to 8% per annum on the five-year anniversary of the original issuance of the Series A Preferred Stock), with such dividends compounded quarterly and payable only by way by increasing the stated value of the Series A Preferred Stock in accordance with the terms of the Certificate of Designation. For the years ended December 31, 2018 and 2017, cumulative dividends paid in-kind to holders of the Series A Preferred

Stock were approximately $0.3 million for both periods.

Shares of Series A Preferred Stock generally have no voting rights, except as required by law; provided, however, that without the prior written consent of the holders of at least 70% of the then outstanding shares of Series A Preferred Stock, the Company may not: (i) alter or change adversely the powers, preferences or rights given to the Series A Preferred Stock or alter or amend the Certificate of Designation; (ii) amend the Company's certificate of incorporation or other charter documents in any manner that adversely affects any rights of a holder of the Series A Preferred Stock; (iii) authorize or create any class of stock ranking as to redemption, distribution of assets upon liquidation or dividends senior to, or otherwise pari passu with, the Series A Preferred Stock; (iv) declare or make any dividends other than dividend payments or other distributions payable solely in the Company's common stock; or (v) enter into any agreement with respect to any of the foregoing.

Upon a liquidation, dissolution or winding up of the Company, the holders of the Series A Preferred Stock are entitled to receive out of the Company's assets, whether capital or surplus, an amount equal to such holder's then stated value for each

share of Series A Preferred Stock before any distribution to the holders of the Company's common stock, any class or series of preferred stock and all other common stock equivalents other than those securities which are explicitly senior or pari passu to the Series A Preferred Stock in redemption, distribution of assets upon a liquidation or dividends. If there are insufficient assets to pay in full such amounts, then the available assets will be ratably distributed to the holders of the Series A Preferred Stock in accordance with the respective amounts that would be payable on such shares if all amounts payable thereon were paid in full.

Common Stock

In July 2016, the Company amended its Restated Certificate of Incorporation, as amended, to increase the number of shares of common stock that the Company is authorized to issue from 100,000,000 to 150,000,000.

On May 24, 2018, the Company implemented the 2018 Reverse Stock Split, as authorized at the annual meeting of stockholders on May 23, 2018. The 2018 Reverse Stock Split became effective on May 24, 2018 at 5:00 pm and the Company's common stock began trading on Nasdaq on a post-split basis at the open of business on May 25, 2018. As a result of the 2018 Reverse Stock Split, every five shares of the Company's issued and outstanding common stock were combined into one share of its common stock, except to the extent that the 2018 Reverse Stock Split resulted in any of the Company's stockholders owning a fractional share, which was rounded up to the next highest whole share. In connection with the 2018 Reverse Stock Split, there was no change in the nominal par value per share of $0.001. The 2018 Reverse Stock Split was effectuated in order to increase the per share trading price of the Company's common stock to satisfy the $1.00 minimum bid price requirement for continued listing on The Nasdaq Capital Market. By letter dated. June 11, 2018, The Nasdaq Capital Market Listing Qualification Department, confirmed that the Company's common stock was in compliance with listing requirements.

On March 10, 2017, the Company implemented the 2017 Reverse Stock Split, as authorized at a special meeting of stockholders on March 1, 2017. The 2017 Reverse Stock Split became effective on March 10, 2017 at 5:00 pm and the Company's common stock began trading on The Nasdaq Capital Market on a post-split basis at the open of business on March 13, 2017. As of a result of the 2017 Reverse Stock Split, every three shares of the Company's issued and outstanding common stock were combined into one share of its common stock, except to the extent that the 2017 Reverse Stock Split resulted in any of the Company's stockholders owning a fractional share, which was rounded up to the next highest whole share. In connection with the 2017 Reverse Stock Split, there was no change in the nominal par value per share of $0.001. The 2017 Reverse Stock Split was effectuated in order to increase the per share trading price of the Company's common stock to satisfy the $1.00 minimum bid price requirement for continued listing on The Nasdaq Capital Market. By letter dated March 27, 2017, The Nasdaq Capital Market Listing Qualification Department, confirmed that the Company's common stock was in compliance with listing requirements.

December 2018 Private Placement

On December 11, 2018, the Company completed the sale of 443,350 shares of its common stock (the December 2018 Private Placement), for approximately $0.9 million. After deducting offering expenses, net proceeds from the December 2018 Private Placement was approximately $0.8 million.

July 2018 Registered Direct Offering and Private Placement

On July 2, 2018, the Company entered into securities purchase agreements (July 2018 Purchase Agreements) with certain institutional and accredited investors for the sale by the Company of 1,474,080 shares of the Company's common stock, par value $0.001 per share at a purchase price of $2.69 per share (the July 2018 Registered Direct Offering). Concurrently with the July 2018 Registered Direct Offering, and pursuant to the July 2018 Purchase Agreements, the Company also sold unregistered warrants exercisable for an aggregate of 958,152 shares of the Company's common stock, which represents 65% of the shares of the Company's common stock sold in the July 2018 Registered Direct Offering, for a purchase price of $0.125 per warrant and with an exercise price of $2.70 per share. Subject to certain ownership limitations, the warrants were exercisable upon issuance. The warrants will expire on the 5.5 years anniversary of the date of issuance.

