assembly
biosciences

2017

ANNUAL REPORT

DEAR FELLOW SHAREHOLDERS

I BEGIN THIS LETTER WITH A GREAT APPRECIATION FOR WHAT WE HAVE ACCOMPLISHED THIS PAST YEAR AND TO DATE AT ASSEMBLY BIOSCIENCES (ASMB). I FEEL HONORED TO LEAD SUCH A HIGHLY EXPERIENCED AND DEDICATED TEAM. TOGETHER WE ARE COMMITTED TO BRINGING INNOVATIVE CURATIVE THERAPIES TO PATIENTS WITH SERIOUS DISEASES THAT ARE UNDERSERVED BY CURRENT TREATMENTS.

I am grateful to these patients and clinicians who have taken part in our clinical trials, enabling us to report our first clinical efficacy and safety data over the past year and to our shareholders whose support has helped make all of this possible.

As I look back to the beginning of 2017—in fact, to our merger and founding in 2014—it is remarkable how much we have accomplished to advance our medicines and novel science and move closer to achieving our goals of advancing cures for patients. While we are proud of our progress, we are even more excited by what is ahead of us. We expect the next year to mark a major inflection point in our growth, particularly as we advance the studies we hope will provide definitive proof of concept that ABI-H0731 ('731) has the potential to bring cures to more of the 250 million people worldwide chronically infected with hepatitis B (HBV).

ADVANCING OUR STRATEGY

Our primary objective at Assembly Biosciences is to deliver truly differentiated medicines developed through innovative cutting-edge science, with a corporate dedication to giving patients a better quality of life. Our strategy is to advance our portfolio of potent, chemically-distinct Core protein Allosteric Modifiers (CpAMs) with the aim of bringing cures to more hepatitis B patients and to harness the power of the microbiome to create targeted, efficacious synthetic biotherapeutics by applying a differentiated, fully-integrated development approach.

The promise of Assembly Biosciences lies in our unique therapeutic development platforms and world class scientific leadership. Everything we do has a purpose—seeking the answers that will drive our programs forward. We are deliberate with our pipeline strategy, identifying opportunities that offer the most impact to patients within our focus areas, which we believe then offer the greatest return on investment. We are also stewards of our resources—human and financial—and we endeavor to apply them wisely. We design our clinical trials to answer key questions in what we believe to be a safe and efficient design, with each study building on the last. In just 18 months, we went from initiating our first human trial of '731, our lead CpAM, to presenting positive data in HBV patients, with Phase 2a proof-of-concept studies on track to initiate this summer.

INVESTING IN OUR BUSINESS

We take this seriously—it's our inherent duty to manage our business effectively and our responsibility to invest for the future growth of the company. Our mindset is that by strategically building the business for long term success, we are building overall value for the company and its shareholders.

Our investments now will help us reach our goals more efficiently and cost-effectively in the future. To that end, we've made a number of key decisions over the past year that further strengthen the company and position us for continued growth. We now have our own research and development laboratories in our San Francisco location as well as in Groton, Connecticut—providing us greater capabilities and flexibility for pipeline development than in the past.

Also, as a talent-led organization, attracting, developing and inspiring the very best people in our industry is critical to our success. We are proud to have great people and great leaders, and we've been fortunate to expand our leadership team in recent months.

These accomplished individuals bring critical skills and experience that will help enable our continued growth and pipeline advancement:

- Graham Cooper, Chief Financial Officer, Chief Operating Officer
- Jackie Papkoff, PhD, Chief Scientific Officer of Microbiome
- Sue Mahony, PhD, Board Director
- Helen S. Kim, Board Director

Additionally, in planning for the development of '731 into Phase 2 studies, our second generation CpAM ABI-H2158 or '2158, into Phase 1, and the advancement of our microbiome program during 2018, we raised net proceeds of approximately $64.8 million in an underwritten offering of common stock in November 2017. We greatly appreciate the support of our new and existing shareholders, and we expect that our current cash position will allow us to reach a number of important inflection points over the next 12 months.

KEY ACCOMPLISHMENTS OF THE PAST YEAR

We are pleased to report the following key accomplishments over the past year.

OPERATIONS & LEADERSHIP – *continuing to build on our great team and programs*

- We augmented the research and development teams for our two innovative platform programs, our HBV - cure program and our Microbiome program and improved operational and administrative functions

- Made key appointments to the management team and the Board of Directors as noted above

HBV-CURE PROGRAM – *we believe we have the deepest CpAM portfolio in the HBV—cure field*

- We completed "first in human" Phase 1a safety studies for '731 in healthy volunteers and presented data at the American Association for the Study of Liver Diseases (AASLD) Annual Meeting
- Initiated a Phase 1b trial in HBV patients in June 2017 for '731 and presented positive interim data at the Annual Meeting of the European Association for the Study of the Liver (EASL) in April 2018
  - The safety and antiviral profile suggest '731 has the potential to be a best-in-class of CpAMs in development
- Presented poster at AASLD in October 2017 on our studies evaluating the half-life or turnover of cccDNA, which is thought to be a mechanism fueling chronic HBV infection and may be inhibited with the addition of CpAM therapy
  - We believe this was an important contribution to the field, as we showed genetic-based evidence that the turnover of cccDNA is measurable in weeks (not years)
- Selected a second generation CpAM, '2158, as the next development candidate in the HBV pipeline
  - Began IND-enabling studies
  - Presented preclinical data at AASLD
- Advanced a number of third-generation molecules toward possible candidate selection in our HBV program
- Established our first office and lab for our Assembly China business in Shanghai, China—a key region for development of Hepatitis B medicines (China has ~90 million HBV patients)

MICROBIOME PROGRAM—*advancing both our partnered and wholly owned programs from this innovative platform*

- We established a collaboration with Allergan Pharmaceuticals International Limited (Allergan) for our Microbiome program focused on our gastrointestinal (GI) indications
  - Provided for an up-front cash payment of $50 million and potential milestone payments of up to $2.8 billion
  - Initial indications consisting of ulcerative colitis, Crohn's disease and irritable bowel syndrome
  - Provisions for joint development and limited co-promotion rights in the US and China
- Announced the initiation of our research and development programs in non-alcoholic steatohepatitis (NASH) and immuno-oncology, among other disease indications of interest in the future for this platform.
- Established manufacturing capabilities enabling the supply of pilot-scale amounts of drug substance and drug product

ON DECK FOR 2018 AND BEYOND

We are highly encouraged by our preclinical and clinical results to date, and are even more excited about what lies ahead.

Our key objectives and anticipated newsflow for 2018 and 2019 build on our story and are as follows:

- Initiate two Phase 2a studies for '731 in HBV patients in summer 2018 with the goals of:
  - Demonstrating that combination with nucleoside therapy can reduce the virus faster than standard of care alone, and can inhibit the generation of cccDNA molecules
  - Presenting initial data in H1 2019
- File IND and initiate Phase 1 for '2158 in 2H 2018
- Identify and advance a third HBV candidate into IND-enabling studies
- Advance microbiome platform
  - IND submission for lead microbiome candidate, M201, by 4Q 2018
  - Identify and advance second microbiome candidates to pre-IND stage
  - Continue to execute on collaboration with Allergan in GI disease
- Advance our Assembly China business for focused development of our HBV programs in the greater China region
- Further grow our translational science expertise to enhance the probability of future development success
- Educating our audiences on the size of our target markets and the substantial unmet needs we have the potential to address

In summary, I am very proud to lead our talented team. They continually impress me with their intelligence, creativity, hard work ethic and dedication they bring to our company and toward achieving our goals. We are unified in our commitment to bring therapies to patients facing serious diseases and are humbled and grateful for the support of patients and families worldwide, who are true partners in our mission. We are making important progress in advancing our strategy and we look forward to updating you on developments throughout the year.

In the meantime, we welcome your questions and comments, which can be sent via the *Contact Us* link on the *Investors page* at our website *assemblybio.com*.

Sincere regards,
Derek A. Small

President and CEO
Assembly Biosciences, Inc.

UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, DC 20549

FORM 10-K

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

For the fiscal year ended December 31, 2017

or

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

For the Transition Period from to

Commission File Number**: 001-35005**

ASSEMBLY BIOSCIENCES, INC.

(Exact name of registrant specified in its charter)

Delaware	**2834**	**20-8729264**
(State or Other Jurisdiction of Incorporation or Organization)	(Primary Standard Industrial Classification Code Number)	(I.R.S. Employer Identification No.)

11711 N. Meridian Street, Suite 310
Carmel, Indiana 46032
(Address of Principal Executive Offices)

Registrant's telephone number, including area code: **(317) 210-9311**

Securities Registered Pursuant to Section 12(b) of the Exchange Act:

Title of Each Class	**Name of Exchange on which Registered**
Common Stock, $0.001 Par Value	Nasdaq Capital Market

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 ☒ No ☐

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

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

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

Indicate by check mark if disclosure of delinquent filers pursuant to Item 405 of Regulation S-K is not contained herein, and will not be contained, to the best of the 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. ☒

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, 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	☐ (Do not check if a smaller reporting company)	Smaller reporting company	☐
		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 Rule 12b-2 of the Exchange Act). Yes ☐ No ☒

The aggregate market value of the voting stock held by non-affiliates of the registrant, as of June 30, 2017, was approximately $324.9 million. Such aggregate market value was computed by reference to the closing price of the common stock as reported on the Nasdaq Capital Market on June 30, 2017. For purposes of making this calculation only, the registrant has defined affiliates as including only (i) directors, (ii) executive officers, and (iii) certain shareholders that hold greater than 10% of the voting stock of the registrant, in each case, as of June 30, 2017. Shares of common stock held by other persons, including certain other holders of more than 10% of the registrant's outstanding common stock, have not been excluded from the above calculation in that such persons are not deemed to be affiliates. The determination of affiliate status is not necessarily a conclusive determination for other purposes.

As of March 5, 2018, there were 20,251,312 shares of the registrant's common stock, $0.001 par value per share, outstanding.

DOCUMENTS INCORPORATED BY REFERENCE

Part III of this Annual Report on Form 10-K incorporates information by reference to portions of the definitive proxy statement for the Company's Annual Meeting of Stockholders to be held in 2018, to be filed within 120 days of the registrant's fiscal year ended December 31, 2017.

ASSEMBLY BIOSCIENCES, INC.

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References to Assembly Biosciences, Inc.

Throughout this Annual Report on Form 10-K, the "Company," "Assembly," "we," "us," and "our," except where the context requires otherwise, refer to Assembly Biosciences, Inc. and its consolidated subsidiaries, and "our board of directors" refers to the board of directors of Assembly Biosciences, Inc.

Forward Looking Information

This Annual Report on Form 10-K contains "forward-looking statements" within the meaning of Section 27A of the Securities Act of 1933, as amended (the Securities Act) and Section 21E of the Securities Exchange Act of 1934, as amended (the Exchange Act). Such forward-looking statements involve substantial risks and uncertainties. All statements, other than statements of historical facts, contained in this Annual Report on Form 10-K, including statements regarding our strategy, future operations, future financial position, future revenues, projected costs, prospects, plans and objectives of management, are forward-looking statements. The words "anticipate," "believe," "estimate," "expect," "intend," "may," "plan," "predict," "project," "target," "potential," "will," "would," "could," "should," "continue" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.

The forward-looking statements in this Annual Report on Form 10-K include, among other things, statements about:

- the initiation, timing, progress and results of nonclinical studies and clinical trials, and our research and development programs;
- the clinical and therapeutic potential of our product candidates;
- our unproven approach to therapeutic intervention;
- the potential benefits of our existing collaborations and our ability to establish and maintain collaborations, including with Allergan Pharmaceuticals International Limited;
- our ability to obtain additional funding;
- the timing or likelihood of regulatory filings and approvals for our product candidates;
- the implementation of our business model, strategic plans for our business, product candidates and technology;
- our commercialization, marketing and manufacturing capabilities and strategy;
- the rate and degree of market acceptance for our product candidates, if approved, and their clinical utility;
- our plans to develop and commercialize our product candidates;
- our ability to retain and recruit key personnel;
- our ability to manage growth;
- our competitive position;
- our intellectual property position;
- developments and projections relating to our competitors and our industry; and
- our estimates regarding expenses, future revenue, capital requirements and needs for additional financing.

We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make. We have included important factors in the cautionary statements included in this Annual Report on Form 10-K, particularly in the "Risk Factors" section, that could cause actual results or events to differ materially from the forward-looking statements that we make.

You should read this Annual Report on Form 10-K and the documents that we have filed as exhibits to this Annual Report on Form 10-K completely and with the understanding that our actual future results may be materially different from what we expect. We do not assume any obligation to update any forward-looking statements, whether as a result of new information, future events or otherwise, except as required by law.

PART I

Item 1. Business

Overview

We are a clinical stage biotechnology company advancing two innovative platform programs: a new class of oral therapeutic candidates for the treatment of hepatitis B virus (HBV) infection and a novel class of oral synthetic live biotherapeutic candidates, which are designed to treat disorders associated with the microbiome.

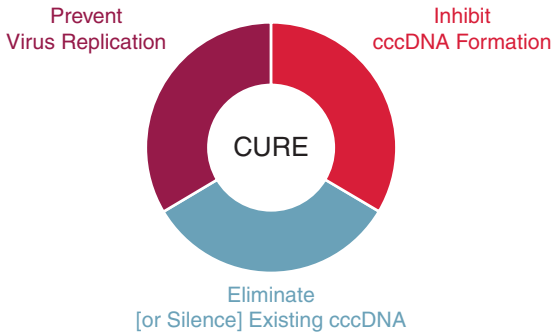
Over 250 million people worldwide are chronically infected with HBV. Our HBV-cure program is pursuing multiple drug candidates that inhibit the HBV lifecycle and block the generation of covalently closed circular DNA (cccDNA), with the aim of increasing the current low cure rate for patients with HBV. We have discovered multiple novel Core protein Allosteric Modifiers (CpAMs), which are small molecules that directly target and allosterically modulate the HBV core (HBc) protein.

Our Microbiome program consists of a fully integrated platform that includes a disease targeted strain identification and selection process, methods for strain isolation and growth under current Good Manufacturing Practice (cGMP) conditions, and a patent pending delivery system that we call GEMICEL®, which is designed to allow for targeted oral delivery of live biologic and conventional therapies to the lower gastrointestinal (GI) tract. Using our microbiome platform, we are developing product candidates for various disease indications, including ulcerative colitis, Crohn's disease, irritable bowel syndrome, non-alcoholic steatohepatitis (NASH), immuno-oncology and Clostridium difficile infections (CDI), which we intend to develop either internally or in collaboration with partners.

Business Strategy

We are currently focused on enhancing the health and well-being of patients with hard-to-treat infectious diseases, such as chronic HBV and illnesses associated with a dysbiotic microbiome. This commitment drives our efforts to forge a new and differentiated path to treating these conditions, inspired by the needs of millions of affected patients. We are pursuing a portfolio of novel CpAMs with potential to substantially increase the cure rates of treated HBV patients and a novel class of oral synthetic live biotherapeutics designed to correct or repair a dysbiotic microbiome. Both of these conditions include substantial numbers of patients for whom current therapies may successfully suppress disease, but offer only low rates of cure. We intend to progress our HBV-cure and Microbiome programs using a variety of strategic arrangements, which may include collaborations, licenses, partnerships and other types of business arrangements. In January 2017, we entered into a Research, Development, Collaboration and License Agreement (the Collaboration Agreement) with Allergan Pharmaceuticals International Limited (Allergan) to develop and commercialize select microbiome gastrointestinal disease therapies. Pursuant to the terms of the Collaboration Agreement, in connection with the closing of the transaction on February 10, 2017, we received from Allergan an upfront payment of $50 million. Additionally, we are eligible to receive up to approximately $630 million in development milestone payments and up to approximately $2.15 billion in commercial milestone payments contingent upon the successful development and commercialization of licensed compounds for up to six different indications. We are also eligible to receive tiered royalties at rates ranging from the mid-single digits to the mid-teens based on net sales.

HBV-Cure Program



The goal of our HBV-cure program is to substantially increase clinical cure rates for those chronically infected with HBV, a pathogen that has infected over 250 million people worldwide and is associated with 887,000 deaths in 2015, according to the World Health Organization (WHO). Our HBV-cure research team is working on discovering and developing multiple CpAMs with the potential to inhibit the functional activities of the HBV core protein (HBc), at multiple points in the viral lifecycle.

HBc is involved in several steps of the HBV lifecycle and is essential for HBV's continued regeneration and prolonged survival. Modulation of HBc with our CpAMs has demonstrated preclinical proof of principle. In multiple cell-based models, CpAMs have been observed to selectively reduce viral load, which is the amount of infectious viral particles released from infected cells, and block the generation of closed circular covalent DNA (cccDNA), a special DNA moiety that resides in the cell nucleus of HBV-infected cells and is associated with viral persistence and chronic infection. The goal is to eradicate the infection with an orally-administered regimen. We believe that we are well positioned to execute on this strategy, with a scientific team that is highly experienced working on treatments for HBV.

Background

HBV is a chronic infectious disease of the liver. It is a leading global cause of chronic liver disease and liver transplants. The WHO estimates that over 250 million people worldwide are infected with HBV. According to the WHO, 887,000 people died in 2015 as a result of HBV, mostly from complications, including cirrhosis and hepatocellular carcinoma. The Centers for Disease Control and Prevention (CDC) estimates that between 850,000 and 2.2 million people in the United States have been infected with HBV, and the WHO has reported that an estimated 90 million people in China have chronic HBV. HBV is a global epidemic infecting more people than hepatitis C virus and HIV infections combined. A relatively small proportion of HBV patients currently receive treatment — according to the WHO, in 2015, of the over 250 million people living with HBV infection, the global treatment coverage only covered approximately 1.7 million. Further, less than 10% of treated patients exhibit a clinical cure, defined as loss of the viral surface antigen (HBsAg) with sustained response off therapy. Despite the low rates of treatment and clinical cure, the current worldwide market for HBV therapies is estimated to be $3.2 billion. If new therapies can improve cure rates, we believe the market could grow substantially due to an increase in the number of HBV patients expected to seek the new therapies.

Current Treatments

Current therapeutic options for HBV include:

- **Direct Acting Antiviral medications (Nucelos(t)ide analogs).** Several antiviral medications — including lamivudine (Epivir®), adefovir (Hepsera®), telbivudine (Tyzeka®), tenofovir disoproxil fumarate and entecavir (Baraclude®) — effectively reduce circulating virus levels by inhibiting reverse transcription. Chronic therapy with these agents can result in reduced liver inflammation and fibrosis. Unfortunately, these are rarely curative, even after years of therapy, and viral replication resumes when therapy is stopped.

- **Pegylated Interferon alfa (PegIFN-).** This synthetic version of a substance produced by the body to fight infection is used mainly for people infected with HBV who do not want to undergo long-term treatment (e.g., patients who might want to become pregnant within a few years). It is administered by injection. Cure rates are relatively low and side effects may be severe, including flu-like symptoms and depression.

Our HBV Focus: Leveraging HBV Core Protein to Achieve a Clinical Cure using Core Protein Allosteric Modifiers (CpAMs)

HBV is a DNA-virus that establishes a reservoir of cccDNA in the nucleus of infected cells that sustains infection in the liver. No current oral therapies target cccDNA activity directly, and thus molecules that can modulate cccDNA are highly sought in the HBV field. A key focus of our HBV-cure effort is targeting the HBc, a highly conserved viral structural protein that has no human homologue and is involved in numerous aspects of the HBV lifecycle, including the generation of the viral cccDNA. We have discovered multiple novel series of CpAMs, which are small molecules that directly target and allosterically

modulate HBc functions. Our HBV pipeline therefore offers the potential for both first in class and best in class opportunities for developing agents that target critical steps involved in the cccDNA viral lifecycle. We believe that our approach of targeting viral cccDNA generation through inhibition of HBc functionality provides a promising foundation for substantially improving clinical cure rates for HBV.

A well accepted benchmark for therapeutic agents aiming to decrease cccDNA levels is the use of several key viral antigens as surrogate biomarkers of active cccDNA. The same biomarkers can be used in both primary human hepatocyte cells and patients. On this basis, our CpAMs have shown preclinical proof of principle. In a variety of cell culture models, CpAMs have demonstrated the ability to reduce production of viral HBV DNA levels and known surrogate markers for cccDNA — HBV E antigen (HBeAg), HBV S antigen (HBsAg) and pre-genomic RNA (pgRNA). Sustained decreases in levels of HBsAg is considered a strong predictor of functional cure in patients and is a key biomarker for clinical development.

Our clinical strategy encompasses testing CpAMs as a monotherapy, as required by regulatory agencies, to demonstrate their antiviral activity. Thereafter, all subsequent clinical trials in patients are anticipated to be in combination with other classes of HBV therapies. The lead product candidate from this program, ABI-H0731, was observed to be active in primary human hepatocytes and exhibited antiviral potency against all HBV genotypes tested (A, B, C and D) as well as nucleoside resistant mutants. ABI-H0731 has completed the Phase 1a portion of a Phase 1a/1b human clinical trial in New Zealand, and commenced the Phase 1b portion of the clinical trial in the second quarter of 2017 in New Zealand and other countries outside the United States. We expect to release topline interim data from the Phase 1b portion of the clinical trial and full results in the first half of 2018. Assuming a successful Phase 1b monotherapy clinical trial, we expect to initiate longer Phase 2 combination clinical trials in mid-2018 and potentially have initial data in the second half of 2018. Larger Phase 2b combination clinical trials are anticipated for 2019. We have also filed an Investigational New Drug (IND) application and have initiated an additional Phase 1a pharmacokinetic, safety and tolerability study of ABI-H0731 in the United States.

In the Phase 1a dose ranging portion of our Phase 1a/1b clinical trial of ABI-H0731 in New Zealand, we assessed the safety, tolerability and pharmacokinetics of ABI-H0731 in 48 healthy volunteers. In this clinical trial, single ascending doses of 100-1,000 mg per day were evaluated in addition to 7-day repeat dosing with 800 mg once daily as well as 800 mg twice daily dosing. ABI-H0731 was reported to be well tolerated at all doses. No serious adverse events, no clinically significant adverse events, no withdrawals due to adverse events nor clinically significant changes in vital signs or electrocardiography findings were observed. Treatment emergent laboratory abnormalities were transient, minor and/or deemed not clinically significant. Treatment-related adverse events deemed by the clinical investigator to be possibly or probably related to the study drug included headaches and rashes, which were reported to be mild and transient and only observed at the highest doses. Pharmacokinetic data from the Phase 1a portion of the trial exhibited dose-dependent increases in plasma exposure levels, low subject-to-subject variability and a half-life supporting the potential for once daily dosing. ABI-H0731 was observed to be well absorbed and associated with plasma concentrations that we believe will be sufficient to suppress viral replication and cccDNA generation.

The Phase 1b portion of the clinical trial commenced in the second quarter of 2017 at sites outside the United States. The Phase 1b trial is intended to assess the safety, tolerability and pharmacokinetics, as well as antiviral efficacy of ABI-H0731 in patients with chronic HBV infection. We expect to commence a separate Phase 2 combination clinical trial in mid-2018 in combination with other approved therapies that will treat patients for several months. This Phase 2 clinical trial will assess safety over a prolonged treatment period and monitor surrogate markers of cccDNA. We expect to commence a larger Phase 2b combination clinical trial in 2019 that is intended to be registrational in nature and monitor both surrogate biomarkers of cccDNA and sustained off-therapy antiviral responses in various patient populations.

Our CpAM program provides opportunities to create a pipeline of antiviral drugs derived from diverse chemical scaffolds. In the fourth quarter of 2017, we announced the selection of our second product candidate from this program, ABI-H2158, which is currently undergoing IND-enabling studies. ABI-H2158 is an internally discovered and developed product candidate. In nonclinical studies, we observed enhanced potency of ABI-H2158 compared to ABI-H0731 while ABI-H2158 maintained a favorable pharmacokinetic profile. Pending the successful completion of IND-enabling studies, this product candidate is expected to begin a Phase 1a human clinical trial in the second half of 2018. We anticipate the selection of a third CpAM product candidate from this program by the end of the first half of 2018.

Our current intention is to focus our development and, if approved, commercialization efforts for our HBV-cure program in major markets throughout the world.

License Agreement and Intellectual Property

On September 3, 2013, we entered into an exclusive license agreement (the IURTC License Agreement) with Indiana University Research and Technology Corporation (IURTC) to acquire the rights to develop and commercialize products associated with multiple patents and patent applications covering aspects of our HBV program held by IURTC. The licensed intellectual property includes platform patent applications covering aspects of HBc, our novel mechanisms of action, formulation, methods of treatment and the novel drug development assays our team is creating. As part of this agreement, we are obligated to make milestone payments based upon the successful accomplishment of clinical and regulatory milestones. The aggregate amount of all performance milestone payments under the IURTC License Agreement, should all performance milestones through development be met, is $825,000. As of December 31, 2017, no performance milestone payments have been made. We anticipate that the first performance milestone payment will be made to IURTC in 2018. Under the IURTC License Agreement, we are also obligated to pay IURTC royalty payments based on net sales of the licensed technology ranging from 0.5% to 1.75%.

In addition, the IURTC License Agreement requires an annual diligence maintenance fee as follows:

2014	$ 25,000
2015	$ 50,000
2016 through the year in which first commercial sale occurs	$ 75,000
Year following first commercial sale and all subsequent years	$100,000

Milestone payments received by IURTC are fully creditable against the diligence maintenance fee for the year in which they are received.

Under the IURTC License Agreement, we also may grant sublicenses to third parties and receive revenues from these sublicenses, a portion of which will be paid to IURTC. Pursuant to this agreement, we must provide IURTC with a development plan, update this plan annually and achieve certain commercial goals. We have also agreed to indemnify IURTC against certain actions and claims in connection with the agreement or the use of any of the licensed patent rights.

The IURTC License Agreement may be terminated by us, with or without cause, upon 90 days advance written notice, by IURTC upon our material breach with 60 days advance written notice or by IURTC, in certain cases, upon our insolvency or bankruptcy immediately upon written notice.

We have one pending U.S. patent application, and related worldwide patent applications, and one pending PCT patent application, directed to ABI-H0731, all of which are co-owned with Indiana University and licensed to us. We also have a pending provisional application directed to formulations of ABI-H0731 which is owned by us. In addition, we have two pending PCT applications, co-owned with Indiana University, and four provisional patent applications, owned by us, directed to compounds related to ABI-H0731. We have one provisional patent application owned by us directed to ABI-H2158, which was developed outside of the IURTC License Agreement.