The July 2018 Registered Direct Offering and the July 2018 Private Placement closed on July 5, 2018. The net proceeds from the transactions were approximately $3.6 million after deducting certain fees due to the placement agent and other estimated transaction expenses. In addition, the placement agent received warrants to purchase 103,186 shares of the Company's common stock. The warrants issued to the placement agent have substantially the same terms as the warrants issued

in the July 2018 Private Placement, except that the exercise price of the warrants issued to the placement agent is $3.464 per share and the term of the warrants issued to the placement agent is five years.

On July 27, 2018, the Company filed a registration statement on Form S-1, registering the resale of shares of the Company's common stock underlying warrants issued in the May 2018 Private Placement and the July 2018 Private Placement. The Resale Registration Statement was declared effective by the SEC on August 8, 2018.

May 2018 Registered Direct Offering and Private Placement

On May 29, 2018, in connection with the May 2018 Registered Direct Offering, the Company entered into securities purchase agreements (May 2018 Purchase Agreements) with certain institutional and accredited investors for the sale by the Company of 2,038,224 shares of the Company's common stock, par value $0.001 per share at a purchase price of $2.85 per share. Concurrently with the May 2018 Registered Direct Offering, and pursuant to the May 2018 Purchase Agreements, the Company in connection with the May 2018 Private Placement, also sold unregistered warrants exercisable for an aggregate of 1,528,668 shares of the Company's common stock, which represents 75% of the shares of the Company's common stock sold in the May 2018 Registered Direct Offering, for a purchase price of $0.125 per warrant and with an exercise price of $2.86 per share. Subject to certain ownership limitations, the warrants were exercisable upon issuance. The warrants will expire on the 5.5 years anniversary of the date of issuance. The May 2018 Purchase Agreements contain representations, warranties and covenants of the investors and the Company that are customary for transactions of this type.

The May 2018 Registered Direct Offering and the May 2018 Private Placement closed on May 31, 2018. The net proceeds from the transactions were approximately $5.3 million after deducting certain fees due to the placement agent and other estimated transaction expenses. In connection with the May 2018 Registered Direct Offering and the May 2018 Private Placement, the placement agent received warrants to purchase up to 7.0% of the aggregate amount of shares of Company common stock sold in the May 2018 Registered Direct Offering. The warrants issued to the placement agent have substantially the same terms as the warrants issued in the May 2018 Private Placement, except that the exercise price of the warrants issued to the placement agent is $3.679 per share and the term of the warrants issued to the placement agent is five years.

December 2017 Public Offering

On December 7, 2017, the Company entered into an underwriting agreement (the Underwriting Agreement) with HCW, relating to the sale of 1,542,832 shares of its common stock, pre-funded warrants to purchase an aggregate of 1,184,422 shares of common stock and common warrants to purchase up to an aggregate of 2,809,404 shares of common stock (the Offering). Each share of common stock or pre-funded warrant, as applicable, was sold together with a common warrant to purchase one share of common stock at a combined effective price to the public of $3.85 per share and accompanying common warrant. At December 31, 2018, all of the pre-funded warrants had been exercised and converted to shares of common stock.

Pursuant to the HCW Underwriting Agreement, the Company granted HCW a thirty day option, which option ended on January 6, 2018, to purchase up to 409,091 additional shares of the Company's common stock at a purchase price of $3.80 per share and/or common warrants to purchase up to an aggregate of 409,091 shares of the Company's common stock at a purchase price of $0.05 per common warrant with an exercise price of $3.85 per share, less the underwriting discounts and commissions. On December 8, 2017, HCW partially exercised this option by purchasing common

warrants to purchase 82,118 shares of common stock. As additional compensation, the Company issued warrants to HCW to purchase 87,274 shares of common stock (the Underwriter Warrants). The Underwriter Warrants have an exercise price of $4.8125 per share, will be exercisable for five years from the date of the HCW Underwriting Agreement and may be exercised on a cashless basis in certain circumstances specified therein.

The Company and HCW completed the Offering on December 11, 2017, resulting in approximately $9.3 million of net proceeds to the Company after deducting the underwriter’s discounts and commissions and other estimated offering expenses payable by the Company.

The common warrants are exercisable immediately at an exercise price of $3.85 per share and will expire five years from the date of issuance. The pre-funded warrants are exercisable immediately at an exercise price of $0.05 per share and may be exercised until they are exercised in full. The Underwriter Warrants have an exercise price of $4.8125 per share and will expire five years from the date of the HCW Underwriting Agreement. The exercise price and number of shares of the Company’s common stock issuable upon exercise of the common warrants, pre-funded warrants and Underwriter Warrants will

Table of Contents
Fibrocell Science, Inc.
Notes to Consolidated Financial Statements
Note 8. Equity (continued)

be subject to adjustment in the event of any stock split, reverse stock split, stock dividend, recapitalization, reorganization or similar transaction, among other events as described in the common warrants and pre-funded warrants.