Microbiome Program Platform

Background

Our Microbiome program is based on the targeted delivery of novel microbiome-based therapies in a patent pending oral formulation, called GEMICEL®, which applies our novel coating and encapsulation technology allowing for targeted delivery of complex agents to select regions of the gastrointestinal (GI) tract. Using this proprietary delivery platform, we aim to deliver selected combinations of monoculture strains of beneficial bacteria in novel "synthetic formats" to the GI tract. In September 2017, we elected not to initiate a Phase 1b clinical trial of our initial product candidate, ABI-M101, in patients with Clostridium difficile infections (CDI) who have relapsed after two or three standard antibiotic regimens. We will continue to assess development activities with respect to ABI-M101 over the next twelve months. The Microbiome program is prioritizing efforts on optimizing our lead product candidates, ABI-M201 (Ulcerative Colitis) and ABI-M301 (Crohn's disease) in preparation for studies to support potential IND applications. Using our microbiome platform, we are exploring additional product candidates for other disease indications, including irritable bowel syndrome, non-alcoholic steatohepatitis (NASH), immuno-oncology and CDI, which indications we may pursue either internally or in collaboration with partners.

In recent years, there has been increasing scientific evidence suggesting the therapeutic potential of the human microbiome — the billions of microbes living in and on people — to impact health and disease. Our approach builds upon experience reported in the literature of successfully treating various disease indications with fecal material transplants (FMT) and seeks to provide a potentially curative therapy using a "drug like" approach that delivers targeted and specific microbiome therapies in an oral capsule.

Collaboration Agreement, License Agreement and Intellectual Property

Allergan

On January 6, 2017, we entered into the Collaboration Agreement with Allergan to develop and commercialize select microbiome gastrointestinal programs. Pursuant to the Collaboration Agreement, we granted Allergan an exclusive worldwide license for rights to preclinical compounds ABI-M201 and ABI-M301, targeting ulcerative colitis (UC) and Crohn's disease (CD), respectively, as well as two additional compounds to be identified by us for irritable bowel syndromes (IBS).

Under the Collaboration Agreement, we and Allergan will collaborate on research and development activities with respect to the licensed compounds in accordance with a mutually agreed upon research and development plan.

Pursuant to the terms of the Collaboration Agreement, in connection with the closing of the transaction on February 10, 2017, we received from Allergan an upfront payment of $50 million. Additionally, we are eligible to receive up to approximately $630 million in development milestone payments and up to approximately $2.15 billion in commercial milestone payments contingent upon the successful development and commercialization of licensed compounds for up to six different indications. We are also eligible to receive tiered royalties at rates ranging from the mid-single digits to the mid-teens based on net sales. We and Allergan have agreed to share development costs up to an aggregate of $75 million through proof-of-concept (POC) studies on a 1/3, 2/3 basis, respectively, and Allergan has agreed to assume all post-POC development costs. In the event any pre-POC development costs exceed $75 million in the aggregate, we may elect either (a) to fund 1/3 of such costs in excess of $75 million or (b) to allow Allergan to deduct from future development milestone payments 1/3 of the development costs funded by Allergan in excess of $75 million plus a premium of 25%. We have an option to co-promote the licensed programs in the United States and China, subject to certain conditions set forth in the Collaboration Agreement.

Allergan may terminate the Collaboration Agreement for convenience at any time upon either 90 days' (prior to the initiation of the first POC trial of a licensed product) or 120 days' (after the initiation of the first POC trial of a licensed product), as applicable, advance written notice to us. The Collaboration Agreement also contains customary provisions for termination by either party, including in the event of breach of the Collaboration Agreement, subject to cure.

Therabiome

On November 8, 2013, we entered into a License and Collaboration Agreement with Therabiome, LLC (Therabiome) for all intellectual property and know-how owned or controlled by Therabiome relating to the oral delivery of pharmaceutical drugs to specific sites in the intestine, using a pH sensitive release platform technology. Under the agreement, Therabiome granted us the exclusive worldwide license, with rights to sublicense, to develop the intellectual property for commercialization (a) in the use of bacteria, viruses, proteins, and small molecules by oral delivery using the licensed intellectual property in (i) gastrointestinal dysbiosis, including but not limited to C. *difficile* associated diseases, irritable bowel syndrome-constipation, irritable bowel syndrome-diarrhea, inflammatory bowel disease, metabolic syndrome, type 2 diabetes, obesity and hypertension, (ii) auto-immune disorders and autism, including but not limited to as controlled by bacteria or virus, and (iii) orally delivered vaccines, including viral and bacterial, and (b) any oral delivery of small molecules using the licensed intellectual property. We will be solely responsible for all research and development activities with respect to any product we develop under the license.

For the license, we paid Therabiome an upfront non-refundable license fee of $300,000. In September 2014, we paid Therabiome $100,000 upon the occurrence of the first proof of principle for a bacteria strain. We will be required to pay an additional $100,000 upon the occurrence of the proof of principle for a virus. We must pay Therabiome clinical and regulatory milestones for each product or therapy advanced from the platform, for U.S. regulatory milestones, depending on whether the milestone occurs before the filing of the first new drug application (NDA) for a product or after the first, second or third NDA filings, as follows:

Regulatory and Clinical Milestones	
Upon the filing of an IND with the FDA:	$100,000 – $130,000
First dose first patient – Phase I Clinical Trial	$250,000 – $325,000
First dose first patient – Phase II Clinical Trial	$500,000 – $650,000
First dose first patient – Phase III Clinical Trial	$750,000 – $975,000
Upon filing of an NDA or BLA with the FDA	$1,000,000 – $1,300,000
Upon marketing approval by the FDA	$3,000,000
Upon approval of a supplemental NDA (sNDA) for a new Indication, in the United States	$1,000,000

We also must pay Therabiome lesser amounts for foreign regulatory milestones, which vary by country and region, and which depend on whether the milestone occurs before the filing of the first NDA filing or after the first, second or third NDA filings. These payments will be: one-third of the U.S. milestones paid

upon a foreign equivalent of an IND and marketing approval for each product in the European Union or Japan; 10% of the U.S. milestones paid upon a foreign equivalent of an IND and marketing approval for each product in China; 10% of the U.S. milestone paid upon marketing approval for each product in India and Brazil; and 1% of the U.S. milestone paid upon marketing approval for each product in all other countries. We also must pay Therabiome royalties on annual net product sales in the low to mid-single digit percentages plus, once annual net sales exceed two specified thresholds, a one-time cash payment upon reaching each threshold. Pursuant to this agreement, we and Therabiome have agreed to indemnify one another against certain claims in connection with the agreement.

This agreement may be terminated by us, with or without cause, upon 90 days prior written notice, by either party upon the other party's material breach with 180 days prior written notice or by either party upon the other party's challenge of the validity or enforceability or any issued patent within the licensed intellectual property with 90 days prior written notice. Additionally, either party may terminate the agreement upon an event of bankruptcy with respect to the other party.

We have two pending U.S. patent application families and related foreign pending patent applications directed to the delivery mechanism licensed to us from Therabiome. We also own the GEMICEL® trademark covering the delivery mechanism we have licensed from Therabiome.

Government Regulation

Government authorities in the United States, at the federal, state and local level, and in other countries extensively regulate, among other things, the research, development, testing, manufacture, including any manufacturing changes, packaging, storage, recordkeeping, labeling, advertising, promotion, distribution, marketing, post-approval monitoring and reporting, import and export of pharmaceutical and biological products, such as those we are developing.

U.S. drug approval process

In the United States, the FDA regulates drugs under the Federal Food, Drug, and Cosmetic Act (FDCA) and implementing regulations, and biological products under both the FDCA and the Public Health Service Act (PHSA) and implementing regulations. The process of obtaining regulatory approvals and the subsequent compliance with appropriate federal, state, local and foreign statutes and regulations requires the expenditure of substantial time and financial resources. Failure to comply with the applicable U.S. requirements at any time during the product development process, approval process or after approval, may subject an applicant to a variety of administrative or judicial sanctions, such as the FDA's refusal to approve pending applications, withdrawal of an approval, imposition of a clinical hold, issuance of warning letters and untitled letters, product recalls, product seizures, total or partial suspension of production or distribution injunctions, fines, refusals of government contracts, restitution, disgorgement of profits or civil or criminal penalties.

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

- completion of nonclinical laboratory tests and animal studies in compliance with the FDA's good laboratory practice (GLP) regulations, as required;
- submission to the FDA of an IND which must become effective before human clinical trials may begin;
- approval by an independent institutional review board (IRB) at each clinical site before each trial may be initiated;
- performance of adequate and well-controlled human clinical trials in accordance with good clinical practices (GCP) to establish the safety and efficacy of the proposed drug or biological product for each indication;
- submission to the FDA of a new drug application (NDA) or a biologics license application (BLA);

- satisfactory completion of an FDA inspection of the manufacturing facility or facilities at which the product is produced to assess compliance with current good manufacturing practices (cGMP) requirements and to assure that the facilities, methods and controls are adequate to preserve the product's identity, strength, quality and purity; and
- FDA review and approval of the NDA or BLA.

Nonclinical Studies and IND

Nonclinical studies include laboratory evaluation of product chemistry and formulation, as well as *in vitro* and animal studies to assess the potential for adverse events and in some cases to establish a rationale for therapeutic use. The conduct of nonclinical studies is subject to federal regulations and requirements, including GLP regulations for safety/toxicology studies. An IND sponsor must submit the results of the nonclinical tests, together with manufacturing information, analytical data, any available clinical data or literature and plans for clinical trials, among other things, to the FDA as part of an IND. Some long-term nonclinical testing, such as animal tests of reproductive adverse events and carcinogenicity, may continue after the IND is submitted. For some products, the FDA may waive the need for certain nonclinical tests. An IND automatically becomes effective 30 days after receipt by the FDA, unless before that time the FDA raises concerns or questions related to one or more proposed clinical trials and places the trial on clinical hold. If an IND or clinical study is placed on clinical hold, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical trial can begin. As a result, submission of an IND may not result in the FDA allowing clinical trials to commence.

Clinical trials

Clinical trials involve the administration of the investigational new drug or biological product to human subjects under the supervision of qualified investigators in accordance with GCP requirements, which include, among other things, the requirement that all research subjects provide their informed consent in writing before their participation in any clinical trial. Clinical trials are conducted under written study protocols detailing, among other things, the objectives of the study, the parameters to be used in monitoring safety, and the effectiveness criteria to be evaluated. A protocol for each clinical trial and any subsequent protocol amendments must be submitted to the FDA as part of the IND. In addition, an IRB at each institution participating in the clinical trial must review and approve the plan for any clinical trial before it commences at that institution, and the IRB must conduct continuing review. The IRB must review and approve, among other things, the study protocol and informed consent information to be provided to study subjects. An IRB must operate in compliance with FDA regulations. Information about certain clinical trials must be submitted within specific timeframes to the National Institutes of Health for public dissemination at www.clinicaltrials.gov.

Human clinical trials are typically conducted in three sequential phases, which may overlap or be combined:

- *Phase 1:* The drug or biological product is initially introduced into healthy human subjects or patients with the target disease or condition and tested for safety, dosage tolerance, absorption, metabolism, distribution, excretion and, if possible, to gain an early indication of its effectiveness.
- *Phase 2:* The drug or biological product is administered to a limited patient population to identify possible adverse effects and safety risks, to preliminarily evaluate the efficacy of the product for specific targeted diseases and to determine dosage tolerance and optimal dosage.
- *Phase 3:* The drug or biological product is administered to an expanded patient population in adequate and well-controlled clinical trials to generate sufficient da ta to statistically confirm the efficacy and safety of the product for approval, to establish the overall risk-benefit profile of the product and to provide adequate information for the labeling of the product.

Progress reports detailing the results of the clinical trials must be submitted at least annually to the FDA. Additionally, IND safety reports must be submitted for serious and unexpected suspected adverse reactions, findings from animal or *in vitro* testing or other studies that suggest a significant risk to humans, and any clinically important increase in the rate of a serious suspected adverse reaction over that listed in

the protocol or investigator brochure. Phase 1, Phase 2 and Phase 3 clinical trials may not be completed successfully within any specified period, or at all. Furthermore, the FDA or the sponsor may suspend or terminate a clinical trial at any time on various grounds, including a finding that the research subjects are being exposed to an unacceptable health risk. Similarly, an IRB can suspend or terminate approval of a clinical trial at its institution if the clinical trial is not being conducted in accordance with the IRB's requirements or if the drug or biological product has been associated with unexpected serious harm to patients.

Marketing approval

Assuming successful completion of the required clinical testing, the results of the nonclinical studies and clinical trials, together with detailed information relating to the product's chemistry, manufacture, controls and proposed labeling, among other things, are submitted to the FDA as part of an NDA or BLA requesting approval to market the product for one or more indications. Under federal law, the submission of most NDAs and BLAs is additionally subject to a substantial application user fee, currently approximately $2.4 million and the sponsor of an approved NDA or BLA is also subject to an annual program fee currently set at approximately $304,000 for fiscal year 2018. These fees are typically adjusted annually.

The FDA conducts a preliminary review of all NDAs and BLAs within the first 60 days after submission before accepting them for filing to determine whether they are sufficiently complete to permit substantive review. The FDA may request additional information rather than accept an NDA or BLA for filing. In this event, the application must be resubmitted with the additional information. The resubmitted application is also subject to review before the FDA accepts it for filing. Once the submission is accepted for filing, the FDA begins an in-depth substantive review. The FDA has agreed to specified performance goals in the review of NDAs and BLAs. Under these goals, the FDA has committed to review most original applications for non-priority products within ten months, and most original applications for priority review products, that is, drugs and biological products that the FDA determines represent a significant improvement over existing therapy, within six months. For NDAs for novel products and all BLAs, the ten and six-month time periods runs from the filing date; for all other original applications, the ten and six-month time periods run from the submission date. The review process may be extended by the FDA for three additional months to consider certain information or clarification regarding information already provided in the submission. Despite these review goals, it is not uncommon for FDA review of an NDA or BLA to extend beyond the goal date. The FDA may also refer applications for novel drugs or products that 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. 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 typically will inspect the facility or facilities where the product is manufactured. The FDA will not approve an application unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. In addition, before approving an NDA or BLA, the FDA will typically inspect one or more clinical sites to assure compliance with GCP and integrity of the clinical data submitted.

The testing and approval process requires substantial time, effort and financial resources, and some trials may take many years to complete. Data obtained from clinical activities are not always conclusive and may be susceptible to varying interpretations, which could delay, limit or prevent regulatory approval. The FDA may not grant approval on a timely basis, or at all. We may encounter difficulties or unanticipated costs in our efforts to develop our product candidates and secure necessary governmental approvals, which could delay or preclude us from marketing our products.

After the FDA's evaluation of the NDA or BLA and inspection of the manufacturing facilities, the FDA may issue an approval letter or a complete response letter. An approval letter authorizes commercial marketing of the drug or biological product with specific prescribing information for specific indications. A complete response letter generally outlines the deficiencies in the submission and may require substantial additional testing or information in order for the FDA to reconsider the application. If and when those deficiencies have been addressed to the FDA's satisfaction in a resubmission of the NDA or BLA, the FDA

will issue an approval letter. The FDA has committed to reviewing such resubmissions in two or six months depending on the type of information included. Even with submission of this additional information, the FDA ultimately may decide that the application does not satisfy the regulatory criteria for approval and refuse to approve the NDA or BLA. Even if the FDA approves a product, it may limit the approved indications for use for the product, require that contraindications, warnings or precautions be included in the product labeling, require that post-approval studies, including Phase 4 clinical trials, be conducted to further assess a drug's safety after approval, require testing and surveillance programs to monitor the product after commercialization, or impose other conditions, including distribution and use restrictions or other risk management mechanisms, including Risk Evaluation and Mitigation Strategies (REMs), which can materially affect the potential market and profitability of the product or impose new labeling, testing or distribution and use requirements. The FDA may prevent or limit further marketing of a product based on the results of post-market studies or surveillance programs. After approval, some types of changes to the approved product, such as adding new indications, manufacturing changes and additional labeling claims, are subject to further testing requirements and FDA review and approval.

Fast track designation

The FDA is required to facilitate and expedite the development and review of drugs and biological products that are intended for the treatment of a serious or life-threatening disease or condition for which there is no effective treatment and which demonstrate the potential to address unmet medical needs for the disease or condition. Under the fast track program, the sponsor of a new product candidate may request the FDA to designate the product for a specific indication as a fast track product concurrent with or after the filing of the IND for the product candidate. The FDA must determine if the product candidate qualifies for fast track designation within 60 calendar days after receipt of the sponsor's request.

In addition to other benefits, such as the ability to use surrogate endpoints and have greater interactions with the FDA, the FDA may initiate review of sections of a fast track product's NDA or BLA before the application is complete. This rolling review is available if the applicant provides and the FDA approves a schedule for the submission of the remaining information and the applicant pays applicable user fees. However, the FDA's time period goal for reviewing a fast track application does not begin until the last section of the NDA or BLA is submitted. In addition, the fast track designation may be withdrawn by the FDA if the FDA believes that the designation is no longer supported by data emerging in the clinical trial process.

Priority review

Under FDA policies, a product candidate may be eligible for priority review, a review generally within a six-month time frame from the time a complete application is received or filed. Products generally are eligible for priority review if they are intended for treatment of a serious or life-threatening disease or condition and provide a significant improvement in safety or effectiveness compared to marketed products in the treatment, diagnosis or prevention of a serious disease or condition. A fast track designated product candidate would ordinarily meet the FDA's criteria for priority review.

Accelerated approval

Under the FDA's accelerated approval regulations, the FDA may approve a drug or biological product for a serious or life-threatening illness that provides meaningful therapeutic benefit to patients over existing treatments based upon a surrogate endpoint that is reasonably likely to predict clinical benefit or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality (IMM). In clinical trials, a surrogate endpoint is a measurement of laboratory or clinical signs of a disease or condition that substitutes for a direct measurement of how a patient feels, functions or survives. Surrogate endpoints can often be measured more easily or more rapidly than clinical endpoints. A product candidate approved on this basis is subject to rigorous post-marketing compliance requirements, including the completion of Phase 4 or post-approval clinical trials to confirm the effect on the clinical endpoint. Failure to conduct required post-approval studies, or confirm a clinical benefit during post-marketing studies, would allow the FDA to withdraw the drug from the market on an expedited basis. All promotional materials for drug candidates approved under accelerated regulations are subject to prior review by the FDA.

Breakthrough therapy designation

Under the provisions of the new Food and Drug Administration Safety and Innovation Act (FDASIA), enacted in 2012, a sponsor can request designation of a product candidate as a "breakthrough therapy." A breakthrough therapy is defined as a drug or biological product that is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the drug or biological product may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. Drugs and biological products designated as breakthrough therapies also may be eligible for priority review. The FDA must take certain actions, such as holding timely meetings and providing advice, intended to expedite the development and review of an application for approval of a breakthrough therapy. Even if a product qualifies for one or more of these programs, the FDA may later decide that the product no longer meets the conditions for qualification or decide that the time period for FDA review or approval will not be shortened.

Qualified Infectious Disease Products

Under the provisions of FDASIA, a sponsor can request designation of a product candidate as a qualified infectious disease product (QIDP). A QIDP is an antibacterial or antifungal drug for human use intended to treat serious or life threatening infections, including those caused by (i) an antibacterial or antifungal resistant pathogen, including novel or emerging infectious pathogens; or (ii) qualifying pathogens listed by the FDA. Examples include (a) resistant gram positive pathogens, including methicillin-resistant Staphylococcus aureus, vancomycin-resistant Staphylococcus aureus, and vancomycin-resistant enterococcus; (b) multi-drug resistant gram negative bacteria, including Acinetobacter, Klebsiella, Pseudomonas, and E. coli species; (c) multi-drug resistant tuberculosis; and (d) Clostridium difficile. A drug that receives QIDP designation is eligible under the statute for fast track designation and priority review and an additional five years of market exclusivity added to certain existing exclusivity periods. The FDA must determine if the product candidate qualifies as a QIDP within 60 calendar days after receipt of the sponsor's request.

Orphan drugs

Under the Orphan Drug Act, the FDA may grant orphan drug designation to drugs or biological products intended to treat a rare disease or condition, which is generally defined as a disease or condition that affects fewer than 200,000 individuals in the United States. Orphan drug designation must be requested before submitting an NDA or BLA. After the FDA grants orphan drug designation, the identity of the product and its potential orphan use are disclosed publicly by the FDA. Orphan drug designation does not shorten the duration of the regulatory review and approval process. The first NDA or BLA applicant to receive FDA approval for a particular active moiety to treat a particular disease with FDA orphan drug designation is entitled to a seven-year exclusive marketing period in the United States for that product and indication. During the seven-year exclusivity period, the FDA may not approve any other applications to market the same drug or biological product for the same orphan indication, except in limited circumstances, such as a showing of clinical superiority to the product with orphan drug exclusivity. A drug or biological product will be considered clinically superior if it is shown to be safer, more effective or makes a major contribution to patient care. Orphan drug exclusivity does not prevent the FDA from approving a different drug or biological product for the same orphan disease or condition, or the same drug or biological product for a different disease or condition. Among the other benefits of orphan drug designation are tax credits for certain research and a waiver of the NDA/BLA application user fee.

Pediatric information

Under the Pediatric Research Equity Act of 2003, as amended, an NDA, BLA or supplement to an NDA or BLA for drug or biological products with certain novel features (e.g., new active ingredient new indication) must contain data that are adequate to assess the safety and effectiveness of the drug or biological product for the claimed indications in all relevant pediatric subpopulations, and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective. The FDA may, on its own initiative or at the request of the applicant, grant deferrals for submission of some or

all pediatric data until after approval of the product for use in adults, or full or partial waivers from the pediatric data requirements. A sponsor of a new drug or biological product subject to the above pediatric testing requirements also is required to submit to the FDA a pediatric study plan generally 60 days after an end-of-Phase 2 meeting with the agency. Unless otherwise required by regulation, the pediatric data requirements do not apply to products with orphan drug designation.

Combination products

The FDA regulates combinations of products that cross FDA centers, such as drug, biologic or medical device components that are physically, chemically or otherwise combined into a single entity, as a combination product. The FDA center with primary jurisdiction for the combination product will take the lead in the premarket review of the product, with the other center consulting or collaborating with the lead center.

The FDA's Office of Combination Products (OCP) determines which center will have primary jurisdiction for the combination product based on the combination product's "primary mode of action." A mode of action is the means by which a product achieves an intended therapeutic effect or action. The primary mode of action is the mode of action that provides the most important therapeutic action of the combination product, or the mode of action expected to make the greatest contribution to the overall intended therapeutic effects of the combination product.

Often it is difficult for the OCP to determine with reasonable certainty the most important therapeutic action of the combination product. In those difficult cases, the OCP will consider consistency with other combination products raising similar types of safety and effectiveness questions, or which center has the most expertise to evaluate the most significant safety and effectiveness questions raised by the combination product.

A sponsor may use a voluntary formal process, known as a Request for Designation, when the product classification is unclear or in dispute, to obtain a binding decision as to which center will regulate the combination product. If the sponsor objects to that decision, it may request that the agency reconsider that decision.

Other regulatory requirements

Any drug or biological product manufactured or distributed by us pursuant to FDA approvals will be subject to pervasive and continuing regulation by the FDA, including, among other things, requirements relating to recordkeeping, periodic reporting, product sampling and distribution, advertising and promotion and reporting of adverse experiences with the product. After approval, most changes to the approved product, such as adding new indications or other labeling claims, are subject to prior FDA review and approval.

The FDA may impose a number of post-approval requirements, including REMs, as a condition of approval of an NDA or BLA. For example, the FDA may require post-marketing testing, including Phase 4 clinical trials, and surveillance to further assess and monitor the product's safety and effectiveness after commercialization.

In addition, drug manufacturers and other entities involved in the manufacture and distribution of approved drugs are required to register their establishments with the FDA and state agencies, and are subject to periodic unannounced inspections by the FDA and these state agencies for compliance with cGMP requirements. Changes to the manufacturing process are strictly regulated and often require prior FDA approval before being implemented. FDA regulations also require investigation and correction of any deviations from cGMP and impose reporting and documentation requirements upon us and any third-party manufacturers that we may decide to use. Accordingly, manufacturers must continue to expend time, money and effort in the areas of production and quality control to maintain cGMP compliance.

Once an approval is granted, the FDA may withdraw the approval if compliance with regulatory requirements and standards is not maintained or if problems occur after the product reaches the market. Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or with manufacturing processes, or failure to comply with regulatory requirements,

may result in revisions to the approved labeling to add new safety information, imposition of post-market studies or clinical trials to assess new safety risks or imposition of distribution or other restrictions under a REM program. Other potential consequences include, among other things:

- restrictions on the marketing or manufacturing of the product, complete withdrawal of the product from the market or product recalls;
- fines, warning letters or holds on post-approval clinical trials;
- refusal of the FDA to approve pending applications or supplements to approved applications, or suspension or revocation of product approvals;
- product seizure or detention, or refusal to permit the import or export of products; or
- consent decrees, injunctions or the imposition of civil or criminal penalties.

The FDA strictly regulates marketing, labeling, advertising and promotion of products that are placed on the market. Drugs and biological products generally may be promoted only for the approved indications and in accordance with the provisions of the approved labeling. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off label uses, and a company that is found to have improperly promoted off label uses may be subject to significant liability.

Additional provisions

In addition to FDA restrictions on marketing of pharmaceutical products, several other types of state and federal laws have been applied to restrict certain marketing practices in the pharmaceutical industry in recent years. These laws include anti-kickback statutes and false claims statutes. 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 the one hand and prescribers, purchasers and formulary managers on the other. Violations of the anti-kickback statute are punishable by imprisonment, criminal fines, civil monetary penalties and exclusion from participation in federal healthcare programs. Although there are a number of statutory exemptions and regulatory safe harbors protecting certain common activities from prosecution or other regulatory sanctions, the exemptions and safe harbors are drawn narrowly, and practices that involve remuneration intended to induce prescribing, purchases or recommendations may be subject to scrutiny if they do not qualify for an exemption or safe harbor.

Federal false claims laws prohibit any person from knowingly presenting, or causing to be presented, a false claim for payment to the federal government, or knowingly making, or causing to be made, a false statement to have a false claim paid. Recently, several pharmaceutical and other healthcare companies have been prosecuted under these laws for allegedly inflating drug prices they report to pricing services, which in turn were used by the government to set Medicare and Medicaid reimbursement rates, and for allegedly providing free product to customers with the expectation that the customers would bill federal programs for the product. In addition, certain marketing practices, including off-label promotion, may also violate false claims laws. The majority of states also have statutes or regulations similar to the federal anti-kickback law and false claims laws, which apply to items and services reimbursed under Medicaid and other state programs, or, in several states, apply regardless of the payor.