In the event of certain transactions involving a sale of the Company, each holder of common warrants has the right, exercisable at its option, to require the Company to purchase such holder's common warrants at a price determined using a Black-Scholes option pricing model as described in the common warrants. The shares of common stock or pre-funded warrants, as applicable, and the accompanying common warrants could only be purchased together in this Offering but were issued separately.

Table of Contents
Fibrocell Science, Inc.
Notes to Consolidated Financial Statements

Note 9. Fair Value Measurements

Assets and Liabilities Measured at Fair Value on a Recurring Basis

The Company follows the guidance in ASC 820, Fair Value Measurement, to account for financial assets and liabilities measured on a recurring basis. Fair value is the price that would be received to sell an asset or paid to transfer a liability in an orderly transaction between market participants at the measurement date. As such, fair value is a market-based measurement that should be determined based on assumptions that market participants would use in pricing an asset or liability. The Company uses a fair value hierarchy, which distinguishes between assumptions based on market data (observable inputs) and an entity's own assumptions (unobservable inputs). The guidance requires fair value measurements be classified and disclosed in one of the following three categories:

- Level 1: Unadjusted quoted prices in active markets that are accessible at the measurement date for identical, unrestricted assets or liabilities;
- Level 2: Quoted prices in markets that are not active or inputs which are observable, either directly or indirectly, for substantially the full term of the asset or liability;
- Level 3: Prices or valuation techniques that require inputs that are both significant to the fair value measurement and unobservable (i.e., supported by little or no market activity).

Determining which category an asset or liability falls within the hierarchy requires significant judgment. The Company evaluates its hierarchy disclosures each reporting period. There were no transfers between Level 1, 2 and 3 during
each of the years ended December 31, 2018 and 2017.

The following fair value hierarchy table presents information about each major category of the Company's financial assets and liabilities measured at fair value on a recurring basis as of December 31, 2018 and 2017:

	December 31, 2018			
($ in thousands)	Level 1	Level 2	Level 3	Total
Assets:				
Cash equivalents	$12,290	$ —	$ —	$12,290
Total Assets	$12,290	$ —	$ —	$12,290
Liabilities:				
Warrant liability	$—	$ —	$152	$152
Derivative liability	—	—	1,474	1,474
Total Liabilities	$—	$ —	$1,626	$1,626

	December 31, 2017			
($ in thousands)	Level 1	Level 2	Level 3	Total
Assets:				
Cash equivalents	$14,670	$ —	$ —	$14,670
Total Assets	$14,670	$ —	$ —	$14,670
Liabilities:				
Warrant liability	$—	$ —	$1,073	$1,073
Derivative liability	—	—	3,136	3,136
Total Liabilities	$—	$ —	$4,209	$4,209

F 25

Table of Contents
Fibrocell Science, Inc.
Notes to Consolidated Financial Statements
Note 9. Fair Value Measurements (continued)

Changes in Level 3 Liabilities Measured at Fair Value on a Recurring Basis

Common Stock Warrants - Warrant Liability

The reconciliation of the Company's warrant liability measured at fair value on a recurring basis using unobservable inputs (Level 3) is as follows:

($ in thousands)	Warrant Liability
Balance at December 31, 2016	$ 6,034
Exercise of warrants (1)	(41)
Change in fair value of warrant liability (2)	(4,920)
Balance at December 31, 2017	$ 1,073
Change in fair value of warrant liability (2)	(921)
Balance at December 31, 2018	$ 152

(1) Warrants were exercised under the cashless exercise method pursuant to the corresponding warrant agreements. As a result of such exercises, the Company issued 6,941 shares of common stock. Consequently, these instruments were no longer classified as liabilities. These common stock warrants were remeasured to their fair value as of the exercise date with the change in fair value recorded to the Company's Consolidated Statement of Operations. The fair value related to the shares issued in connection with the exercised warrants was reclassified from a liability to additional paid-in capital in the Company's Consolidated Balance Sheets.

(2) Includes the fair value as of the beginning of the year for warrants expiring during the year and has been recorded to warrant revaluation income in the Company's Consolidated Statement of Operations for the respective year end.

The fair value of the warrant liability is based on Level 3 inputs. For this liability, the Company developed its own assumptions that do not have observable inputs or available market data to support the fair value. See Note 7 for further discussion of the warrant liability.

Bifurcated Compound Derivative - Derivative Liability

The reconciliation of the derivative liability measured at fair value on a recurring basis using unobservable inputs (Level 3) was as follows:

($ in thousands)	Derivative Liability
Balance at December 31, 2016	$ 1,735
Derivative liability to equity upon note conversion (1)	(6)
Change in fair value of derivative liability	1,407
Balance at December 31, 2017	$ 3,136
Change in fair value of derivative liability	(1,662)
Balance at December 31, 2018	$ 1,474

(1) Convertible notes from the September 2016 Private Placement, were converted to shares of common stock pursuant to the corresponding convertible note agreements. As a result of such conversions, the Company issued 24,911 shares of common stock. Consequently, these instruments were no longer classified as liabilities. These embedded derivatives were remeasured to their fair value as of the exercise date with the change in fair value

recorded to the Company's Consolidated Statement of Operations. The fair value related to the shares issued in connection with the converted notes was reclassified from a liability to additional paid-in capital in the Company's Consolidated Balance Sheets.

Effect of the Company's Stock Price and Volatility Assumptions on the Calculation of Fair Value of Financial Instruments Measured on a Recurring Basis

Common Stock Warrants - Warrant Liability

The fair value of the Company's warrant liability is based on Level 3 inputs. As discussed in Note 7, the Company uses a Monte Carlo simulation valuation method to value its liability-classified warrants. The determination of fair value as of the reporting date is affected by the Company's stock price as well as assumptions regarding a number of subjective variables that do not have observable inputs or available market data to support the fair value. These variables include, but are not limited to, expected stock price volatility over the term of the warrants and the risk-free interest rate. The primary factors affecting the fair value of the warrant liability are the Company's stock price and volatility as well as certain assumptions by the Company as to the likelihood of provisions to the underlying warrant agreements being triggered. The methods described above and in Note 7 may produce a fair value calculation that may not be indicative of net realizable value or reflective of future fair values. Furthermore, while the Company believes its valuation method is appropriate and consistent with other market participants, the use of different methodologies or assumptions to determine the fair value could result in a different fair value measurement at the reporting date.

Bifurcated Compound Derivative - Derivative Liability

The fair value of the derivative liability is based on Level 3 inputs. As discussed in Note 6, the Company uses a binomial lattice model to value the compound embedded derivative bifurcated from the Notes. The determination of fair value as of the reporting date is affected by the Company's stock price as well as assumptions regarding a number of subjective variables that do not have observable inputs or available market data to support the fair value. These variables include, but are not limited to, expected stock price volatility, changes in interest rates, assumptions regarding the adjusted conversion prices in the Notes, and early redemption or conversion of the Notes. The methods described above and in Note 6 may produce a fair value calculation that may not be indicative of net realizable value or reflective of future fair values. Furthermore, while the Company believes its valuation method is appropriate and consistent with other market participants, the use of different methodologies or assumptions to determine the fair value could result in a different fair value measurement at the reporting date.

Fair Value of Certain Financial Assets and Liabilities

The Company believes that the fair values of its current assets and liabilities approximate their reported carrying amounts. The fair value of the long-term convertible promissory notes was approximately $12.5 million at December 31, 2018, and $11.2 million at December 31, 2017, compared to a carrying value of $0, as a result of unamortized debt discounts, for both periods.

Table of Contents
Fibrocell Science, Inc.
Notes to Consolidated Financial Statements

Note 10. Stock-Based Compensation

2009 Equity Incentive Plan

The Board adopted the 2009 Equity Incentive Plan (as amended to date, the Plan) effective September 3, 2009. The Plan is intended to further align the interests of the Company and its stockholders with its employees, including its officers, non-employee directors, consultants and advisers by providing equity-based incentives. The Plan allows for the issuance of up to 506,667 shares of the Company's common stock.

The types of awards that may be granted under the Plan include options (both non-qualified stock options and incentive stock options), stock appreciation rights, stock awards, stock units, and other stock-based awards. The term of each award is determined by the Compensation Committee of the Board at the time each award is granted, provided that the term of the option does not exceed ten years. Vesting schedules for stock options vary, but generally vest 25% per year, over four years for employee options and on the one-year anniversary date for non-employee director options. The Plan had 215,670 options available for grant as of December 31, 2018.

Accounting for Stock-Based Compensation

The Company recognizes non-cash compensation expense for stock-based awards based on their grant date fair value, determined using the Black-Scholes option-pricing model. During the years ended December 31, 2018 and 2017, the weighted average fair market value of options granted was $2.22 and $10.58, respectively.

Total stock-based compensation expense recognized using the straight-line attribution method and included in operating expenses in the Company's Consolidated Statements of Operations was approximately $0.5 million and $0.3 million for the years ended December 31, 2018 and 2017, respectively.

Assumptions Used in Determining Fair Value of Stock Options

Inherent in the Black-Scholes option-pricing model are the following assumptions:

Volatility. The Company estimates stock price volatility based on the Company's historical stock price performance over a period of time that matches the expected term of the stock options.

Risk-free interest rate. The risk-free interest rate is based on the U.S. Treasury yield curve in effect at the time of grant commensurate with the expected term assumption.

Expected term. The expected term of stock options granted is based on an estimate of when options will be exercised in the future. The Company applied the simplified method of estimating the expected term of the options, described in the SEC's Staff Accounting Bulletins 107 and 110. The expected term, calculated under the simplified method, is applied to groups of stock options that have similar contractual terms. Using this method, the expected term is determined using the average of the vesting period and the contractual life of the stock options granted.

Dividend rate. The dividend rate is based on the historical rate, which the Company anticipates will remain at zero.

The fair market value of the stock options at the date of grant was estimated using the Black-Scholes option-pricing model with the following weighted average assumptions for the years ended December 31:

	2018	2017

Expected life (1)	6 years		5 years, 11 months	
Interest rate	2.6	%	1.9	%
Dividend yield	—		—	
Volatility	88.0	%	88.7	%

(1) The Company uses the simplified method for estimating the stock option term.