Physician Drug Samples

As part of the sales and marketing process, pharmaceutical companies frequently provide samples of approved drugs to physicians. The Prescription Drug Marketing Act (PDMA) imposes requirements and limitations upon the provision of drug samples to physicians, as well as prohibits states from licensing distributors of prescription drugs unless the state licensing program meets certain federal guidelines that include minimum standards for storage, handling and record keeping. In addition, the PDMA sets forth civil and criminal penalties for violations.

Foreign Regulation

In order to market any product outside of the United States, we would need to comply with numerous and varying regulatory requirements of other countries regarding safety and efficacy and governing, among other things, clinical trials, marketing authorization, commercial sales and distribution of our products. Whether or not we obtain FDA approval for a product, we would need to obtain the necessary approvals by the comparable regulatory authorities of foreign countries before we can commence clinical trials or marketing of the product in those countries. The approval process varies from country to country and can involve additional product testing and additional administrative review periods. The time required to obtain approval in other countries might differ from and be longer than that required to obtain FDA approval. Regulatory approval in one country does not ensure regulatory approval in another, but a failure or delay in obtaining regulatory approval in one country may negatively impact the regulatory process in others.

New Legislation and Regulations

From time to time, legislation is drafted, introduced and passed in Congress that could significantly change the statutory provisions governing the testing, approval, manufacturing and marketing of products regulated by the FDA. In addition to new legislation, FDA regulations and policies are often revised or interpreted by the agency in ways that may significantly affect our business and our products. It is impossible to predict whether further legislative changes will be enacted or whether FDA regulations, guidance, policies or interpretations changed or what the effect of such changes, if any, may be.

Pharmaceutical Coverage, Pricing and Reimbursement

Significant uncertainty exists as to the coverage and reimbursement status of any drug products for which we may obtain regulatory approval. Sales of any of our product candidates, if approved, will depend, in part, on the extent to which the costs of the products will be covered by third-party payors, including government health programs such as Medicare and Medicaid, commercial health insurers and managed care organizations. The process for determining whether a payor will provide coverage for a drug product may be separate from the process for setting the price or reimbursement rate that the payor will pay for the drug product once coverage is approved. Third-party payors may limit coverage to specific drug products on an approved list, or formulary, which might not include all of the approved drugs for a particular indication.

In order to secure coverage and reimbursement for any product that might be approved for sale, we may need to conduct expensive pharmacoeconomic studies in order to demonstrate the medical necessity and cost-effectiveness of the product, in addition to the trials required to obtain FDA or other comparable regulatory approvals. Our product candidates may not be considered medically necessary or cost-effective. A payor's decision to provide coverage for a drug product does not imply that an adequate reimbursement rate will be approved. Third-party reimbursement may not be sufficient to enable us to maintain price levels high enough to realize an appropriate return on our investment in product development.

The containment of healthcare costs has become a priority of federal, state and foreign governments, and the prices of drugs have been a focus in this effort. Third-party payors are increasingly challenging the prices charged for medical products and services and examining the medical necessity and cost-effectiveness of medical products and services, in addition to their safety and efficacy. If these third-party payors do not consider our products to be cost-effective compared to other available therapies, they may not cover our products after approval as a benefit under their plans or, if they do, the level of payment may not be sufficient to allow us to sell our products at a profit. The U.S. government, state legislatures and foreign governments have shown significant interest in implementing cost containment programs to limit the growth of government-paid health care costs, including price controls, restrictions on reimbursement and requirements for substitution of generic products for branded prescription drugs. Adoption of such controls and measures, and tightening of restrictive policies in jurisdictions with existing controls and measures, could limit payments for pharmaceuticals such as the drug candidates that we are developing and could adversely affect our net revenue and results.

Pricing and reimbursement schemes vary widely from country to country. Some countries provide that drug products may be marketed only after a reimbursement price has been agreed. Some countries may require the completion of additional studies that compare the cost-effectiveness of a particular product

candidate to currently available therapies. For example, the European Union provides options for its member states to restrict the range of drug products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. European Union member states may approve a specific price for a drug product or may instead adopt a system of direct or indirect controls on the profitability of us placing the drug product on the market. Other member states allow companies to fix their own prices for drug products, but monitor and control company profits. The downward pressure on health care costs in general, particularly prescription drugs, has become very intense. As a result, increasingly high barriers are being erected to the entry of new products. In addition, in some countries, cross-border imports from low-priced markets exert competitive pressure that may reduce pricing within a country. There can be no assurance that any country that has price controls or reimbursement limitations for drug products will allow favorable reimbursement and pricing arrangements for any of our products.

The marketability of any products for which we may receive regulatory approval for commercial sale may suffer if the government and third-party payors fail to provide adequate coverage and reimbursement. In addition, an increasing emphasis on managed care in the United States has increased and we expect will continue to increase the pressure on drug pricing. Coverage policies, third-party reimbursement rates and drug pricing regulation may change at any time. In particular, the Patient Protection and Affordable Care Act was enacted in the United States in March 2010 and contains provisions that may reduce the profitability of drug products, including, for example, increased rebates for drugs sold to Medicaid programs, extension of Medicaid rebates to Medicaid managed care plans, mandatory discounts for certain Medicare Part D beneficiaries and annual fees based on pharmaceutical companies' share of sales to federal health care programs. Even if favorable coverage and reimbursement status is attained for one or more products for which we receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

Competition

The pharmaceutical and biotechnology industry is very competitive and the development and commercialization of new drugs and biologics is influenced by rapid technological developments and innovation. We face competition from several companies developing and commercializing products that will be competitive with our drug candidates, including large pharmaceutical and smaller biotechnology companies. Additionally, new entrants may potentially enter the market. For our HBV-cure program, potential competitors include Johnson & Johnson, Roche, Merck & Co., GlaxoSmithKline PLC, Gilead Sciences Inc., Enanta Pharmaceuticals, Inc. and Arbutus Biopharma Corp., among others. Additionally, we may face competition from currently available treatments for HBV. For our Microbiome program, our competitors include Johnson & Johnson, Novartis International AG, Abbvie Inc., Takeda, Merck & Co., Bristol Myers Squibb Co., Pfizer Inc., Seres Therapeutics, Inc., Vedanta Biosciences, Inc., Finch Therapeutics, Inc., Enterome Bioscience S.A. and Second Genome, Inc. Some of the competitive development programs from these companies may be based on scientific approaches that are similar to our approach, and others may be based on entirely different approaches. Potential competitors also include academic institutions, government agencies and other public and private research organizations that conduct research, seek patent protection and establish collaborative arrangements for research, development, manufacturing and commercialization of products similar to ours or that otherwise target indications that we are pursuing.

Manufacturing

We do not currently own or operate any manufacturing facilities. We currently rely on third parties for the manufacture of our product candidates for non-clinical and clinical testing. We currently have no plans to establish any manufacturing facilities for products for our HBV-cure program. In our Microbiome program, we have the capability of performing small scale manufacturing to supply quantities of our microbiome drug substance and drug product for use in our planned nonclinical studies and early phase clinical trials and will use such capability in conjunction with third-party manufacturers to meet the needs of our planned nonclinical studies and early phase clinical trials. As we advance our Microbiome programs through clinical development and potential commercialization, we expect to expand our own manufacturing capabilities for drug substance and drug product for our Microbiome program.

Financial Information

We have not derived any revenue from product sales to date as we currently have no products approved for sale.

Research and Development Expense

Our research and development expenses, excluding stock-based compensation expense, were approximately $38.8 million for fiscal year 2017, of which $23.2 million was expended on the HBV-cure program and $15.6 million was expended on our Microbiome program.

Our research and development expenses, excluding stock-based compensation expense, were approximately $30.1 million for fiscal year 2016, of which $20.0 million was expended on the HBV-cure program and $10.0 million was expended on our Microbiome program.

Our research and development expenses, excluding stock-based compensation expense, were approximately $15.1 million for fiscal year 2015, of which $10.8 million was expended on the HBV-cure program and $4.3 million was expended on our Microbiome program, offset by $6,621 credit due to termination of the VEN 307 study in 2014.

Employees

As of December 31, 2017, we had 79 employees, nine temporary contractors and various consultants and multiple research contract research organizations with whom we have contracted.

Corporate History

We were incorporated in Delaware in October 2005 under the name South Island Biosciences, Inc. (which was changed to Ventrus Biosciences, Inc. in April 2007). On July 11, 2014, we acquired Assembly Pharmaceuticals, Inc., a private company, through a merger with our wholly owned subsidiary (the Merger). In connection with the Merger, we changed our name from Ventrus Biosciences, Inc. to Assembly Biosciences, Inc.

Corporate Information

Our principal executive office is at 11711 N. Meridian Street, Suite 310, Carmel, Indiana 46032. Our telephone number is (317) 210-9311.

Available Information

Our website address is www.assemblybio.com. We routinely post, or have posted, important information for investors on our website in the "Investor Relations" section. We use this website as a means of disclosing material information in compliance with our disclosure obligations under Regulation FD. Accordingly, investors should monitor the "Investor Relations" section of our website, in addition to following our press releases, SEC filings, presentations and webcasts. We make available free of charge through our website our press releases, Annual Report on Form 10-K, Quarterly Reports on Form 10-Q, Current Reports on Form 8-K and all amendments to those reports as soon as reasonably practicable after electronically filed with or furnished to the Securities and Exchange Commission.

The information contained on our website is not a part of, and should not be construed as being incorporated by reference, into this report.

The reports filed with the SEC by us and by our officers, directors and significant shareholders are available for review on the SEC's website at www.sec.gov. You may also read and copy materials that we filed with the SEC at the SEC's Public Reference Room at 100 F Street, NE, Washington, DC 20549. You may obtain information on the operation of the Public Reference Room by calling the SEC at 1-800-SEC-0330.

Information about Segments and Geographic Areas

In accordance with *The Financial Accounting Standards Board (FASB) Accounting Standards Codification (ASC) Topic 280, Segment Reporting,* we have determined that we operate as one operating segment. Decisions regarding our overall operating performance and allocation of our resources are assessed on a consolidated basis. Our operations and assets are predominantly located in the United States.

Item 1A. Risk Factors

This report contains forward-looking statements that involve risks and uncertainties. Our actual results could differ materially from those discussed in this report. Factors that could cause or contribute to these differences include, but are not limited to, those discussed below and elsewhere in this report and in any documents incorporated in this report by reference.

You should carefully consider the following risk factors, together with all other information in this report, including our financial statements and notes thereto, and in our other filings with the Securities and Exchange Commission. If any of the following risks, or other risks not presently known to us or that we currently believe to not be significant, develop into actual events, then our business, financial condition, results of operations or prospects could be materially adversely affected. If that happens, the market price of our common stock could decline, and stockholders may lose all or part of their investment.

Risks Related to Our Business

We have no approved products and currently are dependent on the future success of our HBV-cure and Microbiome programs.

To date, we have no approved products on the market and have generated no product revenues. Our prospects are substantially dependent on our ability to develop and commercialize our HBV and microbiome product candidates. Unless and until we receive approval from the FDA or other regulatory authorities for our product candidates, we cannot sell our product candidates and will not have product revenues. We will have to fund all of our operations and capital expenditures from cash on hand, any future securities offerings or debt financings and any fees we may generate from out-licensing, collaborations or other strategic arrangements. If we are unable to develop and commercialize any product candidates from our HBV-cure and Microbiome programs, we will be unable to generate revenues or build a sustainable or profitable business.

In addition, all of our product candidates are currently in early clinical development or in varying stages of nonclinical development and their risk of failure is high. The data supporting our drug discovery and nonclinical and clinical development programs are derived from either laboratory, nonclinical studies or Phase 1a clinical data. We cannot predict when or if any one of our product candidates will prove safe and effective in humans or will receive regulatory approval. The scientific evidence to support the feasibility of our product candidates and therapeutic approaches is limited, and many companies, some with more resources than we have, are and may be developing competitive product candidates. For these and other reasons, our drug discovery and development may not be successful, and we may not generate viable products or revenue.

We depend entirely on the success of product candidates from our HBV-cure program and our Microbiome program. We cannot be certain that we or our collaborators will be able to obtain regulatory approval for, or successfully commercialize, product candidates from either of our current programs or any other product candidates we may subsequently identify.

We and our collaborators are not permitted to market or promote any product candidates in the United States, Europe or other countries before we receive regulatory approval from the FDA or comparable foreign regulatory authorities, and we may never receive such regulatory approval for our current product candidates. We have not submitted a BLA or NDA to the FDA or comparable applications to other regulatory authorities and do not expect to be in a position to do so in the foreseeable future.

All of our product candidates are currently in early clinical development or in varying stages of nonclinical development. It may be years before the larger, pivotal trials necessary to support regulatory approval of our product candidates are initiated, if ever. The clinical trials of our product candidates are, and the manufacturing and marketing of our product candidates will be, subject to extensive and rigorous review and regulation by numerous government authorities in the United States and in other countries where we intend to test and, if approved, market any product candidate. Before obtaining regulatory approvals for the commercial sale of any product candidate, we must successfully meet a number of critical developmental milestones, including:

- developing dosages that will be tolerated, safe and effective;
- reaching agreement with the FDA or comparable foreign regulatory authorities regarding the scope, design and data necessary to support regulatory approval for the product candidate;
- demonstrating through clinical trials that the product candidate is safe and effective in patients for the intended indication;
- determining the appropriate delivery mechanism;
- demonstrating that the product candidate formulation will be stable for commercially reasonable time periods; and
- completing the development and scale-up to permit manufacture of our product candidates in quantities sufficient to execute on our clinical development plans and, eventually, in commercial quantities and at acceptable prices.

The time necessary to achieve these developmental milestones for any individual product candidate is long and uncertain, and we may not successfully complete these milestones for our HBV and microbiome therapies or any other product candidates that we may develop. We have not yet completed and may never complete the development of any products. If we are unable to complete clinical development of our HBV or microbiome therapies, or any other product candidates that we may identify, we will be unable to generate revenue or build a sustainable or profitable business.

Nonclinical studies may not be representative of disease behavior in clinical trials. The outcomes of nonclinical testing and clinical trials are uncertain, and results of earlier nonclinical studies and clinical trials may not be predictive of future clinical trial results.

The results of nonclinical studies may not be representative of disease behavior in a clinical setting and thus may not be predictive of the outcomes of our clinical trials. In addition, the results of nonclinical studies and early clinical trials of product candidates may not be predictive of the results of later-stage clinical trials, and the results of any study or trial for any of our product candidates may not be as positive as the results for any prior studies or trials, if at all.

Nonclinical studies and clinical testing are expensive, can take many years to complete and their outcomes are highly uncertain. Failure can occur at any time during the nonclinical study and clinical trial processes due to inadequate performance of a drug candidate or inadequate adherence by patients or investigators to clinical trial protocols. Further, clinical trials might not provide statistically significant data supporting a product candidate's safety and effectiveness to obtain the requisite regulatory approvals. In addition, there is a high failure rate for drugs and biologics proceeding through clinical trials. Our failure to replicate earlier positive results in later-stage clinical trials or otherwise demonstrate the required characteristics to support marketing approval for any of our product candidates would substantially harm our business, prospects, financial condition and results of operations.

Top-line or interim data may not accurately reflect the complete results of a particular study or trial.

We may publicly disclose top-line or interim data from time to time, which is based on a preliminary analysis of then-available efficacy and safety data, and the results and related findings and conclusions are subject to change following a more comprehensive review of the data related to the particular study or trial. We also make assumptions, estimations, calculations and conclusions as part of our analyses of data, and we may not have received or had the opportunity to evaluate fully and carefully all data. As a result, the

top-line or interim results that we report may differ from future results of the same studies, or different conclusions or considerations may qualify such results, once additional data have been received and fully evaluated. Top-line data also remain subject to audit and verification procedures that may result in the final data being materially different from the interim or preliminary data we previously published. As a result, top-line and interim data should be viewed with caution until the final data are available.

Further, others, including regulatory agencies, may not accept or agree with our assumptions, estimations, calculations, conclusions or analyses or may interpret or weigh the importance of data differently, which could impact the value of the particular program, the approvability or commercialization of the particular drug candidate or biotherapeutic and our company in general. In addition, the information we may publicly disclose regarding a particular study or clinical trial is based on what is typically extensive information, and you or others may not agree with what we determine is the material or otherwise appropriate information to include in our disclosure, and any information we determine not to disclose may ultimately be deemed significant with respect to future decisions, conclusions, views, activities or otherwise regarding a particular drug, drug candidate or our business. If the top-line or interim data that we report differ from actual results, or if others, including regulatory authorities, disagree with the conclusions reached, our ability to obtain approval for, and commercialize, our product candidates may be harmed or delayed, which could harm our business, financial condition, operating results or prospects.

Nonclinical and clinical testing required for our product candidates is expensive and time-consuming and may result in delays or may fail to demonstrate safety and efficacy for desired indications.

In order to obtain FDA approval to market a new drug product, we must demonstrate safety and effectiveness in humans. To meet these requirements, we must conduct extensive nonclinical testing and sufficient adequate and well-controlled clinical trials. Conducting clinical trials is a lengthy, time consuming, and expensive process. The length of time might vary substantially according to the type, complexity, novelty, and intended use of the product candidate, and often can be several years or more per trial. Delays associated with product candidates for which we are directly conducting nonclinical studies or clinical trials might cause us to incur additional operating expenses. The commencement and rate of completion of clinical trials might be delayed by many factors, including, for example:

- delays in reaching agreement with regulatory authorities on trial design;
- delays in reaching agreement on acceptable terms with prospective contract research organizations (CROs) and clinical trial sites;
- the lack of effectiveness during clinical trials;
- the emergence of unforeseen safety issues;
- inability to manufacture sufficient quantities of qualified materials under cGMP for use in clinical trials;
- slower than expected rates of patient recruitment;
- failure to recruit a sufficient number of patients;
- delays in having patients complete participation in a trial or return for post-treatment follow-up;
- delays caused by patients dropping out of a trial due to product side effects, disease progression or other reasons;
- clinical sites dropping out of a trial to the detriment of enrollment;
- modification of clinical trial protocols;
- delays by our contract manufacturers to produce and deliver sufficient supply of clinical trial materials;
- occurrence of adverse events associated with the product candidate that are viewed to outweigh its potential benefits;
- changes in regulatory requirements for clinical trials;

- delays, suspension, or termination of clinical trials by the institutional review board or ethics committee responsible for overseeing the study at a particular study site; and

- government, institutional review board, ethics committee, or other regulatory delays or clinical holds requiring suspension or termination of the trials.

We have used and intend to continue to rely on one or more CROs to conduct our nonclinical studies and clinical trials. We are highly dependent on these CROs to conduct our studies and trials in accordance with the requirements of the FDA, applicable local laws and good clinical and scientific practice. In the event the CROs fail to perform their duties in such a fashion, we may not be able to complete our clinical trials and may fail to obtain regulatory approval for any of our product candidates.

The failure of nonclinical studies and clinical trials to demonstrate safety and effectiveness of a product candidate for the desired indications could harm the development of that product candidate or other product candidates. This failure could cause us to abandon a product candidate and could delay development of other product candidates. Any delay in, or termination of, our nonclinical studies or clinical trials would delay the filing of our NDAs or BLAs with the FDA and, ultimately, our ability to commercialize our product candidates and generate product revenues. Any change in, or termination of, our clinical trials could materially harm our business, financial condition, and results of operation.

Any product candidates that we may discover and develop may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval, limit the commercial profile of an approved label, or result in significant negative consequences following marketing approval, if any.

Many product candidates that initially showed promise in early stage testing have later been found to cause side effects that prevented their further development. Undesirable side effects caused by any product candidates that we may discover or develop, or safety, tolerability or toxicity issues that may occur in our nonclinical studies, clinical trials or in the future, could cause us or regulatory authorities to interrupt, restrict, delay, or halt clinical trials. Such results could also cause us to, or regulatory authorities to require us to, cease further development of our product candidates for any or all targeted indications. The drug-related side effects could affect patient recruitment or the ability of enrolled patients to complete the trial or result in potential product liability claims, and could result in a more restrictive label or the delay or denial of regulatory approval by the FDA or other comparable foreign authorities. Any of these events could prevent us from achieving or maintaining market acceptance of the particular product candidate, if approved, and could significantly harm our business, prospects, financial condition and results of operations. In the Phase 1a portion of the trial recently completed in New Zealand, the most common treatment-emergent adverse events that we observed were headaches and rashes, which were among the only adverse events deemed by clinical investigators to be probably or possibly related to the study drug.

Additionally, if any of our product candidates receives marketing approval and we or others later identify undesirable or unacceptable side effects caused by these product candidates, a number of potentially significant negative consequences could result, including:

- regulatory authorities may withdraw approvals of such product and require us to take them off the market;

- regulatory authorities may require the addition of labeling statements, specific warnings, a contraindication or field alerts to physicians and pharmacies;

- regulatory authorities may require a medication guide outlining the risks of such side effects for distribution to patients, or that we implement a REMS plan to ensure that the benefits of the product outweigh its risks;

- we may be required to change the way a product is administered, conduct additional clinical trials or change the labeling of a product;

- we may be subject to limitations on how we may promote the product;

- sales of the product may decrease significantly;

- we may be subject to litigation or product liability claims; and
- our reputation may suffer.

Any of these events could prevent us or any collaborators from achieving or maintaining market acceptance of our product candidates or could substantially increase commercialization costs and expenses, which in turn could delay or prevent us from generating significant revenue from the sale of our product candidates.

We have a limited operating history and a history of operating losses, and expect to incur significant additional operating losses.

We were established in October 2005, began active operations in the spring of 2007, terminated programs related to three prior product candidates, then merged with Assembly Pharmaceuticals, Inc. (Assembly Pharmaceuticals), a private company, in July 2014. We have only a limited operating history since the merger. Therefore, there is limited historical financial information upon which to base an evaluation of our performance. Our prospects must be considered in light of the uncertainties, risks, expenses, and difficulties frequently encountered by companies in their early stages of operations. We, and Assembly Pharmaceuticals prior to our merger, have generated losses since we began operations and, as of December 31, 2017 and December 31, 2016, the combined company had an accumulated deficit of approximately $251.0 million and $208.2 million, respectively, and net losses of approximately $42.8 million, $44.3 million and 28.5 million for the years ended December 31, 2017, 2016 and 2015, respectively. These net losses have had, and will continue to have, an adverse effect on our stockholders' equity and working capital. We expect to incur substantial additional losses over the next several years as we continue to pursue our research, development, nonclinical studies and clinical trial activities. Further, since our initial public offering, we have incurred and will continue to incur as a public company significant additional legal, accounting and other expenses to which we were not subject to as a private company, including expenses related to our efforts in complying with the requirements of the Sarbanes-Oxley Act of 2002 (the Sarbanes-Oxley Act), the Dodd-Frank Wall Street Reform and Consumer Protection Act of 2010 and other public company disclosure and corporate governance requirements and responding to requests of government regulators. The amount of future losses and when, if ever, we will achieve profitability are uncertain and will depend, in part, on the rate of increase in our expenses, our ability to generate revenues and our ability to raise additional capital. We have no products that have generated any commercial revenue, do not expect to generate revenues from the commercial sale of products unless and until our HBV or microbiome therapies or any other product candidate is approved by the FDA for sale, and we might never generate revenues from the sale of products.

We are not currently profitable and might never become profitable.

We have a history of losses and expect to incur significant operating and capital expenditures and resultant substantial losses and negative operating cash flow for the next several years and beyond if we do not successfully launch and commercialize any product candidates from our HBV or microbiome programs. We might never achieve or maintain profitability. We anticipate that our expenses will continue to be substantial in the foreseeable future as we:

- advance ABI-H0731, our first HBV-cure candidate, through clinical development and conduct nonclinical studies and clinical trials of ABI-H2158, our second HBV-cure product candidate;
- continue to undertake research and development to identify potential additional product candidates in both our HBV-cure and Microbiome programs;
- seek regulatory approvals for our product candidates; and
- pursue our intellectual property strategy.

Because of the numerous risks and uncertainties associated with pharmaceutical product development, we are unable to accurately predict the timing or amount of increased expenses or when, or if, we will be able to achieve profitability. In addition, our expenses could increase if we are required by the FDA or comparable foreign regulatory authorities to perform studies or trials in addition to those currently expected, or if there are any delays in completing our clinical trials or the development of any of our product candidates.

As a result, we will need to generate significant revenues in order to achieve and maintain profitability. Our ability to generate revenue and achieve profitability will depend on, among other things:

- successful completion of research, nonclinical studies and clinical trials for our product candidates;
- obtaining necessary regulatory approvals from the FDA and comparable foreign regulatory authorities for our product candidates;
- maintaining patent protection for our products, methods, processes and technologies;
- establishing manufacturing, sales, and marketing arrangements internally and/or with third parties for any approved products; and
- raising sufficient funds to finance our activities, if and when needed.

We might not succeed at any of these undertakings. If we are unsuccessful at some or all of these undertakings, our business, prospects, and results of operations might be materially adversely affected.

We are an early stage company and might not be able to commercialize any product candidates.

We are an early stage company and have not demonstrated our ability to perform the functions necessary for the successful commercialization of any product candidates. The successful commercialization of any product candidates will require us to perform a variety of functions, including:

- continuing to undertake research and development and nonclinical studies and clinical trials;
- participating in regulatory approval processes;
- formulating and manufacturing products; and
- conducting sales, marketing and distribution activities.

Our failure to commercialize successfully our product candidates would negatively impact the value of our company and could impair our ability to raise capital, expand our business, diversify our research and development pipeline, market our product candidates, if approved, or continue our operations.

Our development of product candidates is subject to risks and delays.

Our development of our product candidates is subject to the risks of failure and delay inherent in the development of new pharmaceutical products and products based on new technologies, including:

- delays in product development, nonclinical and clinical testing;
- unplanned expenditures in product development, nonclinical and clinical testing;
- failure of a product candidate to demonstrate acceptable safety and efficacy;
- failure to receive regulatory approvals;
- emergence of superior or equivalent products;
- inability to manufacture and sell on our own, or through any others, product candidates on a commercial scale or at a financially viable cost; and
- failure to achieve market acceptance.

Because of these risks, our research and development efforts might not result in any commercially viable products. If we do not successfully complete a significant portion of these development efforts, obtain required regulatory approvals, and have commercial success with any approved products, our business, financial condition and results of operations will be materially harmed.