Table of Contents
Fibrocell Science, Inc.
Notes to Consolidated Financial Statements
Note 10. Stock-Based Compensation (continued)

Stock Option Activity

The following table summarizes stock option activity for the years ended December 31, 2018 and 2017:

($ in thousands, except share and per share data)	Number of shares	Weighted-average exercise price	Weighted-average remaining contractual term (in years)	Aggregate intrinsic value
Outstanding at December 31, 2016	255,906	$ 75.84	7 years, 2 months	$ —
Granted	59,000	14.41		
Expired	(42,360)	106.73		
Forfeited	(54,620)	47.51		
Outstanding at December 31, 2017	217,926	$ 60.31	7 years, 3 months	$ —
Granted	84,800	2.99		
Expired	(3,825)	34.07		
Forfeited	(12,189)	6.26		
Outstanding at December 31, 2018 (1)	286,712	$ 46.01	7 years	$ —
Exercisable at December 31, 2018	166,871	$ 73.25	5 years, 9 months	$ —

(1) Includes both vested stock options as well as unvested stock options for which the requisite Company service period has not been rendered but that are expected to vest based on achievement of reaching the Company service period requirement.

The total fair value of options vested during the years ended December 31, 2018 and 2017 was approximately $0.5 million for each year, based upon the fair value of the options at grant date. Additionally, as of December 31, 2018, there was approximately $0.5 million of unrecognized compensation expense related to non-vested stock options which is expected to be recognized over a weighted-average period of 1.6 years.

During the years ended December 31, 2018 and December 31, 2017, there were no exercises of vested stock options.

Table of Contents

Note 11. Income Taxes

Fibrocell Science, Inc. and Fibrocell Technologies, Inc. file a consolidated U.S. federal income tax return, and file U.S. state income tax returns in several jurisdictions as well. In general, the U.S. federal and state income tax returns remain open to examination by taxing authorities for tax years beginning in 2015 to present. However, if and when the Company claims net operating loss (NOL) carryforwards from years prior to 2015 against future taxable income, those losses may be examined by the taxing authorities. The Company's foreign subsidiaries file income tax returns in their respective jurisdictions.

The components of the income tax expense (benefit) related to operations, were as follows:

	Year ended December 31,	
($ in thousands)	2018	2017
U.S. Federal:		
Current	$ —	$ —
Deferred	—	—
U.S. State:		
Current	—	—
Deferred	—	—
Income tax expense (benefit)	$ —	$ —

The reconciliation between income tax expense (benefit) at the U.S. federal statutory rate and the amount recorded in the accompanying Consolidated Financial Statements were as follows:

	Year ended December 31,	
($ in thousands)	2018	2017
Tax benefit at U.S. federal statutory rate	$(2,158)	$(5,684)
Increase in domestic valuation allowance	4,815	5,914
State income taxes benefit before valuation allowance, net of federal benefit	(897)	6
State rate change	(2,514)	555
State law change	1,418	—
Warrant revaluation income and other financing costs	(366)	(898)
Credits	(478)	(904)
Stock-based compensation	49	61
Return to provision true-ups	75	127
Capital loss carryforward expiration	—	817
Impact of federal rate change	—	34,463
Impact of federal rate change on valuation allowance	—	(34,463)
Other	56	6
Income tax expense (benefit)	$—	$—

Table of Contents
Fibrocell Science, Inc.
Notes to Consolidated Financial Statements
Note 11. Income Taxes (continued)

The components of the Company's net deferred tax assets and liabilities at December 31, 2018 and 2017 were as follows:

	Year ended December 31,	
($ in thousands)	2018	2017
Deferred tax liabilities:		
Convertible notes	$2,842	$2,821
Total deferred tax liabilities	$2,842	$2,821
Deferred tax assets:		
Loss carryforwards (federal and state)	$64,285	$60,428
Intangible assets	57	68
Property and equipment	839	606
License fees	4,645	4,419
Accrued expenses and other	354	401
Stock-based compensation	2,838	2,753
Research and development tax credits	1,945	1,436
Total deferred tax assets before valuation allowance	74,963	70,111
Less: valuation allowance	(72,121)	(67,290)
Total deferred tax assets	$2,842	$2,821
Net deferred tax assets	$—	$—