There are substantial risks inherent in attempting to commercialize new drugs and biologics, and, as a result, we may not be able to develop successfully products for commercial use.

Scientific research and development requires significant amounts of capital and takes a long time to reach commercial viability, if it can be achieved at all. To date, our research and development projects have not produced commercially viable drugs or biologics and may never do so. During the research and development process, we may experience technological barriers that we may be unable to overcome. Further, certain underlying premises in our development programs are not fully proven.

Our HBV therapy research and development efforts involve therapeutics based on modulating forms of HBV core proteins with Core protein Allosteric Modifiers (CpAMS). The development of our CpAM technology is in early stages, and the commercial feasibility and acceptance of our CpAMs technology is unknown. More specifically, the theory that treatment with CpAMs may result in the loss of covalently closed circular DNA (cccDNA) compared to conventional (standard of care) therapies is unproven. It is also unknown if the biomarkers assumed to be indicators of active cccDNA (serum viral antigen levels in HBV patients) will be meaningfully altered in patients on treatment with CpAMS. Additionally, even if CpAM technology is successful at targeting the HBV core protein and treatment is successful at reducing cccDNA levels in HBV patients, it may not result in a commercially viable drug if there is not a corresponding medical benefit related to the underlying HBV infection.

Similarly, our Microbiome program is based on a novel therapeutic approach designed to treat disorders associated with the microbiome. To our knowledge, no companies have received regulatory approval for, or manufactured on a commercial scale, any microbiome-based therapeutics. The technology for our microbiome therapy is in nonclinical development and our GEMICEL® dual-targeted release capsule formulation is novel and has not yet shown to deliver successfully live bacteria in patients. The ability to deliver bacteria effectively and reliably to the GI tract is unproven, and, even if it can be proven, it may be difficult or impossible to provide the treatment economically. Because of these uncertainties, it is possible that no commercial products will be successfully developed. If we are unable to develop successfully commercial products, we will be unable to generate revenue or build a sustainable or profitable business.

We will need additional financing to complete the development of any product candidate and fund our activities in the future.

We anticipate that we will incur operating losses for the next several years as we continue to develop our HBV product candidates and our microbiome platform as well as initiate any development of any other product candidates and will require substantial funds during that time to support our operations. We expect that our current resources will provide us with sufficient capital to fund our operations for at least the next twelve months. However, we might consume our available capital before that time if, for example, we are not efficient in managing our resources or if we encounter unforeseen costs, delays or other issues or if regulatory requirements change. If that happens, we may need additional financing to continue the development of our HBV product candidates and our Microbiome program. There is no assurance that we will be able to generate sufficient revenue from our Collaboration Agreement with Allergan when needed to or that we will be successful in raising any necessary additional capital on terms that are acceptable to us, or at all. If such event or other unforeseen circumstances occurred and we were unable to generate revenue or raise capital, we could be forced to delay, scale back or discontinue product development, sacrifice attractive business opportunities, cease operations entirely and sell or otherwise transfer all or substantially all of our remaining assets.

Our product candidates face significant development and regulatory hurdles prior to marketing, which could delay or prevent our receipt of licensing, sales and/or milestone revenue.

Before we or any commercial partners obtain the approvals necessary to sell any of our product candidates, we must show through nonclinical studies and human testing in clinical trials that each potential product is safe and effective. The rates at which we complete our scientific studies and clinical trials depend on many factors, including, but not limited to, our ability to obtain adequate supplies of the products to be tested and patient enrollment. Patient enrollment is a function of many factors, including the size of the patient population, the proximity of patients to clinical sites, the eligibility criteria for the trial, the design of

the clinical trial, and other potential drug candidates being studied. Delays in patient enrollment for our trials may result in increased costs and longer development times. In addition, we will need additional financing to develop our product candidates, which we might seek and receive from third-party commercial partners. Further, we currently do not have the infrastructure to manufacture, market and sell our product candidates. If we partner with one or more third-party entities, those commercial partners may demand and receive rights to control product development and commercialization. As a result, these commercial partners may conduct these programs and activities more slowly or in a different manner than expected. If any of these events were to occur, the development of any product candidate could be significantly delayed, more expensive or less lucrative to us than anticipated, any of which would have a significant adverse effect on our business.

We are substantially dependent on our Collaboration Agreement with Allergan, which may be terminated or may not be successful due to a number of factors, which could have a material adverse effect on our business and operating results.

On January 6, 2017, we entered into the Collaboration Agreement with Allergan for the development and commercialization of select microbiome gastrointestinal programs in ulcerative colitis, Crohn's disease and irritable bowel syndromes. Our collaboration with Allergan may be terminated, or may not be successful, due to a number of factors. In particular, Allergan may terminate the Collaboration Agreement for convenience at any time upon either 90 days' (prior to the initiation of the first proof of concept (POC) trial of a licensed product) or 120 days' (after the initiation of the first POC trial of a licensed product), as applicable, advance written notice to us. The Collaboration Agreement also contains customary provisions for termination by either party, including in the event of breach of the Collaboration Agreement, subject to cure. In addition, if we are unable to identify product candidates for the licensed indications or we are unable to protect our products by obtaining and defending patents, the collaboration could fail. If the collaboration is unsuccessful for these or other reasons, or is otherwise terminated for any reason, we may not receive all or any of the research program funding, milestone payments or royalties under the agreement. Any of the foregoing could result in a material adverse effect on our business, results of operations and prospects and would likely cause our stock price to decline.

We are dependent on a license relationship for each of our HBV-cure program and our Microbiome program.

Our license agreement with Indiana University Research and Technology Corporation (IURTC) from whom we have licensed ABI-H0731 and certain other HBV therapies, requires us to make milestone payments based upon the successful accomplishment of clinical and regulatory milestones related to ABI-H0731 and certain other HBV therapies. The aggregate amount of all performance milestone payments under the IURTC License Agreement, should all performance milestones through development be met, is $825,000. As of December 31, 2017, no performance milestone payments have been made. We also are obligated to pay IURTC royalty payments based on net sales of the licensed technology. We are also obligated to pay diligence maintenance fees ($25,000-$100,000) each year to the extent that the royalty, sublicensing, and milestone payments to IURTC are less than the diligence maintenance fee for that year. Our license with Therabiome, LLC (Therabiome), from whom we have licensed our delivery platform of our Microbiome program, also requires us to pay regulatory and clinical milestones as well as royalty payments to Therabiome. If we breach any of these obligations, we could lose our rights to the targeted delivery mechanism of our Microbiome program. If we fail to comply with similar obligations to any other licensor, then that licensor would have the right to terminate the license, in which event we would not be able to commercialize drug candidates or technologies that were covered by the license. In addition, the milestone and other payments associated with licenses will make it less profitable for us to develop our drug candidates than if we owned the technology ourselves.

Corporate and academic collaborators might take actions to delay, prevent, or undermine the success of our product candidates.

Our operating and financial strategy for the development, nonclinical and clinical testing, manufacture, and commercialization of drug candidates heavily depends on collaborating with corporations, academic institutions, licensors, licensees, and other parties. However, there can be no assurance that we will successfully establish these collaborations. In addition, should a collaboration be terminated, replacement

collaborators might not be available on attractive terms, or at all. The activities of any collaborator will not be within our control and might not be within our power to influence. There can be no assurance that any collaborator will perform its obligations to our satisfaction or at all, that we will derive any revenue or profits from these collaborations, or that any collaborator will not compete with us. If any collaboration is not successful, we might require substantially greater capital to undertake development and marketing of our proposed products and might not be able to develop and market these products effectively, if at all. In addition, a lack of development and marketing collaborations might lead to significant delays in introducing proposed products into certain markets and/or reduced sales of proposed products in such markets.

We rely on data provided by our collaborators and others that has not been independently verified and could prove to be false, misleading, or incomplete.

We rely on third-party vendors, scientists, and collaborators to provide us with significant data and other information related to our projects, nonclinical studies and clinical trials, and our business. If these third parties provide inaccurate, misleading, or incomplete data, our business, prospects, and results of operations could be materially adversely affected.

Research, development and commercialization goals may not be achieved in the time frames that we publicly estimate, which could have an adverse impact on our business and could cause our stock price to decline.

We set goals, and make public statements regarding our expectations, regarding the timing of certain accomplishments, developments and milestones under our research and development programs. The actual timing of these events can vary significantly due to a number of factors, including, without limitation, the amount of time, effort and resources committed to our programs by us and any collaborators and the uncertainties inherent in the clinical development and regulatory approval process. As a result, there can be no assurance that we or any collaborators will initiate or complete clinical development activities, make regulatory submissions or receive regulatory approvals as planned or that we or any collaborators will be able to adhere to our current schedule for the achievement of key milestones under any of our programs. If we or any collaborators fail to achieve one or more of the milestones as planned, our business could be materially adversely affected, and the price of our common stock could decline.

We lack suitable facilities for certain nonclinical and clinical testing and expect to rely on third parties to conduct some of our research and nonclinical testing and our clinical trials, and those third parties may not perform satisfactorily, including failing to meet deadlines for the completion of such research, testing or trials.

We do not have sufficient facilities to conduct all of our anticipated nonclinical and clinical testing. As a result, we expect to contract with third parties to conduct a significant portion of our nonclinical and clinical testing required for regulatory approval for our product candidates. We will be reliant on the services of third parties to conduct studies on our behalf. If we are unable to retain or continue with third parties for these purposes on acceptable terms, we may be unable to develop successfully our product candidates. In addition, any failures by third parties to perform adequately their responsibilities may delay the submission of our product candidates for regulatory approval, which would impair our financial condition and business prospects.

Our reliance on these third parties for research and development activities also reduces our control over these activities but will not relieve us of our responsibilities. For example, we are responsible for ensuring that each of our studies is conducted in accordance with the applicable protocol, legal and regulatory requirements and scientific standards, including, in the case of clinical trials, good clinical practices, and our reliance on third parties does not relieve us of our regulatory responsibilities. Furthermore, these third parties may also have relationships with other entities, some of which may be our competitors. In addition, these third parties are not our employees, and except for remedies available to us under our agreements with such third parties, we cannot control whether or not they devote sufficient time and resources to our clinical and nonclinical programs. If these third parties do not successfully carry out their contractual duties or obligations or meet expected deadlines, if they need to be replaced or if the quality or accuracy of the clinical data they obtain is compromised due to the failure to adhere to our clinical protocols, regulatory requirements or for other reasons, our research, nonclinical studies or clinical trials may be extended,

delayed or terminated and we may not be able to obtain, or may be delayed in obtaining, regulatory approvals for our product candidates. As a result, our results of operations and business prospects would be harmed, our costs could increase and our ability to generate revenues could be delayed.

We will need to either establish our own clinical and commercial manufacturing capabilities or rely on third parties to formulate and manufacture our product candidates, and we rely on third parties to manufacture products that we study in combination with our product candidates. Our use of third parties to manufacture these materials may increase the risk that we will not have sufficient quantities of our product candidates or other products, or necessary quantities of such materials on time or at an acceptable cost.

We currently do not have our own manufacturing facilities and rely on third-party manufacturers to supply the quantities of ABI-H0731 used in our clinical trials, the quantities of ABI-H2158 used in our nonclinical studies and drug substance and drug product for our Microbiome program. In the past, we have relied exclusively on third-party manufacturers to supply drug substance and drug product materials for our Microbiome program. We are currently transitioning some of the third-party manufacturing to a small scale internal manufacturing facility to supply quantities of our microbiome drug substance and drug product for use in our planned nonclinical studies and early phase clinical trials. In addition, if any product candidate we might develop or acquire in the future receives FDA or other regulatory approval, we will need to either manufacture commercial quantities of the product on our own or rely on one or more third-party contractors to manufacture our products. The establishment of internal manufacturing capabilities is difficult and costly, and we may not be successful in doing so. If, for any reason, we are unable to establish our own manufacturing capabilities and we are unable to rely on any third-party sources we have identified to manufacture our product candidates, either for clinical trials or, at some future date, for commercial quantities, then we would need to identify and contract with additional or replacement third-party manufacturers to manufacture compounds, drug substance and drug products for nonclinical, clinical and commercial purposes. We might not be successful in identifying additional or replacement third-party manufacturers, or in negotiating acceptable terms with any that we do identify. If we are unable to establish and maintain manufacturing capacity either on our own or through third parties, the development and sales of our products and our financial performance will be materially and adversely affected.

In addition, before we or any of our collaborators can begin to commercially manufacture our product candidates, each manufacturing facility and process is subject to regulatory review. Manufacturing of drugs for clinical and commercial purposes must comply with the FDA's cGMPs and applicable non-U.S. regulatory requirements. The cGMP requirements govern quality control and documentation policies and procedures. Complying with cGMP and non-U.S. regulatory requirements will require that we expend time, money, and effort in production, recordkeeping, and quality control to assure that the product meets applicable specifications and other requirements. Any manufacturing facility must also pass a pre-approval inspection prior to FDA approval. Failure to pass a pre-approval inspection might significantly delay FDA approval of our product candidates. If we or any of our future collaborators fails to comply with these requirements with respect to the manufacture of any of our product candidates, regulatory action could limit the jurisdictions in which we are permitted to sell our products, if approved. As a result, our business, financial condition, and results of operations might be materially harmed.

We are exposed to the following risks with respect to the manufacture of our product candidates:

- If we are unable to establish our own manufacturing capabilities, we will need to identify manufacturers for commercial supply on acceptable terms, which we may not be able to do because the number of potential manufacturers is limited, and the FDA must approve any new or replacement contractor. This approval would generally require compliance inspections. In addition, a new manufacturer would have to be educated in, or develop substantially equivalent processes for, production of our products after receipt of FDA approval, if any.
- We or any third-party manufacturers with whom we contract might be unable to formulate and manufacture our product candidates in the volume and of the quality required to meet our clinical and, if approved, commercial needs in a timely manner.
- Any third-party manufacturers with whom we contract might not perform as agreed or might not remain in the contract manufacturing business for the time required to supply our clinical trials or to produce, store and distribute successfully our products.

- One or more of any third-party manufacturers with whom we contract could be foreign, which increases the risk of shipping delays and adds the risk of import restrictions.
- Drug manufacturers are subject to ongoing periodic unannounced inspection by the FDA and corresponding state agencies to ensure strict compliance with cGMP and other government regulations and corresponding foreign requirements. Any internal manufacturing facilities we establish may fail to comply, and we would not have complete control over any third-party manufacturers' compliance, with these regulations and requirements.
- We may be required to obtain additional intellectual property rights from third parties in order to manufacture our product candidates, and if any third-party manufacturer makes improvements in the manufacturing process for our product candidates, we might not own, or might have to share, the intellectual property rights to the innovation with our licensors.
- We may be required to share our trade secrets and know-how with third parties, thereby risking the misappropriation or disclosure of our intellectual property by or to third parties.
- If we contract with third-party manufacturers, we might compete with other companies for access to these manufacturers' facilities and might be subject to manufacturing delays if the manufacturers give other clients higher priority than us.

Each of these risks could delay our development efforts, nonclinical studies and clinical trials or the approval, if any, of our product candidates by the FDA or the commercialization of our product candidates and could result in higher costs or deprive us of potential product revenues. As a result, our business, financial condition, and results of operations might be materially harmed.

If we or our collaborators cannot compete successfully for market share against other companies, we might not achieve sufficient product revenues and our business will suffer.

If our product candidates receive FDA approval, they will compete with a number of existing and future drugs and biologics developed, manufactured and marketed by others. Existing or future competing drugs might provide greater therapeutic convenience or clinical or other benefits for a specific indication than our product candidates, or might offer comparable performance at a lower cost. If our product candidates fail to capture and maintain market share, we might not achieve sufficient product revenues and our business will suffer.

We might compete against fully integrated pharmaceutical or biotechnology companies and smaller companies that are collaborating with larger pharmaceutical companies, academic institutions, government agencies and other public and private research organizations. Many of these competitors, either alone or together with their collaborative partners, operate larger research and development programs or have substantially greater financial resources than we do, as well as significantly greater experience in:

- developing drugs;
- undertaking nonclinical testing and human clinical trials;
- obtaining FDA and other regulatory approvals of drugs;
- formulating and manufacturing drugs; and
- launching, marketing and selling drugs.

We may not have or be able to obtain the same resources and experience as our competitors. If we are unable to perform these tasks effectively and efficiently, our results of operations might be materially adversely affected.

Developments by competitors might render our product candidates or technologies obsolete or non-competitive.

The pharmaceutical and biotechnology industries are intensely competitive. In addition, the clinical and commercial landscape for HBV, ulcerative colitis (UC), inflammatory bowel disease (IBD), including Crohn's disease, irritable bowel syndrome (IBS), nonalcoholic steatohepatitis disease (NASH), immuno-oncology and c. difficile infections (CDI) is rapidly changing; we expect new data from commercial

and clinical-stage products to continue to emerge. We will compete with organizations that have existing treatments and that are or will be developing treatments for the indications that our product candidates target. If our competitors develop effective treatments for HBV, UC, IBD, IBS, NASH, immuno-oncology and CDI or any other indication or field we might pursue, and successfully commercialize those treatments, our business and prospects might be materially harmed, due to intense competition in these markets.

Our product candidates under development in our Microbiome program will be subject to regulation as biologics. These candidates, and any other future product candidates for which we or our collaborators intend to seek approval as biologic products, may face competition sooner than anticipated.

The Patient Protection and Affordable Care Act (ACA) signed into law on March 23, 2010, includes a subtitle called the Biologics Price Competition and Innovation Act of 2009 (BPCIA), which created an abbreviated approval pathway for biological products that are biosimilar to or interchangeable with an FDA-licensed reference biological product. Under the BPCIA, an application for a biosimilar product may not be submitted to the FDA until four years following the date that the reference product was first licensed by the FDA. In addition, the approval of a biosimilar product may not be made effective by the FDA until 12 years from the date on which the reference product was first licensed. During this 12-year period of exclusivity, another company may still market a competing version of the reference product if the FDA approves a full BLA for the competing product containing the sponsor's own preclinical data and data from adequate and well-controlled clinical trials to demonstrate the safety, purity and potency of their product. The law is complex and is still being interpreted and implemented by the FDA. As a result, its ultimate impact, implementation, and meaning are subject to uncertainty. While it is uncertain when such processes intended to implement the BPCIA may be fully adopted by the FDA, any such processes could have a material adverse effect on the future commercial prospects for our biological products.

We believe that if product candidates from our Microbiome program are approved as biological products under a BLA, they should qualify for the 12-year period of exclusivity. However, there is a risk that this exclusivity could be shortened due to congressional action or otherwise, or that the FDA will not consider our product candidates to be reference products for competing products, potentially creating the opportunity for generic competition sooner than anticipated. Other aspects of the BPCIA, some of which may impact the BPCIA exclusivity provisions, have also been the subject of recent litigation. Moreover, the extent to which a biosimilar, once approved, will be substituted for any one of our reference products in a way that is similar to traditional generic substitution for non-biological products is not yet clear, and will depend on a number of marketplace and regulatory factors that are still developing.

If we or our collaborators are not able to develop collaborative marketing relationships with licensees or partners, or create effective internal sales, marketing, and distribution capability, we might be unable to market our products successfully.

To market our product candidates, if approved, we will have to establish our own marketing and sales force or out-license our product candidates to, or collaborate with, larger firms with experience in marketing and selling pharmaceutical products. There can be no assurance that we will be able to successfully establish our own marketing capabilities or establish marketing, sales, or distribution relationships with third parties; that such relationships, if established, will be successful; or that we will be successful in gaining market acceptance for our product candidates. To the extent that we enter into any marketing, sales, or distribution arrangements with third parties, our product revenues will be lower than if we marketed and sold our products directly, and any revenues we receive will depend upon the efforts of such third parties. If we are unable to establish such third-party sales and marketing relationships, or choose not to do so, we will have to establish our own in-house capabilities. We, as a company, have no experience in marketing or selling pharmaceutical products and currently have no sales, marketing, or distribution infrastructure. To market any of our products directly, we would need to develop a marketing, sales, and distribution force that both has technical expertise and the ability to support a distribution capability. To establish our own marketing, sales, and distribution capacity would significantly increase our costs, and require substantial additional capital. In addition, there is intense competition for proficient sales and marketing personnel, and we might not be able to attract individuals who have the qualifications necessary to market, sell, and distribute our products. There can be no assurance that we will be able to establish internal marketing, sales, or distribution capabilities.

The commercial success of our product candidates will depend upon the degree of market acceptance by physicians, patients, third-party payers and others in the medical community.

The commercial success of our products, if approved for marketing, will depend in part on the medical community, patients and third-party payers accepting our product candidates as effective and safe. If these products do not achieve an adequate level of acceptance, we may not generate significant product revenue and may not become profitable. The degree of market acceptance of our products, if approved for marketing, will depend on a number of factors, including:

- the actual or perceived safety and efficacy of the products, and advantages over alternative treatments;
- the pricing and cost-effectiveness of our products relative to competing products or therapies, including generic drugs or biosimilars, if available;
- the labeling of any approved product;
- the prevalence and severity of any side effects, including any limitations or warnings contained in a product's approved labeling;
- the emergence, and timing of market introduction, of competitive products;
- the effectiveness of marketing and distribution efforts by us and our licensees and distributors, if any; and
- the availability of third-party insurance coverage or governmental reimbursement.

Even if a potential product displays a favorable efficacy and safety profile in nonclinical studies and clinical trials, market acceptance of the product will not be known until after it is launched. Any failure to achieve market acceptance for our product candidates will harm our business, results and financial condition.

If we lose key management or scientific personnel, cannot recruit qualified employees, directors, officers, or other significant personnel or experience increases in our compensation costs, our business might materially suffer.

We are highly dependent on the services of our executive officers and senior management team. Our employment agreements with our executive officers and senior management team members do not ensure their retention.

Furthermore, our future success also depends, in part, on our ability to identify, hire, and retain additional management team members as our operations grow and our ability to replace our management team members in the event any leave us for any reason. We expect to experience intense competition for qualified personnel and might be unable to attract and retain the personnel necessary for the development of our business. Finally, we do not currently maintain, nor do we intend to obtain in the future, "key man" life insurance that would compensate us in the event of the death or disability of any of the members of our management team.

The failure by us to retain, attract and motivate executives and other key employees could have a material adverse impact on our business, financial condition and results of operations.

If we are unable to hire additional qualified personnel, our ability to grow our business might be harmed.

As of December 31, 2017, we had 79 employees, nine temporary contractors and various consultants and multiple contract research organizations with whom we have contracted. We will need to hire or contract with additional qualified personnel with expertise in clinical research and testing, formulation and manufacturing and sales and marketing to commercialize our HBV drug candidates and our microbiome biotherapeutic candidates or any other product candidate we may seek to develop. We compete for qualified individuals with numerous biopharmaceutical companies, universities and other research institutions. Competition for these individuals is intense, and we cannot be certain that our search for such personnel will be successful. Attracting and retaining qualified personnel will be critical to our success.

We might not successfully manage our growth.

Our success will depend upon the expansion of our operations and the effective management of our growth, which will place a significant strain on our current and future management and other administrative and operational resources. To manage this growth, we may need to expand our facilities, augment our operational, financial and management systems and hire and train additional qualified personnel. If we are unable to manage our growth effectively, our business would be harmed.

We might seek to develop our business through acquisitions of or investment in new or complementary businesses, products or technologies, and the failure to manage these acquisitions or investments, or the failure to integrate them with our existing business, could have a material adverse effect on us.

We might consider opportunities to acquire or invest in other technologies, products and businesses that might enhance our capabilities or complement our current product candidates. Potential and completed acquisitions and strategic investments involve numerous risks, including potential problems or issues associated with the following:

- assimilating the purchased technologies, products or business operations;
- maintaining uniform standards, procedures, controls and policies;
- unanticipated costs associated with the acquisition or investment;
- diversion of our management's attention from our preexisting business;
- maintaining or obtaining the necessary regulatory approvals or complying with regulatory requirements; and
- adverse effects on existing business operations.

We have no current commitments with respect to any acquisition or investment in other technologies or businesses. We do not know if we will identify suitable acquisitions, whether we will be able to successfully complete any acquisitions, or whether we will be able to successfully integrate any acquired product, technology or business into our business or retain key personnel, suppliers or collaborators.

Our ability to develop successfully our business through acquisitions would depend on our ability to identify, negotiate, complete and integrate suitable target businesses or technologies and obtain any necessary financing. These efforts could be expensive and time consuming and might disrupt our ongoing operations. If we are unable to integrate efficiently any acquired business, technology or product into our business, our business and financial condition might be adversely affected.

Risks Related to Our Regulatory and Legal Environment

We are and will be subject to extensive and costly government regulation and the failure to comply with these regulations may have a material adverse effect on our operations and business.

Product candidates employing our technology are subject to extensive and rigorous domestic government regulation including regulation by the FDA, the Centers for Medicare and Medicaid Services, other divisions of the U.S. Department of Health and Human Services, the U.S. Department of Justice, state and local governments, and their respective foreign equivalents. Both before and after approval of any product, we and our collaborators, suppliers, contract manufacturers and clinical investigators are subject to extensive regulation by governmental authorities in the United States and other countries, covering, among other things, testing, manufacturing, quality control, clinical trials, post-marketing studies, labeling, advertising, promotion, distribution, import and export, governmental pricing, price reporting and rebate requirements. Failure to comply with applicable requirements could result in one or more of the following actions: warning or untitled letters; unanticipated expenditures; delays in approval or refusal to approve a product candidate; voluntary product recall; product seizure; interruption of manufacturing or clinical trials; operating or marketing restrictions; injunctions; criminal prosecution and civil or criminal penalties including fines and other monetary penalties; exclusion from federal health care programs such as Medicare and Medicaid; adverse publicity; and disruptions to our business. Further, government investigations into potential violations of these laws would require us to expend considerable resources and face adverse

publicity and the potential disruption of our business even if we are ultimately found not to have committed a violation. If products employing our technologies are marketed abroad, they will also be subject to extensive regulation by foreign governments, whether or not they have obtained FDA approval for a given product and its uses. Such foreign regulation might be equally or more demanding than corresponding U.S. regulation.

Government regulation substantially increases the cost and risk of researching, developing, manufacturing, and selling our product candidates. The regulatory review and approval process, which includes nonclinical testing and clinical trials of each product candidate, is lengthy, expensive, and uncertain. We or our collaborators must obtain and maintain regulatory authorization to conduct clinical trials and approval for each product we intend to market, and the manufacturing facilities used for the products must be inspected and meet legal requirements. Securing regulatory approval requires submitting extensive nonclinical and clinical data and other supporting information for each proposed therapeutic indication in order to establish the product's safety and efficacy for each intended use. The development and approval process might take many years, requires substantial resources, and might never lead to the approval of a product.

Even if we or our collaborators are able to obtain regulatory approval for a particular product, the approval might limit the intended medical uses for the product, limit our ability to promote, sell, and distribute the product, require that we conduct costly post-marketing surveillance, and/or require that we conduct ongoing post-marketing studies. Material changes to an approved product, such as, for example, manufacturing changes or revised labeling, might require further regulatory review and approval. Once obtained, any approvals might be withdrawn, including, for example, if there is a later discovery of previously unknown problems with the product, such as a previously unknown safety issue.

If we, our collaborators, or our contract manufacturers fail to comply with applicable regulatory requirements at any stage during the regulatory process, such noncompliance could result in, among other things, delays in the approval of applications or supplements to approved applications; refusal by a regulatory authority, including the FDA, to review pending market approval applications or supplements to approved applications; untitled letters or warning letters; fines; import and export restrictions; product recalls or seizures; injunctions; total or partial suspension of production; civil penalties; withdrawals of previously approved marketing applications; recommendations by the FDA or other regulatory authorities against governmental contracts; and/or criminal prosecutions.

The regulatory approval processes of the FDA, the EMA and comparable foreign authorities are lengthy, time consuming and inherently unpredictable, and if we or our collaborators are ultimately unable to obtain regulatory approval for our product candidates, our business will be substantially harmed.

We, or any current or future collaborators, cannot assure you that we will receive the approvals necessary to commercialize for sale any of our product candidates, or any product candidate we acquire or develop in the future. We will need FDA approval to commercialize our product candidates in the United States and approvals from the FDA-equivalent regulatory authorities in foreign jurisdictions to commercialize our product candidates in those jurisdictions. In order to obtain FDA approval of any product candidate, we must submit to the FDA a NDA, in the case of our HBV program, or a BLA, in the case of our product candidates in our Microbiome program, demonstrating that the product candidate is safe for humans and effective for its intended use (for biological products, this standard is referred to as safe, pure and potent). This demonstration requires significant research, nonclinical studies, and clinical trials. Satisfaction of the FDA's regulatory requirements typically takes many years, depends upon the type, complexity and novelty of the product candidate and requires substantial resources for research, development and testing. We cannot predict whether our research and clinical approaches will result in drugs or biological products that the FDA considers safe for humans and effective for their indicated uses. The FDA has substantial discretion in the approval process and might require us to conduct additional nonclinical and clinical testing, perform post-marketing studies or otherwise limit or impose conditions on any approval we obtain.

The approval process might also be delayed by changes in government regulation, future legislation or administrative action or changes in FDA policy that occur prior to or during our regulatory review. Delays in obtaining regulatory approvals might:

- delay commercialization of, and our ability to derive product revenues from, our product candidates;
- impose costly procedures on us; and
- diminish any competitive advantages that we might otherwise enjoy.

Even if we comply with all FDA requests, the FDA might ultimately reject one or more of our NDAs or BLAs. We cannot be sure that we will ever obtain regulatory approval for our product candidates. Failure to obtain FDA approval of our product candidates will severely undermine our business by leaving us without a saleable product, and therefore without any source of revenues, until another product candidate could be developed or obtained. There is no guarantee that we will ever be able to develop an existing, or acquire another, product candidate.

In foreign jurisdictions, we must receive approval from the appropriate regulatory authorities before we can commercialize any product candidates. The risks associated with foreign regulatory approval processes are similar to the risks associated with the FDA approval procedures described above. We cannot assure you that we will receive the approvals necessary to commercialize our product candidates for sale outside the United States.

Even if our product candidates are approved, we and our collaborators will be subject to extensive post-approval regulation, including ongoing obligations and continued regulatory review, which may result in significant additional expense. If approved, our product candidates could be subject to post-marketing restrictions or withdrawal from the market and we, or any collaborators, may be subject to substantial penalties if we, or they, fail to comply with regulatory requirements or if we, or they, experience unanticipated problems with our products following approval.

Once a product candidate is approved, numerous post-approval requirements apply. Among other things, we and our collaborators will be subject to requirements regarding testing, manufacturing, quality control, clinical trials, post-marketing studies, labeling, advertising, promotion, distribution, import and export, governmental pricing, price reporting and rebate requirements. The holder of an approved NDA or BLA is subject to ongoing FDA oversight monitoring and reporting obligations, including obligations to monitor and report adverse events and instances of the failure of a product to meet the specifications in the NDA or BLA. Application holders must submit new or supplemental applications and obtain FDA approval for changes to the approved product, product labeling, or manufacturing process, depending on the nature of the change. Application holders also must submit advertising and other promotional material to the FDA and report on ongoing clinical trials. The FDA also has the authority to require changes in the labeling of approved drug products and to require post-marketing studies. The FDA can also impose distribution and use restrictions under a REMS.

Advertising and promotional materials must comply with FDA rules in addition to other applicable federal and state laws. The distribution of product samples to physicians must comply with the requirements of the Prescription Drug Marketing Act. Manufacturing facilities remain subject to FDA inspection and must continue to adhere to the FDA's cGMP requirements. Sales, marketing, and scientific/educational grant programs, among other activities, must comply with the anti-fraud and abuse provisions of the Social Security Act, the False Claims Act, and similar state laws, each as amended. Pricing and rebate programs must comply with the Medicaid rebate requirements of the Omnibus Budget Reconciliation Act of 1990 and the Veterans Health Care Act of 1992, each as amended. If products are made available to authorized users of the Federal Supply Schedule of the General Services Administration, additional laws and requirements apply. All of these activities are also potentially subject to federal and state consumer protection and unfair competition laws.

Depending on the circumstances, failure to meet these post-approval requirements can result in criminal prosecution, fines, 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. In addition, even if we comply with FDA and other

requirements, new information regarding the safety or effectiveness of a product could lead the FDA to modify or withdraw product approval.

The policies of the FDA and of other regulatory authorities may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of our product candidates. We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative or executive action, either in the United States or abroad. For example, certain policies of the current administration may impact our business and industry. Namely, the current administration has taken several executive actions, including the issuance of a number of Executive Orders, that could impose significant burdens on, or otherwise materially delay, FDA's ability to engage in routine oversight activities such as implementing statutes through rulemaking, issuance of guidance, and review and approval of marketing applications. Notably, on January 30, 2017, an Executive Order was issued directing all executive agencies, including the FDA, that, for each notice of proposed rulemaking or final regulation to be issued in fiscal years 2018 and beyond, the agencies must identify regulations to offset any incremental cost of a new regulation. On September 8, 2017, the FDA published notices in the Federal Register soliciting broad public comment to identify regulations that could be modified in compliance with these Executive Orders. It is difficult to predict how these requirements will be implemented, and the extent to which they will impact the FDA's ability to exercise its regulatory authority. If these executive actions impose restrictions on FDA's ability to engage in oversight and implementation activities in the normal course, our business may be negatively impacted. In addition, if we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval that we may have obtained and we may not achieve or sustain profitability.

Even if we or our collaborators are able to commercialize any product candidates, those products may become subject to unfavorable pricing regulations, third-party coverage and reimbursement practices or healthcare reform initiatives, which would harm our business.

The regulations that govern marketing approvals, pricing and reimbursement for new medicines vary widely from country to country. In the United States, recently enacted legislation may significantly change the approval requirements in ways that could involve additional costs and cause delays in obtaining approvals. Some countries require approval of the sale price of a medicine before it can be marketed. In many countries, the pricing review period begins after marketing or product licensing approval is granted. In some foreign markets, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. As a result, we might obtain marketing approval for a medicine in a particular country, but then be subject to price regulations that delay our commercial launch of the medicine, possibly for lengthy time periods, and negatively impact the revenues we are able to generate from the sale of the medicine in that country. Adverse pricing limitations may hinder our ability to recoup our investment in one or more product candidates, even if our product candidates obtain marketing approval.

Our ability to commercialize any medicines successfully also will depend in part on the extent to which reimbursement for these medicines and related treatments will be available from government health administration authorities, private health insurers and other organizations. Government authorities and third-party payors, such as private health insurers and health maintenance organizations, decide which medications they will pay for and establish reimbursement levels. A primary trend in the U.S. healthcare industry and elsewhere is cost containment. Government authorities and third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. Increasingly, third-party payors are requiring that drug companies provide them with predetermined discounts from list prices and are challenging the prices charged for medical products. We cannot be sure that reimbursement will be available for any product candidate that we commercialize and, if reimbursement is available, the level of reimbursement. Reimbursement may impact the demand for, or the price of, any product candidate for which we obtain marketing approval. If reimbursement is not available or is available only to limited levels, we may not be able to commercialize successfully any product candidate for which we obtain marketing approval.

There may be significant delays in obtaining reimbursement for newly approved medicines, and coverage may be more limited than the purposes for which the medicine is approved by the FDA or similar regulatory authorities outside the United States. Moreover, eligibility for reimbursement does not imply that

any medicine will be paid for in all cases or at a rate that covers our costs, including research, development, manufacture, sale and distribution. Interim reimbursement levels for new medicines, if applicable, may also not be sufficient to cover our costs and may not be made permanent. Reimbursement rates may vary according to the use of the medicine and the clinical setting in which it is used, may be based on reimbursement levels already set for lower cost medicines and may be incorporated into existing payments for other services. Net prices for medicines may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors and by any future relaxation of laws that presently restrict imports of medicines from countries where they may be sold at lower prices than in the United States. Third-party payors often rely upon Medicare coverage policy and payment limitations in setting their own reimbursement policies. Our inability to obtain promptly coverage and profitable payment rates from both government-funded and private payors for any approved product candidates that we develop could have a material adverse effect on our operating results, our ability to raise capital needed to commercialize product candidates and our overall financial condition.

In the United States and in other countries, there have been, and we expect there will continue to be, a number of legislative and regulatory proposals to change the healthcare system in ways that could significantly affect our business. International, federal and state lawmakers regularly propose and, at times, enact legislation that would result in significant changes to the healthcare system, some of which are intended to contain or reduce the costs of medical products and services. The U.S. government and other governments have shown significant interest in pursuing healthcare reform, as evidenced by the Patient Protection and Affordable Care Act and its amendment, the Health Care and Education Reconciliation Act (the ACA).

Among the provisions of the ACA of importance to our potential drug candidates are the following:

- an annual, nondeductible fee on any entity that manufactures or imports specified branded prescription drugs and biologics;
- an increase in the statutory minimum rebates a manufacturer must pay under the Medicaid Drug Rebate Program;
- expansion of healthcare fraud and abuse laws, including the False Claims Act and the Anti-Kickback Statute, new government investigative powers, and enhanced penalties for noncompliance;
- a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 50% point-of-sale discounts off negotiated prices;
- extension of manufacturers' Medicaid rebate liability;
- expansion of eligibility criteria for Medicaid programs;
- expansion of the entities eligible for discounts under the Public Health Service Act pharmaceutical pricing program;
- new requirements to report financial arrangements with physicians and teaching hospitals;
- a new requirement to annually report drug samples that manufacturers and distributors provide to physicians; and
- a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research.

Since its enactment, there have been many judicial, Presidential, and Congressional challenges to numerous aspects of the ACA. In 2012, the U.S. Supreme Court heard challenges to the constitutionality of the individual mandate and the viability of certain provisions of the Healthcare Reform Act. The Supreme Court's decision upheld most of the Healthcare Reform Act and determined that requiring individuals to maintain "minimum essential" health insurance coverage or pay a penalty to the Internal Revenue Service was within Congress's constitutional taxing authority. However, as a result of tax reform legislation passed in late December 2017, the individual mandate has been eliminated. The long ranging effects of the elimination of the individual mandate on the viability of the ACA are unknown at this time.

On January 20, 2017, President Trump signed an Executive Order directing federal agencies with authorities and responsibilities under the ACA to waive, defer, grant exemptions from, or delay the implementation of any provision of the ACA that would impose a fiscal burden on states or a cost, fee, tax, penalty or regulatory burden on individuals, healthcare providers, health insurers, or manufacturers of pharmaceuticals or medical devices. On October 13, 2017, President Trump signed an Executive Order terminating the cost-sharing subsidies that reimburse insurers under the ACA. Several state Attorneys General filed suit to stop the administration from terminating the subsidies, but their request for a restraining order was denied by a federal judge in California on October 25, 2017. In addition, Centers for Medicare & Medicaid Services has recently proposed regulations that would give states greater flexibility in setting benchmarks for insurers in the individual and small group marketplaces, which may have the effect of relaxing the essential health benefits required under the ACA for plans sold through such marketplaces. The full impact of the ACA, any law repealing and/or replacing elements of it, and the political uncertainty surrounding any repeal or replacement legislation on our business remains unclear.

In addition, other legislative changes have been proposed and adopted since the ACA was enacted. These changes included aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, starting in 2013. In January 2013, then President Obama signed into law the American Taxpayer Relief Act of 2012, which, among other things, reduced Medicare payments to several providers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These laws may result in additional reductions in Medicare and other healthcare funding. Further, in some foreign jurisdictions, there have been a number of legislative and regulatory proposals to change the healthcare system in ways that could affect our ability to sell our products profitably. The continuing efforts of U.S. and other governments, insurance companies, managed care organizations and other payors of healthcare services to contain or reduce healthcare costs may adversely affect our ability to set satisfactory prices for our products, to generate revenues, and to achieve and maintain profitability.

We and our collaborators may be subject, directly or indirectly, to applicable U.S. federal and state anti-kickback, false claims laws, physician payment transparency laws, fraud and abuse laws or similar healthcare and security laws and regulations, which could expose us or them to criminal sanctions, civil penalties, contractual damages, reputational harm and diminished profits and future earnings.

Healthcare providers, physicians and others play a primary role in the recommendation and prescription of any products for which we obtain regulatory approval. If we obtain FDA approval for any of our drug candidates and begin commercializing those drugs in the United States, our operations may be subject to various federal and state fraud and abuse laws, including, without limitation, the federal Anti-Kickback Statute, the federal False Claims Act, and physician payment sunshine laws and regulations. These laws may impact, among other things, our proposed sales, marketing and education programs. In addition, we may be subject to 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:

- the federal Anti-Kickback Statute, which prohibits, among other things, knowingly and willfully soliciting, receiving, offering or paying any remuneration (including any kickback, bribe, or rebate), directly or indirectly, overtly or covertly, in cash or in kind, to induce, or in return for, either the referral of an individual, or the purchase, lease, order or recommendation of any good, facility, item or service for which payment may be made, in whole or in part, under a federal healthcare program, such as the Medicare and Medicaid programs;

- federal civil and criminal false claims laws and civil monetary penalty laws, such as the federal False Claims Act, which impose criminal and civil penalties and authorize civil whistleblower or qui tam actions, against individuals or entities for, among other things: knowingly presenting, or causing to be presented, to the federal government, claims for payment that are false or fraudulent; making a false statement or record material to a false or fraudulent claim or obligation to pay or transmit money or property to the federal government; or knowingly concealing or knowingly and improperly avoiding or decreasing an obligation to pay money to the federal government. In addition, the government may assert that a claim including items and services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the False Claims Act;

- the federal Health Insurance Portability and Accountability Act of 1996 (HIPAA), which created new federal criminal statutes that prohibit knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program or obtain, by means of false or fraudulent pretenses, representations, or promises, any of the money or property owned by, or under the custody or control of, any healthcare benefit program, regardless of the payor (e.g., public or private) and knowingly and willfully falsifying, concealing or covering up by any trick or device a material fact or making any materially false statements in connection with the delivery of, or payment for, healthcare benefits, items or services relating to healthcare matters;

- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009, and their respective implementing regulations, which impose requirements on certain covered healthcare providers, health plans, and healthcare clearinghouses as well as their respective business associates that perform services for them that involve the use, or disclosure of, individually identifiable health information, relating to the privacy, security and transmission of individually identifiable health information;

- the U.S. federal Food, Drug and Cosmetic Act (FDCA), which prohibits, among other things, the adulteration or misbranding of drugs, biologics and medical devices, and the Public Health Service Act (PHSA), which prohibits, among other things, the introduction into interstate commerce of a biological product unless a biologics license is in effect for that product;

- the federal false statements statute prohibits knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statement in connection with the delivery of or payment for healthcare benefits, items or services;

- the federal transparency requirements under the ACA, including the provision commonly referred to as the Physician Payments Sunshine Act, 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 to report annually to the U.S. Department of Health and Human Services information related to payments or other transfers of value made to physicians and teaching hospitals, as well as ownership and investment interests held by physicians and their immediate family members; and

- federal consumer protection and unfair competition laws, which broadly regulate marketplace activities and activities that potentially harm consumers.

Additionally, we are subject to state and non-U.S. equivalents of each of the healthcare laws described above, among others, some of which may be broader in scope and may apply regardless of the payor. Many U.S. states have adopted laws similar to the federal Anti-Kickback Statute, some of which apply to the referral of patients for healthcare services reimbursed by any source, not just governmental payors, including private insurers. In addition, some states have passed laws that require pharmaceutical companies to comply with the April 2003 Office of Inspector General Compliance Program Guidance for Pharmaceutical Manufacturers and/or the Pharmaceutical Research and Manufacturers of America's Code on Interactions with Healthcare Professionals. Several states also impose other marketing restrictions or require pharmaceutical companies to make marketing or price disclosures to the state. There are ambiguities as to what is required to comply with these state requirements and if we fail to comply with an applicable state law requirement we could be subject to penalties.

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. In addition, recent health care reform legislation has strengthened these laws. For example, the ACA, among other things, amends the intent requirement of the federal Anti-Kickback and criminal healthcare fraud statutes. As a result of such amendment, a person or entity no longer needs to have actual knowledge of these statutes or specific intent to violate them in order to have committed a violation. Moreover, the ACA 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 False Claims Act.

Violations of fraud and abuse laws may be punishable by criminal and/or civil sanctions, including penalties, fines and/or exclusion or suspension from federal and state healthcare programs such as Medicare and Medicaid and debarment from contracting with the U.S. government. In addition, private individuals have the ability to bring actions on behalf of the U.S. government under the federal False Claims Act as well as under the false claims laws of several states.

Law enforcement authorities are increasingly focused on enforcing fraud and abuse laws, and it is possible that some of our practices may be challenged under these laws. Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. It is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of civil, criminal and administrative penalties, damages, disgorgement, monetary fines, possible exclusion from participation in Medicare, Medicaid and other federal healthcare programs, contractual damages, reputational harm, diminished profits and future earnings, and curtailment of our operations, any of which could adversely affect our ability to operate our business and our results of operations. In addition, the approval and commercialization of any of our drug candidates outside the United States will also likely subject us to non-U.S. equivalents of the healthcare laws mentioned above, among other non-U.S. laws.

If any of the physicians or other providers or entities with whom we expect to do business with are found to be not in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs, which may also adversely affect our business.

If we fail to maintain an effective system of disclosure controls and internal control over financial reporting, our ability to produce timely and accurate financial statements or comply with applicable regulations could be impaired.

As a public company, we are subject to the reporting requirements of the Securities Exchange Act of 1934, as amended (the Exchange Act), the Sarbanes-Oxley Act and the listing standards of NASDAQ, the exchange on which our common stock is listed. We expect that the requirements of these rules and regulations will continue to increase our legal, accounting and financial compliance costs, make some activities more difficult, time consuming and costly and place significant strain on our personnel, systems and resources.

The Sarbanes-Oxley Act requires, among other things, that we maintain effective disclosure controls and procedures and internal control over financial reporting. We are continuing to refine our disclosure controls and other procedures that are designed to ensure that the information that we are required to disclose in the reports that we will file with the SEC is properly recorded, processed, summarized and reported within the time periods specified in SEC rules and forms. We are also continuing to improve our internal control over financial reporting. We have expended, and anticipate that we will continue to expend, significant resources in order to maintain and improve the effectiveness of our disclosure controls and procedures and internal control over financial reporting.

Our current controls and any new controls that we develop in the future may become inadequate because of changes in conditions in our business. Further, weaknesses in our disclosure controls or our internal control over financial reporting may be discovered in the future. Any failure to develop or maintain effective controls, or any difficulties encountered in their implementation or improvement, could harm our operating results or cause us to fail to meet our reporting obligations and may result in a restatement of our financial statements for prior periods. Any failure to implement and maintain effective internal control over financial reporting could also adversely affect the results of management reports and independent registered public accounting firm audits of our internal control over financial reporting that we will be required to include in our periodic reports that will be filed with the SEC. If we were to have ineffective disclosure controls and procedures or internal control over financial reporting, our investors could lose confidence in our reported financial and other information, which would likely have a negative effect on the market price of our common stock.

We face the risk of product liability claims and might not be able to obtain insurance.

Our business exposes us to the risk of product liability claims that are inherent in the development of drugs and biotherapeutics. If the use of one or more of our product candidates or approved drugs, if any, harms people, we might be subject to costly and damaging product liability claims brought against us by clinical trial participants, consumers, health care providers, pharmaceutical companies or others selling our products. Our inability to obtain sufficient product liability/clinical trial insurance at an acceptable cost to protect against potential product liability claims could prevent or inhibit the commercialization of pharmaceutical products we develop. We cannot predict all of the possible harms or side effects that might result and, therefore, the amount of insurance coverage we maintain might not be adequate to cover all liabilities we might incur. We intend to expand our insurance coverage to include product liability insurance covering the sale of commercial products if we obtain marketing approval for our drug candidates in development, but we might be unable to obtain commercially reasonable product liability insurance for any products approved for marketing. If we are unable to obtain insurance at an acceptable cost or otherwise protect against potential product liability claims, we will be exposed to significant liabilities, which might materially and adversely affect our business and financial position. If we are sued for any injury allegedly caused by our products, our liability could exceed our total assets and our ability to pay the liability. Any successful product liability claims or series of claims brought against us would decrease our cash and could cause the value of our common stock to decrease.

We might be exposed to liability claims associated with the use of hazardous materials and chemicals.

Our research, development and manufacturing activities and/or those of our third-party contractors might involve the controlled use of hazardous materials and chemicals. Although we will strive to have our safety procedures, and those of our contractors, for using, storing, handling and disposing of these materials comply with federal, state and local laws and regulations, we cannot completely eliminate the risk of accidental injury or contamination from these materials. In the event of such an accident, we could be held liable for any resulting damages, and any liability could materially adversely affect our business, financial condition and results of operations. In addition, the federal, state and local laws and regulations governing the use, manufacture, storage, handling and disposal of hazardous or radioactive materials and waste products might require us to incur substantial compliance costs that could materially adversely affect our business, financial condition and results of operations. We currently do not carry hazardous materials liability insurance. We intend to obtain such insurance in the future if necessary, but cannot give assurance that we could obtain such coverage.

Our employees, independent contractors, consultants, collaborators and contract research organizations may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements, which could result in significant liability for us and harm our reputation.

We are exposed to the risk of fraud or other misconduct, including intentional failure to:

- comply with FDA regulations or similar regulations of comparable foreign regulatory authorities;
- provide accurate information to the FDA or comparable foreign regulatory authorities;
- comply with federal and state healthcare fraud and abuse laws and regulations and similar laws and regulations established and enforced by comparable foreign regulatory authorities;
- comply with the United States Foreign Corrupt Practices Act (the FCPA), the U.K. anti-bribery laws, the China anti-bribery laws and other anti-bribery laws;
- report financial information or data accurately; or
- disclose unauthorized activities to us.

Misconduct could also involve the improper use or misrepresentation of information obtained in the course of clinical trials, creating fraudulent data in our nonclinical studies or clinical trials or illegal misappropriation of product materials, which could result in regulatory sanctions, delays in clinical trials, or serious harm to our reputation. We have adopted a code of conduct for our directors, officers and employees (the Code of Conduct), but it is not always possible to identify and deter employee misconduct.

The precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws or regulations. Additionally, we are subject to the risk that a person or government could allege such fraud or other misconduct, even if none occurred. If any such actions are instituted against us and we are not successful in defending ourselves or asserting our rights, those actions could harm our business, results of operations, financial condition and cash flows, including through the imposition of significant fines or other sanctions.

We are subject to certain U.S. and foreign anti-corruption, anti-money laundering, export control, sanctions, and other trade laws and regulations (collectively, Trade Laws). We can face serious consequences for violations.

Among other matters, Trade Laws prohibit companies and their employees, agents, clinical research organizations, legal counsel, accountants, consultants, contractors, and other partners from authorizing, promising, offering, providing, soliciting, or receiving directly or indirectly, corrupt or improper payments or anything else of value to or from recipients in the public or private sector. Violations of Trade Laws can result in substantial criminal fines and civil penalties, imprisonment, the loss of trade privileges, debarment, tax reassessments, breach of contract and fraud litigation, reputational harm, and other consequences. We have direct or indirect interactions with officials and employees of government agencies or government-affiliated hospitals, universities, and other organizations. We also expect our non-U.S. activities to increase in time. We engage third parties for clinical trials and/or to obtain necessary permits, licenses, patent registrations, and other regulatory approvals. We can be held liable for the corrupt or other illegal activities of our personnel, agents, or partners, even if we do not explicitly authorize or have prior knowledge of such activities.

We are establishing international operations and conducting clinical trials outside of the United States and a number of risks associated with international operations could materially and adversely affect our business.

We expect to be subject to a number of risks related with our international operations, many of which may be beyond our control. These risks include:

- different regulatory requirements for drug approvals in foreign countries;
- different standards of care in various countries that could complicate the evaluation of our product candidates;
- different U.S. and foreign drug import and export rules;
- different reimbursement systems and different competitive drugs indicated to treat the indication for which our product candidates are being developed;
- reduced protection for intellectual property rights in certain countries;
- unexpected changes in tariffs, trade barriers and regulatory requirements;
- compliance with the FCPA and other anti-corruption and anti-bribery laws;
- compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;
- foreign taxes, including withholding of payroll taxes;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenues, and other obligations incident to doing business in another country; and
- business interruptions resulting from geopolitical actions, including war and terrorism, or natural disasters.

Risks Related to Our Intellectual Property

Our business depends on protecting our intellectual property.

If we and our licensors, IURTC and Therabiome, do not obtain protection for our respective intellectual property rights, our competitors might be able to take advantage of our research and

development efforts to develop competing drugs. Our success, competitive position and future revenues, if any, depend in part on our ability and the abilities of our licensors to obtain and maintain patent protection for our products, methods, processes and other technologies, to preserve our trade secrets, to prevent third parties from infringing on our proprietary rights and to operate without infringing the proprietary rights of third parties.