As of December 31, 2018, the Company had generated U.S. NOL carryforwards of approximately $251 million of which $237.5 million will expire from 2019 to 2037 and $13.5 million can be carried forward indefinitely. As of December 31, 2018, the Company had generated federal R&D credits of $1.9 million which expire from 2033 to 2038. The NOL carryforwards are available to reduce future taxable income. However, the NOL carryforwards may be, or become subject to, an annual limitation in the event of certain cumulative changes in the ownership interest of significant stockholders over a three-year period in excess of 50%, as defined under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, as well as similar state tax provisions. This could limit the amount of NOL's that the Company can utilize annually to offset future taxable income or tax liabilities. The amount of the annual limitation, if any, will be determined based on the value of the Company immediately prior to an ownership change. Subsequent ownership changes may further affect the limitation in future years. If and when the Company utilizes the NOL carryforwards in a future period, it will perform an analysis to determine the effect, if any, of these loss limitation rules on the NOL carryforward balances. Additionally, U.S. tax laws limit the time during which these carryforwards may be applied against future taxes, therefore, the Company may not be able to take full advantage of these carryforwards for federal income tax purposes. In addition, the Company has NOL carryforwards in certain non-U.S. jurisdictions of approximately $0.3 million. However, it is not expected that these non-U.S. loss carryforwards will ever be utilized, so they are not included in the components of deferred taxes listed above. Finally, there are no unremitted earnings in foreign jurisdictions, so no provision for taxes thereupon is required.

As the Company has had cumulative losses and there is no assurance of future taxable income, valuation allowances have been recorded to fully offset deferred tax assets at December 31, 2018 and 2017. The valuation allowance increased by $4.8 million in 2018 which was due to the current year taxable loss. The valuation allowance decreased by $28.5 million in 2017, which was partly due to a $34.4 million decrease due to the reduction of the federal corporate tax rate and partly due to a $5.9 million increase primarily due to the impact of the net losses incurred in 2017.

As of December 22, 2017, the United States enacted tax reform legislation “known as H.R. 1”, commonly referred to as the “Tax Cuts and Jobs Act” (TCJA or the “Act”), resulting in significant modifications to existing law. Among other changes, the TCJA permanently lowers the corporate federal income tax rate to 21% from the existing maximum rate of 35% effective for tax years beginning after December 31, 2017. As a result of the reduction of the corporate federal income tax rate to 21%, U.S. GAAP required companies to revalue their deferred tax assets and deferred tax liabilities as of the date of enactment, with the resulting tax effects accounted for in the reporting period of enactment. As a result of this revaluation, the Company incurred $34.5 million income tax expense in continuing operations with a corresponding reduction in the valuation allowance. Therefore, there was no impact on the Company’s consolidated statements of operations or statements of financial

position from the reduction in the federal tax rate. The other provisions of the TCJA did not have a material impact on the consolidated financial statements.

In response to the enactment of the TCJA, the SEC staff issued Staff Accounting Bulletin No. 118 ("SAB 118"), which provided guidance on accounting for the tax effects of the Act. SAB 118 provided a measurement period that should not extend beyond one year from the Act enactment date for companies to complete the accounting under ASC 740. In accordance with SAB 118, the Company has finalized the accounting for the TCJA as of December 31, 2018 and the final amount recorded in these financial statements for the effect of the corporate tax rate change did not change from the provisional estimate recorded at December 31, 2017.

Note 12. Related Party Transactions

Overview of Related Parties

The Company and Intrexon Corporation (Intrexon) are parties to two distinct exclusive channel collaboration agreements, as more fully described below. Pursuant to these agreements, the Company engages Intrexon for support services for the research and development of product candidates covered under the respective agreements and reimburses Intrexon for its cost for time and materials for such work. Additionally, the Company's future commitments pursuant to the agreements include cash royalties and various developmental and commercial milestone payments as more fully described below.

For the years ended December 31, 2018 and 2017, the Company incurred expenses of approximately $0.5 million and $5.2 million, respectively, for goods and services received from Intrexon. Of the expenses incurred during the 2018 period, $0.1 million related to direct expenses for work performed by Intrexon and $0.4 million related to pass-through costs for work performed under the 2012 ECC. These December 31, 2018 and December 31, 2017 expenses do not include approximately $0.5 million in costs we estimated to settle a dispute with one of Intrexon's vendors in 2017, and for which there was a corresponding reduction in 2018. Of the expenses incurred during the 2017 period, $1.3 million related to direct expenses for work performed by Intrexon and $3.9 million related to pass-through costs, both for work performed under the 2012 ECC.

As of December 31, 2018 and 2017, the Company had outstanding payables with Intrexon of $0.1 million and $2.3 million, respectively. In connection with the 2015 ECC, in consideration for the license and the other rights that the Company receives under the agreement, the Company paid Intrexon an up-front technology access fee of $10 million in cash in January 2016.

In the second quarter of 2017, Intrexon notified the Company that it had received invoices for approximately $1.1 million in charges from a vendor who provided services to Intrexon and which are passed-through to the Company under the 2012 ECC. Additional charges were presented after the second quarter of 2017, and the total of disputed charges at March 31, 2018, was approximately $1.4 million. The Company, Intrexon and Intrexon's vendor have resolved the dispute with the parties agreeing to settle all obligations for approximately $0.2 million. This is a reduction of approximately $0.5 million from the approximately $0.7 million recorded at December 31, 2017 for this liability and was recorded in the three months ended March 31, 2018. The approximately $0.2 million settlement amount was paid in August 2018.

Randal J. Kirk is the chairman of the board and chief executive officer of Intrexon and, together with his affiliates, owns more than 50% of Intrexon's common stock. Affiliates of Randal J. Kirk (including Intrexon) own approximately 17% of the Company's common stock. Additionally, two of the Company's directors, Julian Kirk (who is the son of Randal J. Kirk) and Marcus Smith, are employees of Third Security, LLC, which is an affiliate of Randal J. Kirk.