We seek to protect our proprietary position by filing patent applications in the United States and abroad related to our novel technologies and chemical and biological compositions that are important to our business. To date, although our licensors have filed patent applications, we do not own or have any rights to any issued patents that cover any of our product candidates, and we cannot be certain that we will secure any rights to any issued patents with claims that cover any of our proprietary product candidates and technologies. The patent prosecution process is expensive and time-consuming, and we may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection.

The patent process also is subject to numerous risks and uncertainties, and there can be no assurance that we will be successful in protecting our products by obtaining and defending patents. These risks and uncertainties include the following:

- Any patent rights, if obtained, might be challenged, invalidated, or circumvented, or otherwise might not provide any competitive advantage;
- Our competitors, many of which have substantially greater resources than we do and many of which might make significant investments in competing technologies, might seek, or might already have obtained, patents that will limit, interfere with, or eliminate our ability to make, use, and sell our potential products either in the United States or in international markets;
- As a matter of public policy regarding worldwide health concerns, there might be significant pressure on the U.S. government and other international governmental bodies to limit the scope of patent protection both inside and outside the United States for disease treatments that prove successful; and
- Countries other than the United States might have patent laws that provide less protection than those governing U.S. courts, allowing foreign competitors the ability to exploit these laws to create, develop, and market competing products.

In addition, the U.S. Patent and Trademark Office (the USPTO) and patent offices in other jurisdictions have often required that patent applications concerning pharmaceutical and/or biotechnology-related inventions be limited or narrowed substantially to cover only the specific innovations exemplified in the patent application, thereby limiting the scope of protection against competitive challenges. Thus, even if we or our licensors are able to obtain patents, the patents might be substantially narrower than anticipated.

Patent and other intellectual property protection is crucial to the success of our business and prospects, and there is a substantial risk that such protections, if obtained, will prove inadequate. Our business and prospects will be harmed if we fail to obtain these protections or they prove insufficient.

If we fail to comply with our obligations under our license agreements, we could lose rights to our product candidates or key technologies.

We have obtained rights to develop, market and sell some of our product candidates and technologies through intellectual property license agreements with third parties, including IURTC and Therabiome. These license agreements impose various diligence, milestone payment, royalty and other obligations on us. If we fail to comply with our obligations under our license agreements, we could lose some or all of our rights to develop, market and sell products covered by these licenses, and our ability to form collaborations or partnerships may be impaired. In addition, disputes may arise under our license agreements with third parties, which could prevent or impair our ability to maintain our current licensing arrangements on acceptable terms and to develop and commercialize the affected product candidates.

We may incur substantial costs as a result of litigation or other proceedings relating to patent and other intellectual property rights.

If we choose to go to court to stop another party from using the inventions claimed in any patents we obtain, that individual or company has the right to ask the court to rule that such patents are invalid or should not be enforced against that third party. These lawsuits are expensive and would consume time and resources and divert the attention of managerial and scientific personnel even if we were successful in stopping the infringement of such patents. There is a risk that the court will decide that such patents are not valid and that we do not have the right to stop the other party from using the inventions. There is also the risk that, even if the validity of such patents is upheld, the court will refuse to stop the other party on the ground that such other party's activities do not infringe our rights to such patents. If we were not successful in defending our intellectual property, our competitors could develop and market products based on our discoveries, which may reduce demand for our products.

We rely on trade secret protections through confidentiality agreements with our employees, customers and other parties, and the breach of these agreements could adversely affect our business and prospects.

We rely on trade secrets and proprietary know-how, which we seek to protect, in part, through confidentiality, invention, and non-disclosure agreements with our employees, scientific advisors, consultants, collaborators, suppliers, and other parties. There can be no assurance that these agreements will not be breached, that we would have adequate remedies for any such breach or that our trade secrets will not otherwise become known to or independently developed by our competitors. If any of these events occurs, or we otherwise lose protection for our trade secrets or proprietary know-how, the value of this information may be greatly reduced.

If our employees or consultants breach their confidentiality obligations, to be able to enforce these confidentiality provisions, we would need to know of the breach and have sufficient funds to enforce the provisions. We cannot assure you that we would know of or be able to afford enforcement of any breach. In addition, such provisions are subject to state law and interpretation by courts, which could limit the scope and duration of these provisions. Any limitation on or non-enforcement of these confidentiality provisions could have an adverse effect on our business.

We may infringe the intellectual property rights of others, which may prevent or delay our product development efforts and stop us from commercializing or increase the costs of commercializing our product candidates.

Our success will depend in part on our ability to operate without infringing the proprietary rights of third parties. Our competitors may have filed, and may in the future file, patent applications covering products and technologies similar to ours. Any such patent application may have priority over our patent applications, which could further require us to obtain rights from third parties to issued patents covering such products and technologies. We cannot guarantee that the manufacture, use or marketing of any product candidates that we develop will not infringe third-party patents.

A third party may claim that we are using inventions covered by the third party's patent rights and may go to court to stop us from engaging in our normal operations and activities, including making or selling our product candidates. Patent litigation is costly and time consuming. We may not have sufficient resources to address these actions, and such actions could affect our results of operations and divert the attention of managerial and scientific personnel.

If a patent infringement suit were brought against us, we may be forced to stop or delay developing, manufacturing, or selling potential products that are claimed to infringe a third party's intellectual property, unless that third party grants us rights to use its intellectual property. In such cases, we may be required to obtain licenses to patents or proprietary rights of others in order to continue development, manufacture or sale of our products. If we are unable to obtain a license or develop or obtain non-infringing technology, or if we fail to defend an infringement action successfully, or if we are found to have infringed a valid patent, we may incur substantial monetary damages, encounter significant delays in bringing our product candidates to market and be precluded from manufacturing or selling our product candidates, any of which could harm our business significantly.

If our efforts to protect our proprietary technologies are not adequate, we may not be able to compete effectively in our market.

We rely upon a combination of patents, trade secret protection and contractual arrangements to protect the intellectual property related to our technologies. We will only be able to protect our products and proprietary information and technology by preventing unauthorized use by third parties to the extent that our patents, trade secrets, and contractual position allow us to do so. Any disclosure to or misappropriation by third parties of our trade secrets or confidential information could compromise our competitive position. Moreover, we may in the future be involved in legal or administrative proceedings involving our intellectual property initiated by third parties, and which proceedings can result in significant costs and commitment of management time and attention. As our product candidates continue in development, third parties may attempt to challenge the validity and enforceability of our patents and proprietary information and technologies.

We may in the future be involved in initiating legal or administrative proceedings involving the product candidates and intellectual property of our competitors. These proceedings can result in significant costs and commitment of management time and attention, and there can be no assurance that our efforts would be successful in preventing or limiting the ability of our competitors to market competing products.

Composition-of-matter patents relating to the active pharmaceutical ingredient (API) are generally considered to be the strongest form of intellectual property protection for pharmaceutical products. Such patents provide protection not limited to any one method of use. Method-of-use patents protect the use of a product for the specified method(s), and do not prevent a competitor from making and marketing a product that is identical to our product for an indication that is outside the scope of the patented method. We rely on a combination of these and other types of patents to protect our product candidates, and there can be no assurance that our intellectual property will create and sustain the competitive position of our product candidates.

Biotechnology and pharmaceutical product patents involve highly complex legal and scientific questions and can be uncertain. Any patent applications that we own or license may fail to result in issued patents. Even if patents do successfully issue from our applications, third parties may challenge their validity or enforceability, which may result in such patents being narrowed, invalidated, or held unenforceable. Even if our patents and patent applications are not challenged by third parties, those patents and patent applications may not prevent others from designing around our claims and may not otherwise adequately protect our product candidates. If the breadth or strength of protection provided by the patents and patent applications we hold with respect to our product candidates is threatened, competitors with significantly greater resources could threaten our ability to commercialize our product candidates. Discoveries are generally published in the scientific literature well after their actual development, and patent applications in the United States and other countries are typically not published until 18 months after filing, and in some cases are never published. Therefore, we cannot be certain that we or our licensors were the first to make the inventions claimed in our owned and licensed patents or patent applications, or that we or our licensors were the first to file for patent protection covering such inventions. Subject to meeting other requirements for patentability, for U.S. patent applications filed prior to March 16, 2013, the first to invent the claimed invention is entitled to receive patent protection for that invention while, outside the United States, the first to file a patent application encompassing the invention is entitled to patent protection for the invention. The United States moved to a "first to file" system under the Leahy-Smith America Invents Act (AIA), effective March 16, 2013. The effects of this change and other elements of the AIA are currently unclear, as the USPTO is still implementing associated regulations, and the applicability of the AIA and associated regulations to our patents and patent applications have not been fully determined. This new system also includes new procedures for challenging issued patents and pending patent applications, which creates additional uncertainty. We may become involved in any variety of proceedings challenging our patents and patent applications or the patents and patent applications of others, and the outcome of any such proceedings are highly uncertain. An unfavorable outcome in any such proceedings could reduce the scope of, invalidate, and/or find our patent rights unenforceable, allowing third parties to commercialize our technology and compete directly with us, or result in our inability to manufacture, develop or commercialize our product candidates without infringing the patent rights of others. In addition to ongoing changes with

the AIA and USPTO regulations, recent decisions of the Supreme Court of the United States, and the possibility of statutory change to patent subject matter eligibility law advocated by such groups as the Intellectual Property Owners Association and the American Intellectual Property Law Association, provide additional uncertainty.

In addition to the protection afforded by patents, we seek to rely on trade secret protection and confidentiality agreements to protect proprietary know-how, information, or technology that is not covered by our patents. Although our agreements require all of our employees to assign their inventions to us, and we require all of our employees, consultants, advisors and any third parties who have access to our trade secrets, proprietary know-how and other confidential information and technology to enter into appropriate confidentiality agreements, we cannot be certain that our trade secrets, proprietary know-how and other confidential information and technology will not be subject to unauthorized disclosure or that our competitors will not otherwise gain access to or independently develop substantially equivalent trade secrets, proprietary know-how and other information and technology. Furthermore, the laws of some foreign countries, in particular China, where we anticipate increasing our activity and commercializing our product candidates, do not protect proprietary rights to the same extent or in the same manner as the laws of the United States. As a result, we may encounter significant problems in protecting and defending our intellectual property globally. If we are unable to prevent unauthorized disclosure of our intellectual property related to our product candidates and technology to third parties, we may not be able to establish or maintain a competitive advantage in our market, which could materially adversely affect our business and operations.

Our reliance on third parties and agreements with collaboration partners requires us to share our trade secrets, which increases the possibility that a competitor may discover them or that our trade secrets will be misappropriated or disclosed.

Our reliance on third-party contractors to develop and manufacture our product candidates is based upon agreements that limit the rights of the third parties to use or disclose our confidential information, including our trade secrets and know-how. Despite the contractual provisions, the need to share trade secrets and other confidential information increases the risk that such trade secrets and information are disclosed or used, even if unintentionally, in violation of these agreements. In the highly competitive markets in which our product candidates are expected to compete, protecting our trade secrets, including our strategies for addressing competing products, is imperative, and any unauthorized use or disclosure could impair our competitive position and may have a material adverse effect on our business and operations.

In addition, some of our collaboration partners are larger, more complex organizations than ours, and the risk of inadvertent disclosure of our proprietary information may be increased despite their internal procedures and contractual obligations in place with our collaboration partners. Despite our efforts to protect our trade secrets and other confidential information, a competitor's discovery of such trade secrets and information could impair our competitive position and have an adverse impact on our business.

We are developing an extensive worldwide patent portfolio. The cost of maintaining our patent protection is high and maintaining our patent protection requires continuous review and compliance in order to maintain worldwide patent protection. We may not be able to maintain effectively our intellectual property position throughout the major markets of the world.

The USPTO and foreign patent authorities require maintenance fees and payments as well as continued compliance with a number of procedural and documentary requirements. Noncompliance may result in abandonment or lapse of the subject patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. Non-compliance may result in reduced royalty payments for lack of patent coverage in a particular jurisdiction from our collaboration partners or may result in competition, either of which could have a material adverse effect on our business.

We have made, and will continue to make, certain strategic decisions in balancing costs and the potential protection afforded by the patent laws of certain countries. As a result, we may not be able to prevent third parties from practicing our inventions in all countries throughout the world, or from selling or importing products made using our inventions in and into the United States or other countries. Third

parties may use our technologies in territories in which we have not obtained patent protection to develop their own products and, further, may infringe our patents in territories which provide inadequate enforcement mechanisms, even if we have patent protection. Such third-party products may compete with our product candidates, and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

The laws of some foreign countries do not protect proprietary rights to the same extent as do the laws of the United States, and we may encounter significant problems in securing and defending our intellectual property rights outside the United States

Many companies have encountered significant problems in protecting and defending intellectual property rights in certain countries. The legal systems of certain countries, particularly countries such as China, do not always favor the enforcement of patents, trade secrets, and other intellectual property rights, particularly those relating to pharmaceutical and biotechnology products, which could make it difficult for us to stop infringement of our patents, misappropriation of our trade secrets, or marketing of competing products in violation of our proprietary rights. In China, our intended establishment of significant operations will depend in substantial part on our ability to enforce effectively our intellectual property rights in that country. Proceedings to enforce our intellectual property rights in foreign countries could result in substantial costs and divert our efforts and attention from other aspects of our business, and could put our patents in these territories at risk of being invalidated or interpreted narrowly, or our patent applications at risk of not being granted, and could provoke third parties to assert claims against us. We may not prevail in all legal or other proceedings that we may initiate and, if we were to prevail, the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

Intellectual property rights do not address all potential threats to any competitive advantage we may have.

The degree of future protection afforded by our intellectual property rights is uncertain because intellectual property rights have limitations, and intellectual property rights 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 compounds that are the same as or similar to our current or future product candidates but that are not covered by the claims of the patents that we own or have exclusively licensed.
- We or any of our licensors or 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 any of our licensors or strategic partners 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.
- The prosecution of our pending patent applications may not result in granted patents.
- Granted 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 by our competitors.
- Patent protection on our product candidates may expire before we are able to develop and commercialize the product, or before we are able to recover our investment in the product.
- Our competitors might conduct research and development activities in the United States and other countries that provide a safe harbor from patent infringement claims for such activities, as well as in countries in which we do not have patent rights, and may then use the information learned from such activities to develop competitive products for sale in markets where we intend to market our product candidates.

The existence of counterfeit pharmaceutical products in pharmaceutical markets may damage our brand and reputation and have a material adverse effect on our business, operations and prospects.

Counterfeit products, including counterfeit pharmaceutical products, are a significant problem, particularly in China. Counterfeit pharmaceuticals are products sold or used for research under the same or similar names, or similar mechanism of action or product class, but which are sold without proper licenses or approvals. The proliferation of counterfeit pharmaceuticals has grown in recent years and may continue to grow in the future. Such products may be used for indications or purposes that are not recommended or approved or for which there is no data or inadequate data with regard to safety or efficacy. Such products divert sales from genuine products, often are of lower cost, often are of lower quality (having different ingredients or formulations, for example), and have the potential to damage the reputation for quality and effectiveness of the genuine product. If counterfeit pharmaceuticals illegally sold or used for research result in adverse events or side effects to consumers, we may be associated with any negative publicity resulting from such incidents. Consumers may buy counterfeit pharmaceuticals that are in direct competition with our pharmaceuticals, which could have an adverse impact on our revenues, business and results of operations. In addition, counterfeit products could be used in nonclinical studies or clinical trials or could otherwise produce undesirable side effects or adverse events that may be attributed to our products as well, which could cause us or regulatory authorities to interrupt, delay or halt clinical trials and could result in the delay or denial of regulatory approval by the FDA or other regulatory authorities and potential product liability claims. With respect to China, although the government has recently been increasingly active in policing counterfeit pharmaceuticals, there is not yet an effective counterfeit pharmaceutical regulation control and enforcement system in China. As a result, we may not be able to prevent third parties from selling or purporting to sell our products in China. The existence of and any increase in the sales and production of counterfeit pharmaceuticals, or the technological capabilities of counterfeiters, could negatively impact our revenues, brand reputation, business and results of operations.

Risks Related to Our Common Stock

We might not be able to maintain the listing of our common stock on The NASDAQ Capital Market.

Our common stock is listed on The NASDAQ Capital Market under the symbol "ASMB." We might not be able to maintain the listing standards of that exchange. If we fail to maintain the listing requirements, our common stock might trade on the OTC Bulletin Board or in the "pink sheets" maintained by OTC Markets Group Inc. These alternative markets are generally considered to be markets that are less efficient and less broad than The NASDAQ Capital Market. A delisting of our common stock from The NASDAQ Capital Market and our inability to list the stock on another national securities exchange could negatively impact us by: (i) reducing the liquidity and market price of our common stock; (ii) reducing the number of investors willing to hold or acquire our common stock, which could negatively impact our ability to raise equity financing; (iii) limiting our ability to use a registration statement to offer and sell freely tradable securities, thereby preventing us from accessing the public capital markets and (iv) impairing our ability to provide equity incentives to our employees.

The price of our common stock might fluctuate significantly, and you could lose all or part of your investment.

Since our merger with Assembly Pharmaceuticals on July 11, 2014 through December 31, 2017, the closing price of our common stock has fluctuated between $4.54 and $49.91. Continued volatility in the market price of our common stock might prevent a stockholder from being able to sell shares of our common stock at or above the price paid for such shares. The trading price of our common stock might be volatile and subject to wide price fluctuations in response to various factors, including:

- the progress, results and timing of our clinical trials and nonclinical studies and other studies involving our product candidates;
- success or failure of our product candidates;
- the receipt or loss of required regulatory approvals for our product candidates;
- availability of capital;

- future issuances by us of our common stock or securities exercisable for or convertible into common stock;
- sale of shares of our common stock by our significant stockholders or members of our management;
- additions or departures of key personnel;
- investor perceptions of us and the pharmaceutical industry;
- issuance of new or changed securities analysts' reports or recommendations, or the announcement of any changes to our credit rating;
- introduction of new products or announcements of significant contracts, acquisitions or capital commitments by us or our competitors;
- threatened or actual litigation and government investigations;
- legislative, political or regulatory developments;
- the overall performance of the equity markets;
- actual or anticipated fluctuations in our quarterly financial and operating results;
- general economic conditions;
- changes in interest rates; and
- changes in accounting standards, policies, guidance, interpretations or principles.

These and other factors might cause the market price of our common stock to fluctuate substantially, which might limit or prevent investors from readily selling their shares of our common stock and might otherwise negatively affect the liquidity of our common stock. In addition, in recent years, the stock market has experienced significant price and volume fluctuations. This volatility has had a significant impact on the market price of securities issued by many companies across many industries. The changes frequently appear to occur without regard to the operating performance of the affected companies. Accordingly, the price of our common stock could fluctuate based upon factors that have little or nothing to do with our company, and these fluctuations could materially reduce our share price.

We may be subject to securities litigation, which is expensive and could divert management attention.

The market price of our common stock may be volatile, and in the past, companies that have experienced volatility in the market price of their stock have been subject to securities class action litigation. We may be the target of this type of litigation in the future. Securities litigation against us could result in substantial costs and divert our management's attention from other business concerns, which could seriously harm our business.

Our principal stockholders and management own a significant percentage of our stock and will be able to exert significant control over matters subject to stockholder approval.

At December 31, 2017, our executive officers and directors owned approximately 8.1% of our outstanding voting common stock, and this group together with other stockholders holding beneficially 5% of more of our outstanding voting common stock, owned approximately 61.9% of our outstanding voting common stock. Therefore, these stockholders, if acting together, have the ability to influence us through their ownership position. These stockholders may be able to determine the outcome of certain significant matters requiring stockholder approval. For example, these stockholders may be able to control elections of directors, amendments of our organizational documents, or approval of any merger, sale of assets, or other major corporate transaction. This may prevent or discourage unsolicited acquisition proposals or offers for our common stock that you may feel are in your best interest as one of our stockholders.

Our ability to use our net operating loss and credit carryforwards to offset future taxable income may be subject to certain limitations.

At December 31, 2017, we had potentially utilizable gross Federal net operating loss carryforwards of approximately $153.2 million, State net operating loss carry-forwards of approximately $165.0 million and research and development credit carry forward of approximately $5.6 million, all of which expire between 2027 and 2037. Our ability to utilize our net operating loss and credit carryforwards is dependent upon our ability to generate taxable income in future periods and may be limited due to restrictions imposed on utilization of net operating loss and credit carryforwards under federal and state laws upon a change in ownership.

Under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, a corporation that undergoes an "ownership change," is subject to limitations on its ability to use its pre-change net operating loss carryforwards (NOLs) and other pre-change tax attributes (such as research tax credits) to offset its post-change income or taxes. For these purposes, an ownership change generally occurs where the equity ownership of one or more stockholders or groups of stockholders who owns at least 5% of a corporation's stock increases its ownership by more than 50 percentage points over its lowest ownership percentage within a three-year period (calculate on a rolling basis). We may have experienced such ownership changes in the past, and we may experience ownership changes in the future, some of which are outside our control. These ownership changes may subject our existing net operating losses or credits to substantial limitations under Sections 382 and 383. Accordingly, we may not be able to utilize a material portion of our net operating losses or credits. Limitations on our ability to utilize our net operating losses to offset U.S. federal taxable income could potentially result in increased future tax liability to us. In addition, at the state level, there may be periods during which the use of net operating losses is suspended or otherwise limited, which could accelerate or permanently increase state taxes owed.

Because U.S. federal net operating losses generally may be carried forward for up to 20 years, the annual limitation may effectively provide a cap on the cumulative amount of pre-ownership change losses, including certain recognized built-in losses that may be utilized. Such pre-ownership change losses in excess of the cap may be lost. In addition, if an ownership change were to occur, it is possible that the limitations imposed on our ability to use pre-ownership change losses and certain recognized built-in losses could cause a net increase in our U.S. federal income tax liability and require U.S. federal income taxes to be paid earlier than otherwise would be paid if such limitations were not in effect. Further, if for financial reporting purposes the amount or value of these deferred tax assets is reduced, such reduction would have a negative impact on the book value of our common stock.

In addition, under the Tax Cuts and Jobs Act (the Tax Act), the amount of post 2017 NOLs that we are permitted to deduct in any taxable year is limited to 80% of our taxable income in such year, where taxable income is determined without regard to the NOL deduction itself. The Tax Act generally eliminates the ability to carry back any NOL to prior taxable years, while allowing post 2017 unused NOLs to be carried forward indefinitely. There is a risk that due to changes under the Tax Act, regulatory changes or other unforeseen reasons, our existing NOLs could expire or otherwise be unavailable to offset future income tax liabilities.

We do not intend to pay dividends for the foreseeable future and our stock may not appreciate in value.

We currently intend to retain our future earnings, if any, to finance the operation and growth of our business and do not expect to pay any cash dividends in the foreseeable future. As a result, the success of an investment in shares of our common stock will depend upon any future appreciation in its value. There is no guarantee that shares of our common stock will appreciate in value or that the price at which our stockholders have purchased their shares will be able to be maintained.

The requirements of being a public company add to our operating costs and might strain our resources and distract our management.

As a public company, we face increased legal, accounting, administrative and other costs and expenses not faced by private companies. We are subject to the reporting requirements of the Exchange Act, which requires that we file annual, quarterly and current reports with respect to our business and financial

condition, and the rules and regulations implemented by the SEC, the Sarbanes-Oxley Act, and The NASDAQ Capital Market, each of which imposes additional reporting and other obligations on public companies. These rules and regulations increase our legal and financial compliance costs and make some activities more time-consuming and costly, although we are currently unable to estimate these costs with any degree of certainty. Complying with these requirements might divert management's attention from other business concerns, which could have a material adverse effect on our prospects, business, and financial condition.

Additionally, the expenses incurred by public companies generally for reporting and corporate governance purposes have been increasing. These increased costs will require us to divert a significant amount of money that we could otherwise use to develop our product candidates or otherwise expand our business. If we are unable to satisfy our obligations as a public company, we could be subject to delisting of our common stock, fines, sanctions and other regulatory action and potentially civil litigation.

Several provisions of the Delaware General Corporation Law and our charter documents could discourage, delay or prevent a merger or acquisition, which could adversely affect the market price of our securities.

Several provisions of the Delaware General Corporation Law and our charter documents could discourage, delay or prevent a merger or acquisition that stockholders may consider favorable, and the market price of our securities could be reduced as a result. These provisions may include:

- authorizing the issuance of "blank check" preferred stock, the terms of which we may establish and shares of which we may issue without stockholders approval;
- prohibiting us from engaging in a "business combination" with an "interested stockholder" for a period of three years after the date of the transaction in which the person became an interested stockholder unless certain provisions are met;
- prohibiting cumulative voting in the election of directors;
- prohibiting shareholder action by written consent;
- limiting the persons who may call special meetings of stockholders; and
- establishing advance notice requirements for nominations for election to our board of directors or for proposing matters that can be acted on by stockholders at stockholder meetings.

If securities analysts downgrade our stock or cease coverage of us, the price of our stock could decline.

The trading market for our common stock relies in part on the research and reports that industry or financial analysts publish about us or our business. Currently, four financial analysts publish reports about us and our business. We do not control these analysts or any other analysts. Furthermore, there are many large, well-established, publicly traded companies active in our industry and market, which may mean that it is less likely that we will receive widespread analyst coverage. If any analyst who covers us downgrades our stock, our stock price would likely decline rapidly. If one or more analysts cease coverage of our company, we could lose visibility in the market, which in turn could cause our stock price to decline.

Item 1B. Unresolved Staff Comments

None.

Item 2. Properties

We lease office space for corporate and administrative functions in Carmel, Indiana under a lease agreement that expires in August 2023. The leased location in Carmel, Indiana supports both the HBV-cure and Microbiome programs. We lease office and laboratory space in San Francisco, California under a sublease that expires on December 31, 2018 unless we request a six-month extension. The leased location in San Francisco, California supports both the HBV-cure and Microbiome programs. We transferred the activities that we performed at Indiana University to our Carmel, Indiana and San Francisco, California

locations. Research activities for the Microbiome program are also being conducted at office and laboratory space in Groton, Connecticut under a lease that expires in March 2019. In May 2017, we ceased leasing office and laboratory space from Indiana University in Bloomington, Indiana and the University of Florida Research Foundation in Alachua, Florida.

We believe these leased facilities are adequate for our current needs and that additional space will be available in the future on commercially reasonable terms as needed.

Item 3. Legal Proceedings

We are not a party to any material legal proceedings at this time. From time to time, we may be subject to various legal proceedings and claims that arise in the ordinary course of our business activities. Although the results of litigation and claims cannot be predicted with certainty, we do not believe we are party to any claim or litigation the outcome of which, if determined adversely to us, would individually or in the aggregate be reasonably expected to have a material adverse effect on our business. Regardless of the outcome, litigation can have an adverse effect on us because of defense and settlement costs, diversion of management resources and other factors.

Item 4. Mine Safety Disclosures

Not applicable.

PART II

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

Market Information for Common Stock

Our common stock is traded under the symbol "ASMB" and is quoted on the NASDAQ Capital Market. The following table sets forth the high and low sales prices for shares of our common stock, as reported by NASDAQ for the periods indicated.

	2017		2016	
	High	Low	High	Low
First quarter	$28.18	$11.85	$ 7.50	$4.33
Second quarter	$28.24	$20.01	$ 6.67	$4.60
Third quarter	$35.13	$18.60	$ 8.30	$5.15
Fourth quarter	$52.37	$27.00	$14.50	$7.25

On February 22, 2018, the closing price for our common stock as reported on the NASDAQ Capital Market was $55.79.

Holders of Record

As of February 22, 2018, there were 86 stockholders of record, which excludes stockholders whose shares were held in nominee or street name by brokers.

Dividends

We have never declared or paid cash dividends on our common stock, and we currently do not plan to declare dividends on shares of our common stock in the foreseeable future. We expect to retain our future earnings, if any, for use in the operation and expansion of our business.

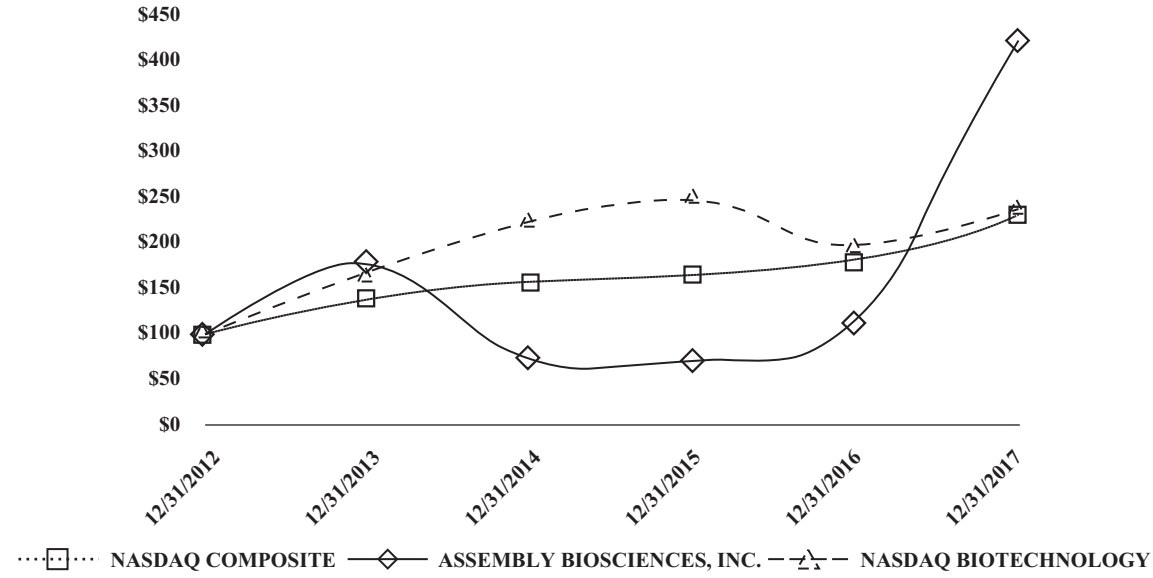
Comparative Stock Performance Graph

The information included under the heading "Comparative Stock Performance Graph" in this Item 5 of Part II of this Form 10-K shall not be deemed to be "soliciting material" or subject to Regulation 14A or 14C, shall not be deemed "filed" for purposes of Section 18 of the Exchange Act, or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act or the Exchange Act.

Set forth below is a graph comparing the total cumulative returns of our common stock, the NASDAQ Composite Index and the NASDAQ Biotechnology Index. The graph assumes $100 was invested in our common stock and each of the indices on December 31, 2012 and that all dividends, if any, are reinvested.

COMPARISON OF 5 YEAR CUMULATIVE TOTAL RETURN*

Among Assembly Biosciences, Inc., the NASDAQ Composite Index, and the NASDAQ Biotechnology Index



* $100 invested on December 31, 2012 in stock or index, including reinvestment of dividends.

	12/31/2012	12/31/2013	12/31/2014	12/31/2015	12/31/2016	12/31/2017
Assembly Biosciences, Inc. . . .	$100.00	$176.85	$ 72.78	$ 69.54	$112.50	$418.98
NASDAQ Composite	100.00	138.32	156.85	165.84	178.28	228.63
NASDAQ Biotechnology	100.00	165.54	221.53	247.10	194.19	235.12

Equity Compensation Plans

The following table sets forth the indicated information as of December 31, 2017 with respect to our equity compensation plans.

Plan Category	Number of securities to be issued upon exercise of outstanding options, warrants, RSUs and rights (a)	Weighted average exercise price of outstanding options, warrants, RSUs and rights (b)	Number of securities remaining available for future issuance under equity compensation plans (excluding securities reflected in column (a)) (c)
Equity compensation plans approved by our stockholders:			
2014 Stock Incentive Plan (*)	3,336,246	$11.51	587,391
2010 Equity Incentive Plan	595,334	$ 7.77	—
Options assumed in Assembly Pharmaceuticals Merger	551,239	$ 2.22	—
Equity compensation plans not approved by our stockholders:			
Consultant Warrants	15,296	$30.00	—
2017 Inducement Award Plan	189,000	26.57	611,000
Total	4,687,115		1,198,391

(*) 2014 Stock Incentive Plan shares available includes 73,876 shares of common stock forfeited under the 2010 Equity Incentive Plan on or after June 2, 2016.

Our shareholder approved equity compensation plans consist of the Amended and Restated 2014 Stock Incentive Plan (2014 Plan) and 2010 Equity Incentive Plan (2010 Plan). Effective on June 2, 2016, the 2010 Plan was frozen and no further grants will be made under the 2010 Plan. Shares that are forfeited under the 2010 Plan on or after June 2, 2016 will become available for issuance under the 2014 Plan.

Our outstanding equity compensation arrangements that have not been approved by our stockholders consist of (i) the 2017 Inducement Award Plan (the Inducement Plan) and (ii) warrants to purchase shares of our common stock issued to one consultant. On April 3, 2017, our board of directors adopted the Inducement Plan and reserved 800,000 shares of our common stock for issuance under the Inducement Plan. The only persons eligible to receive grants of awards under the Inducement Plan are individuals who satisfy the standards for inducement grants under Nasdaq Marketplace Rule 5635(c)(4) and the related guidance under Nasdaq IM 5635-1 — that is, generally, a person not previously an employee or director of the Company, or following a bona fide period of non-employment, as an inducement material to the individual's entering into employment with the Company. An "Award" is any right to receive our common stock pursuant to the Inducement Plan, consisting of nonstatutory stock options, stock appreciation rights, restricted stock awards, restricted stock unit awards, or any other stock award.

Shelf Registration

On December 30, 2015, we filed a registration statement on Form S-3 with the SEC using a "shelf" registration process, file number 333-208806, which became effective January 19, 2016. Under this shelf registration process, we may from time to time sell any combination of the securities described in the registration statement in one or more offerings for an aggregate offering price of up to $150,000,000. The amount to be registered under the shelf registration consists of up to $150,000,000 of an indeterminate amount of common stock, preferred stock, debt securities, warrants and/or units. There is also being registered under the shelf registration a currently indeterminate number of (i) shares of common stock or other securities of ours as may be issued upon conversion of, or in exchange for, convertible or exchangeable debt securities and/or preferred stock registered under the registration statement, or (ii) shares of preferred stock, common stock, debt securities or units as may be issued upon exercise of warrants registered by the registration statement, as the case may be. On November 6, 2017, we closed an offering of an aggregate offering price of approximately $69.3 million of common stock. As a result, securities with an aggregate offering price of approximately $80.7 million remain available under this registration statement.

On December 29, 2017, we filed a registration statement on Form S-3 with the SEC using a "shelf" registration process, file number 333-222366, which became effective January 10, 2018. Under this shelf registration process, we may from time to time sell any combination of the securities described in the registration statement in one or more offerings for an aggregate offering price of up to $250,000,000. The amount to be registered under the shelf registration consists of up to $250,000,000 of an indeterminate amount of common stock, preferred stock, debt securities, warrants and/or units. There is also being registered under the shelf registration a currently indeterminate number of (i) shares of common stock or other securities of ours as may be issued upon conversion of, or in exchange for, convertible or exchangeable debt securities and/or preferred stock registered under the registration statement, or (ii) shares of preferred stock, common stock, debt securities or units as may be issued upon exercise of warrants registered by the registration statement, as the case may be. In connection with the filing of this registration statement, we entered into a sales agreement under which we may offer and sell shares of our common stock having an aggregate offering price of up to $75.0 million under this registration statement through "at the market offerings." We have not issued any securities under this registration statement.

Recent Sales of Unregistered Securities

There were no unregistered sales of equity securities in 2017.

Item 6. Selected Financial Data

The following selected Balance Sheet data for the years ended December 31, 2017 and 2016 and the Statement of Operations data for the years ended December 31, 2017, 2016 and 2015 should be read in conjunction with Part II, Item 7 "Management's Discussion and Analysis of Financial Condition and Results of Operations" and in conjunction with the consolidated financial statements, related notes and other financial information included elsewhere in this Annual Report. The selected consolidated results of operation data for the years ended December 31, 2014 and 2013 and the balance sheet data for the years ended December 31, 2015, 2014 and 2013 have been derived from audited consolidated financial statements not included herein. Our historical results are not necessarily indicative of the results to be expected in the future.

	December 31,				
(In thousands)	**2017**	**2016**	**2015**	**2014**	**2013**
Balance Sheet Data:					
Total assets	$169,303	$ 98,119	$133,744	$ 71,225	$ 27,132
Total stockholders' equity	113,120	79,878	118,742	58,571	24,494
Statement of Operations Data:					
Collaboration revenue	$ 9,019	$ —	$ —	$ —	$ —
Operating expenses	61,246	45,278	29,656	23,956	19,605
Loss from operations	(52,227)	(45,278)	(29,656)	(23,956)	(19,605)
Interest income	983	1,539	1,229	167	201
Realized loss from marketable securities	(615)	(1,140)	(27)	—	—
Loss before income taxes	(51,859)	(44,879)	(28,454)	(23,789)	(19,404)
Income tax benefit	9,050	617	—	—	—
Net loss	$ (42,809)	$(44,262)	$ (28,454)	$(23,789)	$(19,404)
Unrealized loss recognized in accumulated other comprehensive loss before reclassification, net of tax benefit	(258)	(482)	(849)	—	—
Reclassification adjustment of unrealized loss included in net loss, net of tax expense	466	703	27	—	—
Loss per Shares Data:					
Basic and dilutive loss per share data	$ (2.41)	$ (2.57)	$ (1.81)	$ (3.40)	$ (5.00)

The increase in total assets from approximately $27.1 million as of December 31, 2013 to approximately $71.2 million as of December 31, 2014 was primarily due to the merger with Assembly Pharmaceuticals, Inc. in July 2014 and a capital raise of $15.0 million in net proceeds on October 6, 2014. The increase in total assets from approximately $71.2 million as of December 31, 2014 to approximately $133.7 million as of December 31, 2015 was primarily due to a capital raise of approximately $81.0 million in net proceeds to us. We did not engage in any financing activities in 2016; accordingly, total assets as of December 31, 2016 declined to approximately $98.0 million. The increase in total assets from approximately $98.1 million as of December 31, 2016 to approximately $169.3 million as of December 31, 2017 was primarily due to a capital raise of $64.8 million in net proceeds in November 2017 and receipt from Allergan of an upfront payment of $50.0 million in February 2017. Since 2013, our operating expenses have increased primarily due to increases in research and development activities. See, Part II, Item 7 "Management's Discussion and Analysis of Financial Condition and Results of Operations" for a discussion on results of operations and financing activities since 2014.

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

The following discussion of our financial condition and results of operations should be read in conjunction with our consolidated financial statements and the related notes thereto and other financial information appearing elsewhere in this Form 10-K. The following discussion contains forward-looking statements that involve risks and uncertainties. Our actual results could differ materially from those expressed or implied in any forward-looking statements as a result of various factors, including those set forth in this Form 10-K under "Item 1A. Risk Factors."

Overview

We are a clinical stage biotechnology company advancing two innovative platform programs: a new class of oral therapeutic candidates for the treatment of hepatitis B virus (HBV) infection and a novel class of oral synthetic live biotherapeutic candidates, which are designed to treat disorders associated with the microbiome.

Over 250 million people worldwide are chronically infected with HBV. Our HBV-cure program is pursuing multiple drug candidates that inhibit the HBV lifecycle and block the generation of covalently closed circular DNA (cccDNA), with the aim of increasing the current low cure rate for patients with HBV. We have discovered multiple novel Core protein Allosteric Modifiers (CpAMs), which are small molecules that directly target and allosterically modulate the HBV core (HBc) protein. The lead product candidate from this program, ABI-H0731, has completed the Phase 1a portion of a Phase 1a/1b human clinical trial in New Zealand, and commenced the Phase 1b portion of the clinical trial in the second quarter of 2017 in New Zealand and other countries outside the United States. We expect topline interim data from the Phase 1b portion of the clinical trial and full results in the first half of 2018. Assuming a successful Phase 1b monotherapy clinical trial, we expect to initiate a longer Phase 2 combination clinical trial in mid-2018 and have initial data in the second half of 2018. A larger Phase 2b combination clinical trial is anticipated for 2019. We have also successfully filed an Investigational New Drug application (IND) and have initiated an additional Phase 1a pharmacokinetic, safety and tolerability study of ABI-H0731 in the United States. In the fourth quarter of 2017, we announced the selection of our second product candidate from this program, ABI-H2158, which is currently undergoing IND enabling studies. ABI-H2158 is an internally discovered and developed drug product candidate.

Our Microbiome program consists of a fully integrated platform that includes a disease targeted strain identification and selection process, methods for strain isolation and growth under current Good Manufacturing Practice (cGMP) conditions, and a patent pending delivery system that we call GEMICEL®, which is designed to allow for targeted oral delivery of live biologic and conventional therapies to the lower gastrointestinal (GI) tract. The Microbiome program is prioritizing efforts on optimizing our lead product candidates, ABI-M201 (Ulcerative Colitis) and ABI-M301 (Crohn's disease), in preparation for studies to support potential INDs. Using our microbiome platform, we are exploring additional product candidates for other disease indications, including irritable bowel syndrome, non-alcoholic steatohepatitis (NASH), immuno-oncology and Clostridium difficile infections (CDI), which indications we may pursue either internally or in collaboration with partners. In September 2017, we elected not to initiate a Phase 1b clinical trial of our initial product candidate, ABI-M101, in patients with Clostridium difficile infections (CDI) who have relapsed after two or three standard antibiotic regimens.

On January 6, 2017, we entered into the Research, Development, Collaboration and License Agreement (the Collaboration Agreement) with Allergan Pharmaceuticals International Limited (Allergan) to develop and commercialize select microbiome gastrointestinal programs. Pursuant to the terms of the Collaboration Agreement, in connection with the closing of the transaction on February 10, 2017, Allergan paid us an upfront payment of $50 million. Additionally, we are eligible to receive up to approximately $630 million in payments related to seven development milestones and up to approximately $2.15 billion in payments related to 12 commercial development and sales milestones in connection with the successful development and commercialization of licensed compounds for up to six different indications. We have agreed with Allergan to share development costs up to an aggregate of $75 million through proof-of-concept (POC) studies on a ⅔, ⅓ basis, respectively, and Allergan has agreed to assume all post-POC development costs. Additionally, we have an option to co-promote the licensed programs in the United States and China, subject to certain conditions set forth in the Collaboration Agreement.

We currently have corporate and administrative offices in Carmel, Indiana and research facilities in Groton, Connecticut and San Francisco, California.

Since our inception, we have had no revenue from product sales, and have funded our operations principally through debt financings prior to our initial public offering in 2010 and through equity financings and collaborations since then. Our operations to date have been primarily limited to organizing and staffing our company, licensing our product candidates, discovering and developing our product candidates, establishing initial manufacturing capabilities for our product candidates, maintaining and improving our patent portfolio and raising capital. We have generated significant losses to date, and we expect to continue to generate losses as we continue to develop our product candidates. As of December 31, 2017, we had an accumulated deficit of approximately $251.0 million. Because we do not generate revenue from any of our product candidates, our losses will continue as we further develop and seek regulatory approval for, and commercialize, our product candidates. As a result, our operating losses are likely to be substantial over the next several years as we continue the development of our product candidates and thereafter if none is approved or successfully launched. We are unable to predict the extent of any future losses or when we will become profitable, if at all.

Financial Operations Overview

Research and Development Expense

Research and development expenses consist primarily of costs incurred for our research activities, including our drug discovery efforts, target validation, lead optimization and the development of our product candidates, which include:

- employee-related expenses including salaries, benefits, and stock-based compensation expense;
- expenses incurred under agreements with third parties, including contract research organizations (CROs) that conduct research and development, nonclinical and clinical activities on our behalf and the cost of consultants;
- the cost of lab supplies and acquiring, developing, and manufacturing nonclinical study materials; and
- facilities, depreciation, and other expenses, which include direct and allocated expenses for rent and maintenance of facilities, insurance, and other operating costs.

Research and development costs are expensed as incurred. Nonrefundable advance payments for goods or services to be received in the future for use in research and development activities are deferred and capitalized. The capitalized amounts are expensed as the related goods are delivered or the services are performed.

We use our employee and infrastructure resources across multiple research and development programs, and we allocate internal employee-related and infrastructure costs, as well as certain third-party costs, to each of our programs based on the personnel resources allocated to such program. Our research and development expenses, by major program, are outlined in the table below:

	Year Ended December 31,		
	2017	2016	2015
HBV	$23,220,949	$20,024,898	$10,810,517
Microbiome	15,581,341	10,015,153	4,296,309
Diltiazem	—	—	(6,621)
Other	—	27,748	—
Stock-based compensation	5,422,731	3,025,178	3,257,732
Total	$44,225,021	$33,092,977	$18,357,937

Diltiazem was a prior product candidate that we are no longer developing. Since the Merger in July 2014, the HBV-cure and Microbiome programs are the sole focus of our company.

The successful discovery and development of our product candidates is highly uncertain. As such, at this time, we cannot reasonably estimate, or know the nature, timing and estimated costs, of the efforts that will be necessary to complete the remainder of their development. We are also unable to predict when, if ever, material net cash inflows will commence from our product candidates. This is due to the numerous risks and uncertainties associated with developing medicines, including the uncertainty of:

- the timing, progress and success of our Phase 1 and Phase 2 clinical development of ABI-H0731 and our nonclinical and planned clinical development activities for ABI-H2158 and other product candidates we may identify in each of the HBV-cure and Microbiome programs;
- establishing an appropriate safety profile with IND-enabling toxicology studies sufficient to advance additional product candidates into clinical development;
- successful enrollment in, and completion of, clinical trials;
- receipt of marketing approvals from applicable regulatory authorities;
- establishing internal commercial manufacturing capabilities or making arrangements with third-party manufacturers;
- obtaining and maintaining patent and trade secret protection and regulatory exclusivity for our product candidates;
- launching commercial sales of the products, if and when approved, whether alone or in collaboration with others; and
- maintaining a continued acceptable safety profile of the products following approval and wide use.

A change in the outcome of any of these variables or variables discussed in "Item 1A. Risk Factors" with respect to the development of any of our product candidates would significantly change the costs and timing associated with the development of that product candidate.

Research and development activities are central to our business model. Product candidates in later stages of clinical development generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of later-stage clinical trials. We expect research and development costs to increase significantly for the foreseeable future as our product candidate development programs progress. However, we do not believe that it is possible at this time to accurately project total program-specific expenses through commercialization. There are numerous factors associated with the successful commercialization of any of our product candidates, including future trial design and various regulatory requirements, many of which cannot be determined with accuracy at this time based on our stage of development. Additionally, future commercial and regulatory factors beyond our control will impact our clinical development programs and plans.

General and administrative expenses

General and administrative expenses consist primarily of salaries and other related costs, including stock-based compensation, for personnel in executive, finance, accounting, business development, legal and human resources functions. Other significant costs include facility costs not otherwise included in research and development expenses, legal fees relating to patent and corporate matters and fees for accounting and consulting services.

We anticipate that our general and administrative expenses will increase in the future to support continued research and development activities, potential commercialization of our product candidates and increased costs of operating as a public company. These increases will likely include increased costs related to the hiring of additional personnel and fees to outside consultants, lawyers and accountants, among other expenses. Additionally, we anticipate increased costs associated with being a public company, including expenses related to services associated with maintaining compliance with exchange listing and SEC requirements, insurance, and investor relations costs.

Interest income

Interest income consists of interest earned on our cash and cash equivalents and available-for-sale securities.

Critical Accounting Policies and Significant Judgments and Estimates

Our management's discussion and analysis of our financial condition and results of operations are based on our consolidated financial statements, which we have prepared in accordance with accounting principles generally accepted in the United States. The preparation of these consolidated financial statements requires us to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of the financial statements, as well as the reported amounts of revenues and expenses during the reporting periods. On an ongoing basis, we evaluate our estimates and judgments, including those described in greater detail below. We base our estimates on historical experience and on various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions.

While our significant accounting policies are described in more detail in the notes to our consolidated financial statements included elsewhere in this Annual Report on Form 10-K, we believe that the following accounting policies are the most critical to aid you in fully understanding and evaluating our financial condition and results of operations.

Revenue Recognition

We recognize revenue from the sale of products and services to end customers and distributors under the provisions of FASB ASC 605, *Revenue Recognition*. Accordingly, revenue is recognized only when persuasive evidence of an arrangement exists, delivery has occurred or services have been rendered, and collectability of the fixed or determinable sales price is reasonably assured.

We recognize revenue under the Collaboration Agreement based on the relevant accounting literature. Under this guidance, multiple elements or deliverables may include (i) grants of licenses, or options to obtain licenses, to intellectual property, (ii) research and development services, (iii) participation on joint research and/or joint development committees, and/or (iv) manufacturing or supply of services. The payments entities may receive under these arrangements typically include one or more of the following: non-refundable, upfront license fees; option exercise fees; funding of research and/or development efforts; amounts due upon the achievement of specified objectives; and/or royalties on future product sales.

Multiple-element arrangements require the separability of deliverables included in an arrangement into different units of accounting and the allocation of arrangement consideration to the units of accounting. The evaluation of multiple-element arrangements requires management to make judgments about (i) the identification of deliverables, (ii) whether such deliverables are separable from the other aspects of the contractual relationship, (iii) the estimated selling price of each deliverable, and (iv) the expected period of performance for each deliverable.

To determine the units of accounting under a multiple-element arrangement, management evaluates certain separation criteria, including whether the deliverables have stand-alone value, based on the relevant facts and circumstances for each arrangement. Management then estimates the selling price for each unit of accounting and allocates the arrangement consideration to each unit using the relative selling price method. The relative selling price for each deliverable is determined using vendor specific objective evidence (VSOE), of selling price or third-party evidence of selling price if VSOE does not exist. If neither VSOE nor third-party evidence of selling price exists, we use our best estimate of the selling price for the deliverable. The allocated consideration for each unit of accounting is recognized based on the method most appropriate for that unit of account and in accordance with the revenue recognition criteria detailed above.

If there are deliverables in an arrangement that are not separable from other aspects of the contractual relationship, they are treated as a combined unit of accounting, with the allocated revenue for the combined unit recognized in a manner consistent with the revenue recognition applicable to the final deliverable in the combined unit. Payments received prior to satisfying the relevant revenue recognition criteria are recorded as deferred revenue in the accompanying balance sheets and recognized as revenue when the related revenue recognition criteria are met.

The Collaboration Agreement provides for non-refundable milestone payments. We recognize revenue that is contingent upon the achievement of a substantive milestone in its entirety in the period in which the milestone is achieved. A milestone is considered substantive when the consideration payable to us for such milestone (i) is consistent with our performance necessary to achieve the milestone or the increase in value to the collaboration resulting from our performance, (ii) relates solely to our past performance and (iii) is reasonable relative to all of the other deliverables and payments within the arrangement. In making this assessment, we consider all facts and circumstances relevant to the arrangement, including factors such as the scientific, regulatory, commercial and other risks that must be overcome to achieve the milestone, the level of effort and investment required to achieve the milestone and whether any portion of the milestone consideration is related to future performance or deliverables.

The Collaboration Agreement provides Allergan with options to license additional intellectual property rights, or purchase additional research, development, or supply services. We concluded that these were "substantive options" under the multiple-element arrangement guidance, and accordingly, associated fees have not been considered in allocating contract consideration among deliverables with stand-alone value. If Allergan exercises one or more of these options, the associated revenue would be recognized using the method most appropriate for the particular deliverable.

We will periodically review the estimated performance periods under the Collaboration Agreement, which provides for non-refundable upfront payments and fees. We will adjust the periods over which revenue should be recognized when appropriate to reflect changes in assumptions relating to the estimated performance periods. We could accelerate revenue recognition in the event of early termination of programs or if our expectations change. Alternatively, we could decelerate revenue recognition if programs are extended or delayed. While such changes to our estimates have no impact on our reported cash flows, the amount of revenue recorded in future periods could be materially impacted.

We record revenues related to the reimbursement of costs incurred under the Collaboration Agreement where we act as a principal, controls the research and development activities and bears credit risk. Under the Collaboration Agreement, we are reimbursed for associated out-of-pocket costs. The gross amount of these pass-through reimbursed costs is reported as revenue in the accompanying statements of operations, while the actual expenses for which we are reimbursed are reflected as research and development costs. We have also accounted for the milestone payments under ASC 605 *Revenue Recognition — Milestone Method*.

Marketable Securities

We have designated marketable securities as of December 31, 2017 as available-for-sale securities and measure these securities at their respective fair values. Marketable securities are classified as short-term or long-term based on the maturity date and their availability to meet current operating requirements. Marketable securities that mature in one year or less are classified as short-term available-for-sale securities and are reported as a component of current assets. Marketable securities that are not considered available for use in current operations are classified as long-term available-for-sale securities and are reported as a component of long-term assets.