Affiliates of Randal J. Kirk (including Intrexon) participated in the Company's private placement of convertible debt securities in September 2016, more fully described in Note 6, and were issued an aggregate of $6,762,500 in principal of Notes and accompanying Warrants to purchase an aggregate of 450,835 shares of common stock. Affiliates of Randal J. Kirk (including Intrexon) participated in the Company's March 2017 Series A Convertible Preferred Stock offering, more fully described in Note 8, and were issued an aggregate of 3,016 shares of convertible preferred stock and accompanying warrants to purchase 259,176 shares of common stock. Additionally, affiliates of Randal J. Kirk (including Intrexon) participated in the Company's December 2017 public offering, and were issued an aggregate of 545,456 shares of common stock and accompanying warrants to purchase 545,456 shares of common stock.

Table of Contents
Fibrocell Science, Inc.
Notes to Consolidated Financial Statements
Note 12. Related Party Transactions (continued)

Intrexon Collaboration - 2012 ECC

In October 2012, the Company entered into an Exclusive Channel Collaboration Agreement with Intrexon which was amended in June 2013 and January 2014 (as amended, the 2012 ECC) pursuant to which the Company is Intrexon's exclusive channel collaborator in the research, development and commercialization of products in the following fields (the 2012 Fields):

- the enhanced production and purification of autologous fibroblasts, without gene therapy, for all aesthetic and therapeutic indications;
- the enhanced production and purification of autologous dermal cells, without gene therapy, for aesthetic and therapeutic treatment of dermal, vocal cord, and periodontal indications;
- the development of our gene therapies applied to autologous fibroblasts for all aesthetic and therapeutic indications;
- the development of our gene therapies applied to autologous dermal cells for aesthetic and therapeutic treatment of dermal, vocal cord, and periodontal indications;
- autologous human fibroblasts with gene therapy to express a therapeutic protein and/or bioactive ribonucleic acid for the treatment of autoimmune and non-infectious inflammatory disorders that manifest in cutaneous tissues, fascia and/or muscle; and
- autologous human fibroblasts with gene therapy to express bioactive Tenascin-X locally to correct connective tissue disorders associated with Ehlers-Danlos Syndrome (hypermobility type).

Pursuant to the terms of the 2012 ECC, Intrexon has granted the Company a license to use its proprietary technologies and other intellectual property to research, develop and commercialize products in the 2012 Fields within the United States.

The Company is responsible for all costs incurred in connection with the research, development and commercialization of products under the 2012 ECC and will own all clinical data, regulatory filings and regulatory approvals relating to such products. The Company engages Intrexon for support services for the research and development of products under the 2012 ECC and reimburses Intrexon for its cost for time and materials for such services.

In September 2015, the Company and Intrexon entered into a letter of agreement pursuant to which the parties mutually agreed to terminate their collaboration with respect to the development of potential therapies to treat Ehlers-Danlos Syndrome (hypermobility type) due to technical hurdles. As a result, the Company no longer has any rights or obligations under the 2012 ECC with respect to the development of "autologous human fibroblasts genetically modified to express bioactive Tenascin-X locally to correct connective tissue disorders".

The Company is required to pay Intrexon quarterly cash royalties on all products developed under the 2012 ECC in an amount equal to 7% on aggregate quarterly net sales up to $25 million, plus 14% on aggregate quarterly net sales greater than $25 million. The Company is also required to pay Intrexon half of any sublicensing revenues that it receives from third parties in consideration for sublicenses granted by the Company with respect to products developed under the 2012 ECC, but only to the extent such sublicensing revenues are not included in net sales subject to royalties. Sales from LAVIV (azficel-T), including new indications, or other products that the Company develops and commercializes outside of the 2012 ECC are not subject to royalty payments unless the Company is able to reduce the product's cost of goods sold through the 2012 ECC, in which case, the Company is required to pay quarterly cash royalties on such products equal to one third of such cost of goods sold savings. No royalties have been paid to date in connection with the 2012 ECC.

Intrexon Collaboration - 2015 ECC

In December 2015, the Company entered into an additional Exclusive Channel Collaboration Agreement with Intrexon (the 2015 ECC) pursuant to which the Company is Intrexon's exclusive channel collaborator in the research, development and commercialization of products for the treatment of chronic inflammatory and degenerative diseases of human joints through intra-articular or other local administration of genetically modified fibroblasts (the 2015 Field).

Pursuant to the terms of the 2015 ECC, Intrexon has granted the Company a license to use its proprietary technologies and other intellectual property to develop and commercialize collaboration products in the 2015 Field throughout the world. The Company is responsible for all costs incurred in connection with the development and commercialization of collaboration

products and will own all clinical data, regulatory filings and regulatory approvals relating to such products. The Company engages Intrexon for support services in connection with the research and development of products under the 2015 ECC and reimburses Intrexon for its cost for time and materials for such services.

In consideration for the license and the other rights that the Company receives under the 2015 ECC, the Company paid Intrexon an up-front technology access fee of $10 million in cash in January 2016. For each collaboration product the Company develops under the 2015 ECC, the Company is required to pay Intrexon development milestones of up to $30 million and commercialization milestones of up to $22.5 million, a low double-digit royalty on its net sales of such products and half of any sublicensing revenues received from third parties for such products. No royalties or milestone payments have been paid to date in connection with the 2015 ECC.

Table of Contents
Fibrocell Science, Inc.
Notes to Consolidated Financial Statements

Note 13. Loss Per Share

Details in the computation of basic and diluted loss per share were as follows:

	For the Year Ended December 31,	
($ in thousands except share and per share data)	2018	2017
Loss per share — Basic:		
Net loss	$(10,277)	$(16,240)
Less: Dividend paid in-kind to preferred stockholders	(336)	(264)
Less: Deemed dividend on preferred stock	(513)	(4,099)
Net loss attributable to common stockholders - basic	$(11,126)	$(20,603)
Numerator for basic loss per share	$(11,126)	$(20,603)
Denominator for basic loss per share	7,693,191	3,092,543
Basic loss per common share	$(1.45)	$(6.66)
Loss per share — Diluted:		
Numerator for basic loss per share	$(11,126)	$(20,603)
Adjust: Warrant revaluation income for dilutive warrants	—	(34)
Numerator for diluted loss per share	$(11,126)	$(20,637)
Denominator for basic loss per share	7,693,191	3,092,543
Plus: Incremental shares underlying “in the money” warrants outstanding	—	1,184
Denominator for diluted loss per share	7,693,191	3,093,727
Diluted loss per common share	$(1.45)	$(6.67)

The following potentially dilutive securities have been excluded from the computations of diluted weighted-average shares outstanding, as their effect would be anti-dilutive:

	For the Year Ended December 31,	
	2018	2017
“In the money” stock options	19,233	46,869
“Out of the money” stock options	247,897	171,057
“In the money” warrants	—	—
“Out of the money” warrants	7,392,966	4,969,702
Shares underlying convertible notes	1,056,068	1,056,068
Shares underlying accrued interest on convertible notes	101,937	56,752
Shares underlying convertible preferred stock	736,000	704,000

Note 14. Commitments and Contingencies

Leases

On April 6, 2005, the Company entered into a non-cancellable operating lease (the Lease) for its office, warehouse and laboratory facilities in Exton, Pennsylvania. The lease agreement had an original term of 8 years. On February 17, 2012, the Company entered into an amended and restated lease (the Amended Lease) for an additional term of 10 years through the year 2023. The Lease and the Amended Lease provide for rent payments escalating on a periodic basis. In accordance with ASC 840-20, Operating Leases, the Company accounts for total minimum

payments under the lease on a straight-line basis over the life of the lease. The difference between actual rent payments and payments accounted for using the straight-line basis are reflected as deferred rent on the Company's Consolidated Balance Sheets. The Company has the option to renew the lease for an additional 5 years at fair market value. Rental expense totaled approximately $1.6 million for both the years ended December 31, 2018 and 2017.

Table of Contents
Fibrocell Science, Inc.
Notes to Consolidated Financial Statements
Note 14. Commitments and Contingencies (continued)

Collaboration with Related Party (Intrexon)

The Company is a party to two separate exclusive channel collaboration agreements with Intrexon, a related party. Pursuant to the agreements, the Company is Intrexon's exclusive channel collaborator in the research, development and commercialization of products in certain defined fields. The Company is required to pay future royalties, as well as development and commercialization milestones, under these agreements. See Note 12 for additional details.

Contractual Obligations

The following table summarizes the Company's minimum contractual obligations as of December 31, 2018:

	Payments due by period						
($ in thousands)	Total	2019	2020	2021	2022	2023	2024 and thereafter
Operating lease obligations (1)	6,197	1,416	1,471	1,471	1,471	368	—
Debt obligations (2)	21,968	—	—	21,968	—	—	—
Total [3]	$28,165	$1,416	$1,471	$23,439	$1,471	$368	$ —

(1) Operating lease obligations are stated based on the Amended Lease agreement for the office, warehouse and laboratory facilities executed in February 2012.

(2) Obligations under the Notes issued in connection with the 2016 Private Placement which includes principal and accrued interest through September 7, 2021, based on stated fixed rates, as the Company has elected to accrue interest. The Notes have a maturity date of the earlier of (i) September 7, 2026 and (ii) one-hundred and eighty (180) days after the date on which the Company's product candidate, FCX-007, is approved by the FDA for the treatment of RDEB. However, each Note holder has the right to require the Company to repay all or any portion of the unpaid principal and accrued interest from time to time on or after September 7, 2021. See details within Note 6.

(3) This table does not include (a) any milestone payments which may become payable to third parties under license agreements as the timing and likelihood of such payments are not known, (b) any royalty payments to third parties as the amounts of such payments, timing and/or the likelihood of such payments are not known, and (c) contracts that are entered into in the ordinary course of business which are not material in the aggregate in any period presented above.