Securities that are classified as available-for-sale are measured at fair value with temporary unrealized gains and losses reported in other comprehensive loss, and as a component of stockholders' equity until their disposition. We review all available-for-sale securities at each period end to determine if they remain available-for-sale based on then current intent and ability to sell the security if it is required to do so. The cost of securities sold is based on the specific identification method.

Marketable securities are subject to a periodic impairment review. We may recognize an impairment charge when a decline in the fair value of investments below the cost basis is determined to be other-than-temporary. There were no marketable securities deemed to be impaired as of December 31, 2017 or 2016.

Goodwill and Other Intangible Assets

Goodwill is the excess of purchase price over the fair value of identified net assets of businesses acquired. Our intangible assets with an indefinite life are related to in-process research and development (IPR&D) programs acquired in the Merger, as we expect future research and development on these programs to provide us with substantial benefit for a period that extends beyond the foreseeable horizon. Intangible assets with indefinite useful lives are measured at their respective fair values as of the acquisition date. We do not amortize goodwill and intangible assets with indefinite useful lives. Intangible assets related to IPR&D projects are considered to be indefinite lived until the completion or abandonment of the associated R&D efforts. If and when development is complete, which generally occurs if and when regulatory approval to market a product is obtained, the associated assets would be deemed finite lived and would then be amortized based on their respective estimated useful lives at that point in time.

We review goodwill and indefinite-lived intangible assets at least annually for possible impairment. Goodwill and indefinite-lived intangible assets are reviewed for possible impairment between annual tests if an event occurs or circumstances change that would more likely than not reduce the fair value of the reporting unit or the indefinite-lived intangible assets below their carrying values. We test goodwill and indefinite-lived intangible assets each year on October 1. We review the carrying value of goodwill utilizing a market value approach which was based upon the closing price of our stock price as of the beginning of the 4th quarter. As of October 1, 2017 and 2016, the fair value of our reporting unit was in excess of carrying value and goodwill was not deemed to be impaired.

On October 1, 2017, we elected to bypass the qualitative assessment and performed a quantitative impairment test. On October 1, 2016, we performed a qualitative assessment of IPR&D. We utilized a discounted probable future cash flow model to value acquired IPR&D. Significant assumptions used in the model include the period in which material net cash inflows are expected to commence, anticipated material changes from historical pricing, margins and expense levels and an appropriate risk adjusted discount rate applied to the estimated cash flows. As of October 1, 2017 and 2016, IPR&D was not deemed to be impaired.

Accrued Research and Development Expenses

As part of the process of preparing our consolidated financial statements, we are required to estimate our accrued expenses. This process involves reviewing quotations and contracts, identifying services that have been performed on our behalf and estimating the level of service performed and the associated cost incurred for the service when we have not yet been invoiced or otherwise notified of the actual cost. The majority of our service providers invoice us monthly in arrears for services performed or when contractual milestones are met. We make estimates of our accrued expenses as of each balance sheet date in our financial statements based on facts and circumstances known to us at that time.

Impairment of Long-lived Assets

We monitor the carrying value of long-lived assets for potential impairment and test the recoverability of such assets whenever events or changes in circumstances indicate that the carrying amounts may not be recoverable. If a change in circumstance occurs, we perform a test of recoverability by comparing the carrying value of the asset or asset group to its undiscounted expected future cash flows. If cash flows cannot be separately and independently identified for a single asset, we will determine whether impairment has occurred for the group of assets for which we can identify the projected cash flows. If the carrying values are in excess of undiscounted expected future cash flows, we measure any impairment by comparing the fair value of the asset or asset group to its carrying value. There were no indicators of impairment of long-lived assets during the years ended December 31, 2017 and 2016.

Fair Value Measurements

We follow accounting guidance on fair value measurements for financial instruments measured on a recurring basis, as well as for certain assets and liabilities that are initially recorded at their estimated fair values. Fair value is defined as the exit price, or the amount that would be received from selling an asset or

paid to transfer a liability in an orderly transaction between market participants at the measurement date. We use the following three-level hierarchy that maximizes the use of observable inputs and minimizes the use of unobservable inputs to value our financial instruments:

Level 1: Observable inputs such as unadjusted quoted prices in active markets for identical instruments.

Level 2: Quoted prices for similar instruments that are directly or indirectly observable in the marketplace.

Level 3: Significant unobservable inputs which are supported by little or no market activity and that are financial instruments whose values are determined using pricing models, discounted cash flow methodologies, or similar techniques, as well as instruments for which the determination of fair value requires significant judgment or estimation.

Financial instruments measured at fair value are classified in their entirety based on the lowest level of input that is significant to the fair value measurement. Our assessment of the significance of a particular input to the fair value measurement in its entirety requires us to make judgments and consider factors specific to the asset or liability. The use of different assumptions and/or estimation methodologies may have a material effect on estimated fair values. Accordingly, the fair value estimates disclosed or initial amounts recorded may not be indicative of the amount that we or holders of the instruments could realize in a current market exchange.

The carrying amounts of cash equivalents and marketable securities approximate their fair value based upon quoted market prices. Certain of our financial instruments are not measured at fair value on a recurring basis, but are recorded at amounts that approximate their fair value due to their liquid or short-term nature, such as cash, accounts receivable, accounts payable and accrued expenses.

Stock-Based Compensation

We apply the fair value recognition provisions of FASB ASC Topic 718, Compensation-Stock Compensation (ASC 718) to account for stock-based compensation. We record stock-based compensation on stock options and performance restricted stock units issued to employees and directors based on the estimated fair value on the date of grant and recognize compensation cost over the requisite service period for awards expected to vest.

The fair value of stock options is estimated on the date of grant using the Black-Scholes option pricing model ("Black-Scholes Model"). The fair value of stock-based payment awards as determined by the Black-Scholes Model are affected by our stock price as well as other assumptions. These assumptions include, but are not limited to, the expected term, the expected stock price volatility, the risk-free interest rate and the expected dividend yield. The fair value of stock options issued to employees and directors with service conditions are typically amortized to expense on a straight-line basis over the requisite service periods of the awards, which is generally the vesting period.

We account for stock options granted to non-employees, which primarily consist of consultants and members of our scientific advisory board, using the fair value method. Stock options granted to non-employees are subject to periodic revaluation over their vesting terms and stock-based compensation expense is recognized using an accelerated recognition model.

The fair value of restricted stock units is determined based on the number of shares granted and the quoted market price of our common stock on the date of grant. The fair value of restricted stock units with performance conditions deemed probable of being achieved and vesting are amortized to expense over the requisite service period using the straight-line method of expense recognition.

Effective on January 1, 2017, we elected to account for forfeited awards as they occur as permitted by Accounting Standards Update ("ASU") 2016-09. Ultimately, the actual expenses recognized over the vesting period will be for those shares that vested. Prior to making this election, we estimated a forfeiture rate for awards at 0%, as we did not have a significant history of forfeitures.

Off-Balance Sheet Arrangements

Since our inception, we have not engaged in any off-balance sheet arrangements, including the use of structured finance, special purpose entities or variable interest entities.

Contractual Obligations

We have contractual and commercial obligations under our operating leases and other obligations related to research and development activities, purchase commitments and licenses. The following table summarizes our future contractual obligations and commercial commitments at December 31, 2017.

	Payments Due By Period				
	Less than 1 year	1 – 3 years	3 – 5 years	More than 5 years	Total
Operating leases	$2,974,537	$2,274,773	$318,937	$—	$5,568,247
R&D and purchase commitments	3,274,191	61,857	—	—	3,336,048
License obligations	400,000	75,000	150,000	—	625,000
Total contractual obligations	$6,648,728	$2,411,630	$468,937	$—	$9,529,295

In general, milestone and royalty payments associated with certain license agreements (other than contingent performance milestone payments anticipated to be paid in 2018) have not been included in the above table of contractual obligations as we cannot reasonably estimate if or when they will occur. The milestone payments included in the table of contractual obligations above are payments we believe are reasonably likely to occur during the indicated time periods. Excluded from future R&D and purchase commitments is our portion of the potential future shared research and development expenses under the Collaboration Agreement with Allergan of up to $25 million. Further, we anticipate that our operating lease obligations will be higher than projected as we renew existing real estate leases that expire in 2018 and enter into new or expanded real estate leases.

Results of Operations

General

During the year ended December 31, 2017, we generated approximately $9.0 million of collaboration revenue, which included the amortization of deferred revenue and reimbursement revenue in each case incurred under the Collaboration Agreement. At December 31, 2017, we had an accumulated deficit of approximately $251.0 million primarily as a result of research and development expenses, purchases of in-process research and development and general and administrative expenses. While we may in the future generate revenue from a variety of sources, including license fees, milestone payments, research and development payments in connection with strategic partnerships and/or product sales, our product candidates are at an early stage of development and may never be successfully developed or commercialized. Accordingly, we expect to continue to incur substantial losses from operations for the foreseeable future and there can be no assurance that we will ever generate significant revenues.

Comparison of the Years Ended December 31, 2017 and December 31, 2016

Research and Development Expense

Research and development expense, excluding stock-based compensation expense, was approximately $38.8 million for the year ended December 31, 2017, an increase of approximately $8.7 million from approximately $30.1 million for the same period in 2016. The net increase in research and development expenses was primarily due to an increase of $3.2 million in research expenses for our HBV-cure program and an increase of approximately $5.6 million for nonclinical development of our Microbiome program.

Stock-based compensation expense was approximately $5.4 million for the year ended December 31, 2017, an increase of approximately $2.4 million from approximately $3.0 million for the year ended December 31, 2016.

General and Administrative Expense

General and administrative expense, excluding stock-based compensation expense, was approximately $13.8 million for the year ended December 31, 2017, an increase of approximately $3.6 million from approximately $10.2 million for the same period in 2016. The net increase in general and administrative expenses was primarily due to an increase of approximately $2.5 million of compensation and bonus expenses, $0.4 million in professional expenses and $0.3 million in legal expenses.

Stock-based compensation expense was approximately $3.2 million for the year ended December 31, 2017, an increase of approximately $1.2 million from approximately $2.0 million for the year ended December 31, 2016.

Interest and Other Income

Interest and other income was approximately $1.0 million for the year ended December 31, 2017 compared to approximately $1.5 million for the same period in 2016. Interest income for the years ended December 31, 2017 and 2016 was primarily related to interest income on marketable securities — corporation bonds and money market fund.

Income Tax Benefit

We maintain a valuation allowance on deferred tax assets due to the uncertainty regarding the ability to utilize these deferred tax assets in the future. The deferred tax liability was recorded in connection with the acquisition of Assembly Pharmaceuticals in 2014 and relates to the difference between the carrying amount of in-process research and development for financial statement purposes relative to the amount used for income tax purposes. The net deferred tax liability was $2.1 million and $11.1 million as of December 31, 2017 and 2016, respectively. The decrease to our deferred tax liabilities of $9.0 million for the year ended December 31, 2017 is primarily related to the effects of the Tax Act.

Comparison of the Years Ended December 31, 2016 and December 31, 2015

Research and Development Expense

Research and development expense, excluding stock-based compensation expense, was approximately $30.1 million for the year ended December 31, 2016, an increase of approximately $15.0 million from approximately $15.1 million for the same period in 2015. The net increase in research and development expenses was primarily due to an increase of $9.2 million in research expenses for our HBV-cure program and an increase of approximately $5.7 million for nonclinical development of our Microbiome program.

Stock-based compensation expense was approximately $3.0 million for the year ended December 31, 2016, a decrease of approximately $0.3 million from approximately $3.3 million for the year ended December 31, 2015.

General and Administrative Expense

General and administrative expense, excluding stock-based compensation expense, was approximately $10.2 million for the year ended December 31, 2016, an increase of approximately $3.5 million from approximately $6.7 million for the same period in 2015. The net increase in general and administrative expenses was primarily due to an increase of approximately $0.8 million of compensation and bonus expenses related to new employees hired in 2016, $0.8 million in consulting expenses, $0.5 million in professional expenses, $0.5 million in legal expenses, $0.4 million in travel expenses, $0.3 million in tax expenses and $0.1 million in board fees classified as operating expense.

Stock-based compensation expense was approximately $2.0 million for the year ended December 31, 2016, a decrease of approximately $2.6 million from approximately $4.6 million for the year ended December 31, 2015.

Interest and Other Income

Interest and other income was approximately $1.5 million for the year ended December 31, 2016 compared to approximately $1.2 million for the same period in 2015. Interest income for the years ended December 31, 2016 and 2015 was primarily related to interest income on marketable securities — corporation bonds.

Income Tax Benefit

We maintain a valuation allowance on deferred tax assets due to the uncertainty regarding the ability to utilize these deferred tax assets in the future. The deferred tax liability was recorded in connection with the acquisition of Assembly Pharmaceuticals in 2014 and relates to the difference between the carrying amount of in-process research and development for financial statement purposes relative to the amount used for income tax purposes. The deferred tax liability was $11.1 and $11.6 million as of December 31, 2016 and 2015, respectively.

Liquidity and Capital Resources

As a result of our significant research and development expenditures and the lack of any FDA-approved products to generate product sales revenue, we have not been profitable and have generated operating losses since we were incorporated in October 2005. We have funded our operations through December 31, 2017 principally with debt prior to our initial public offering, and thereafter with equity financing, raising an aggregate of $257.3 million in net proceeds from public offerings and private placements from inception to December 31, 2017. Additionally, in February 2017, we received a $50.0 million upfront payment in connection with the closing of the Collaboration Agreement with Allergan.

In January 2014, we sold an aggregate of 92,472 shares of common stock under the amended at-the-market common equity sales program, resulting in net proceeds of approximately $1.8 million.

On October 6, 2014, we sold to various institutional investors an aggregate of 1,959,000 shares of common stock in a registered direct offering. The purchase price paid by the investors was $8.04 per share and an aggregate of approximately $15.0 million in net proceeds were received. In connection with the offering, we entered into a placement agent agreement with William Blair & Company, L.L.C., who acted as sole placement agent in the offering, and pursuant to which we paid a placement agent fee equal to 5.0% of the gross proceeds of the offering.

On March 19, 2015, we sold to various investors an aggregate of 5,555,555 shares of common stock in a public offering. The purchase price paid by investors was $13.50 per share and an aggregate of $70.4 million (net of underwriting discounts and commissions and offering expenses) was received. In addition, we granted the underwriters a 30-day option to purchase up to an additional 833,333 shares of common stock.

On April 6, 2015, the underwriters exercised in full their option to purchase an additional 833,333 shares of common stock at the public offering price of $13.50 per share. Proceeds from the sale of shares on the exercise of the underwriters' option (net of underwriting discounts and commissions) were approximately $10.6 million.

On November 1, 2017, we sold to various investors an aggregate of 2,210,000 shares of common stock in a public offering. The purchase price paid by investors was $27.25 per share and an aggregate of $64.8 million (net of underwriting discounts and commissions and offering expenses) was received, which includes proceeds received pursuant to the underwriters' exercise of their 30-day option to purchase of up to an additional 331,500 shares in full, which occurred on November 2, 2017.

Cash Flows for the Three Years Ended December 31, 2017, 2016 and 2015

	Year Ended December 31,		
	2017	**2016**	**2015**
Operating activities	$ 1,860,081	$(34,881,655)	$(18,697,334)
Investing activities	(15,642,453)	36,196,574	(64,855,510)
Financing activities	67,240,496	152,640	81,569,257
Net increase (decrease) in cash and cash equivalents	$ 53,458,124	$ 1,467,559	$ (1,983,587)

Net Cash Provided by (Used in) Operating Activities

Net cash provided by operating activities was approximately $1.9 million for the year ended December 31, 2017 and funded our research and development program build out and general and administrative expenses. Net cash provided by continuing operations for the year ended December 31, 2017 was primarily driven by approximately $8.6 million non-cash expenses recorded for the stock-based compensation, an approximately $44.3 million increase in cash from changes in operating assets and liabilities (primarily due to an increase in deferred revenue of $45.8 million related to the Allergan Collaboration) and $0.6 million realized loss from marketable securities, and offset by an approximately $42.8 million net loss and $9.1 million deferred income tax benefit.

Net cash used in operating activities was approximately $34.9 million for the year ended December 31, 2016 and funded our research and development program build out and general and administrative expenses. Net cash used in continuing operations for the year ended December 31, 2016 was primarily driven by an approximately $44.3 million net loss and $0.6 million deferred income tax benefit, and offset by approximately $5.0 million non-cash expense recorded for the stock-based compensation, an approximately $3.8 million increase in cash from changes in operating assets and liabilities and $1.1 million realized loss from marketable securities.

Net cash used in operating activities was approximately $18.7 million for the year ended December 31, 2015 and funded our research and development program build out and general and administrative expenses. Net cash used in continuing operations for the year ended December 31, 2015 was primarily driven by an approximately $28.5 million net loss and offset by approximately $7.9 million non-cash expense recorded for the stock-based compensation, plus an approximately $1.8 million increase in cash from changes in operating assets and liabilities.

Net Cash Provided by (Used in) Investing Activities

Net cash used in investing activities from continuing operations for the year ended December 31, 2017 was approximately $15.6 million primarily due to the purchase of approximately $48.2 million of marketable securities and $0.9 million of fixed assets, and offset by a $33.5 million redemption of marketable securities during the year.

Net cash provided by investing activities from continuing operations for the year ended December 31, 2016 was approximately $36.2 million primarily due to the purchase of approximately $8.0 million of marketable securities and offset by a $44.3 million redemption of marketable securities during the year.

Net cash used in investing activities from continuing operations for the year ended December 31, 2015 was approximately $64.9 million primarily due to the purchase of approximately $69.8 million of marketable securities and offset by a $5.0 million redemption of marketable securities during the year.

Net Cash Provided by Financing Activities

Net cash flows provided by financing activities from continuing operations in the year ended December 31, 2017 was generated by the net proceeds of approximately $64.8 million in our public offering of 2,541,500 shares of common stock, including 331,500 shares of common stock purchased by the underwriters pursuant to their 30-day option to purchase additional shares, and approximately $2.4 million from the exercise of stock options to purchase 353,612 shares of common stock resulting in 349,720 shares issued due to utilization of net exercise provisions by some option holders.

Net cash flows provided by financing activities from continuing operations in the year ended December 31, 2016 was approximately $153,000 from the exercise of stock options to purchase 21,200 shares of common stock.

Net cash flows provided by financing activities from continuing operations in the year ended December 31, 2015 was primarily generated by the net proceeds of approximately $81.0 million from our public offering of 6,388,888 shares of common stock, including 833,333 shares of common stock purchased by the underwriters pursuant to their 30-day option to purchase additional shares, and approximately $554,000 from the exercise of stock options to purchase 76,422 shares of common stock.

Funding Requirements

We expect our expenses to increase in connection with our ongoing activities, particularly as we continue the research, development and clinical trials of our product candidates and pursue our intellectual property strategy. Furthermore, we expect to continue to incur additional costs associated with operating as a public company. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. If we are unable to raise capital when needed or on attractive terms, we would be forced to delay, reduce or eliminate our research and development programs or future commercialization efforts.

We monitor our cash needs and the status of the capital markets on a continuous basis. From time to time, we opportunistically raise capital and have done so numerous times since our initial public offering by issuing equity securities, most recently in November 2017. We expect to continue to raise capital when and as needed and at the time and in the manner most advantageous to us.

We expect that our existing cash, cash equivalents and marketable securities, will enable us to fund our operating expenses and capital expenditure requirements for at least the next twelve months. Our future capital requirements will depend on many factors, including:

- the scope, progress, results and costs of our ongoing drug discovery, nonclinical development, laboratory testing and clinical trials of our product candidates and any additional clinical trials we may conduct in the future;
- the extent to which we further acquire or in-license other medicines and technologies;
- our ability to manufacture, and to contract with third parties to manufacture, adequate supplies of our product candidates for our clinical trial and any eventual commercialization;
- the costs, timing and outcome of regulatory review of our product candidates;
- the costs of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending intellectual property-related claims; and
- our ability to establish and maintain collaborations on favorable terms, if at all.

Identifying potential product candidates and conducting nonclinical testing and clinical trials is a time-consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain marketing approval and achieve product sales. In addition, our product candidates, if approved, may not achieve commercial success. Our commercial revenues, if any, will be derived from sales of medicines that we do not expect to be commercially available for many years, if at all. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives. Adequate additional financing may not be available to us on acceptable terms, or at all.

Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances and licensing arrangements. We do not have any committed external source of funds. To the extent that we raise additional capital through the sale of equity or convertible debt securities, the ownership interest of our stockholders will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of our common stockholders. Debt financing, if available, 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.

If we raise funds through additional collaborations, strategic alliances or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or to grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

Recent Accounting Pronouncements

See Note 2 of notes to the consolidated financial statements for a discussion of recent accounting standards and pronouncements.

Cautionary Statement

We operate in a highly competitive environment that involves a number of risks, some of which are beyond our control. The following statement highlights some of these risks. For more detail, see "Item 1A. Risk Factors."

Statements contained in this Form 10-K that are not historical facts, are or might constitute forward-looking statements under the safe harbor provisions of the Private Securities Litigation Reform Act of 1995. Although we believe the expectations reflected in such forward-looking statements are based on reasonable assumptions, our expectations might not be attained. Forward-looking statements involve known and unknown risks that could cause actual results to differ materially from expected results. Factors that could cause actual results to differ materially from our expectations expressed in the report include, among others: risks related to the costs, timing, regulatory review and results of our nonclinical studies and clinical trials; our ability to obtain FDA approval of our product candidates; our anticipated capital expenditures, our estimates regarding our capital requirements, and our need for future capital; our liquidity and working capital requirements; our expectations regarding our revenues, expenses and other results of operations; the unpredictability of the size of the markets for, and market acceptance of, any of our products; our ability to sell any approved products and the price we are able realize; our ability to establish and maintain collaborations on favorable terms; our ability to obtain future funding on acceptable terms; our ability to hire and retain necessary employees and to staff our operations appropriately; our ability to compete in our industry and innovation by our competitors; our ability to stay abreast of and comply with new or modified laws and regulations that currently apply or become applicable to our business; estimates and estimate methodologies used in preparing our financial statements; the future trading prices of our common stock and the impact of securities analysts' reports on these prices; and the risks set out in our filings with the SEC.

Item 7A. Quantitative and Qualitative Disclosures about Market Risk

Our primary exposure to market risk is interest income sensitivity, which is affected by changes in the general level of U.S. interest rates.

We do not believe that our cash and equivalents have significant risk of default or illiquidity. Under our current investment policies, we invest our cash and cash equivalents in money market funds which invest in short-term U.S. Treasury securities with insignificant rates of return. We also invest our cash and cash equivalents in readily marketable, high-quality securities that are diversified and structured to minimize market risks. Our exposure to market risk for changes in interest rates relates primarily to our investments in marketable securities. Marketable securities held in our investment portfolio are subject to changes in market value in response to changes in interest rates and liquidity. A significant change in market interest rates could have a material impact on interest income earned from our investment portfolio. Changes in interest rates may affect the fair value of our investment portfolio; however, we will not recognize such gains or losses in our statement of operations and comprehensive income (loss) unless the investments are sold.

While we believe our cash and equivalents do not contain excessive risk, we cannot provide absolute assurance that in the future our investments will not be subject to adverse changes in market value. In addition, we maintain significant amounts of cash and equivalents at one or more financial institutions that are in excess of federally insured limits.

Inflation generally affects us by increasing our cost of labor and clinical trial costs. We do not believe that inflation has had a material effect on our results of operations during 2017 or 2016.

Item 8. Financial Statements and Supplementary Data

The financial statements required to be filed pursuant to this Item 8 are appended to this report. An index of those financial statements is found on page F-1.

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

None.

Item 9A. Controls and Procedures

Evaluation of Disclosure Controls and Procedures

We maintain a system of disclosure controls and procedures, as defined in Rule 13a-15(e) promulgated under the Securities and Exchange Act of 1934, as amended (the Exchange Act), which is designed to provide reasonable assurance that information, which is required to be disclosed in our reports filed pursuant to the Exchange Act, is accumulated and communicated to management in a timely manner. At the end of fiscal year ending December 31, 2017, we carried out an evaluation, under the supervision, and with the participation of, our management, including our Chief Executive Officer and our Chief Financial Officer, of the effectiveness of the design and operation of our disclosure controls and procedures pursuant to Exchange Act Rule 13a-15(b). Based upon that evaluation, our Chief Executive Officer and Chief Financial Officer concluded that our disclosure controls and procedures for the fiscal year ending as of December 31, 2017 were effective at reasonable assurance levels.

Management's Annual Report on Internal Control over Financial Reporting

Our management is responsible for establishing and maintaining adequate internal control over financial reporting, as such term is defined in Exchange Act Rule 13a-15(f). Our internal control over financial reporting is designed to provide reasonable assurance to our management and Board of Directors regarding the preparation and fair presentation of published financial statements. A control system, no matter how well designed and operated, can only provide reasonable, not absolute, assurance that the objectives of the control system are met. Because of these inherent limitations, management does not expect that our internal controls over financial reporting will prevent all error and all fraud. Under the supervision and with the participation of our management, including our Chief Executive Officer and our Chief Financial Officer, we conducted an evaluation of the effectiveness of our internal control over financial reporting based on the framework in Internal Control-Integrated Framework issued in 2013 by the Committee of Sponsoring Organizations of the Treadway Commission. Based on our evaluation under the framework in Internal Control-Integrated Framework, our management concluded that our internal control over financial reporting was effective as of December 31, 2017.

Our independent registered public accounting firm, Ernst & Young LLP, has audited the financial statements included in this Form 10-K and has issued an unqualified opinion on the effectiveness of our internal control over financial reporting as of December 31, 2017. The report of Ernst & Young LLP is included with the financial statements appended to this Form 10-K pursuant to Item 8.

Changes in Internal Control over Financial Reporting

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

Item 9B. Other Information

Not applicable.

PART III

ITEM 10. Directors, Executive Officers and Corporate Governance

Except as set forth below, the information required by this item will be contained in our definitive proxy statement to be filed with the SEC in connection with the Annual Meeting of Stockholders (Proxy Statement) within 120 days after the conclusion of our fiscal year ended December 31, 2017 and is incorporated in this Annual Report on Form 10-K by reference.

Code of Ethics

Our Board has adopted a Code of Ethics for our principal executive officer and all senior financial officers and a Code of Conduct applicable to all of our employees and our directors. Both Codes are available under the Investors Corporate Governance section of our website at www.assemblybio.com. If we make any substantive amendments to, or grant any waivers from, the Code of Ethics for our principal executive officer, principal financial officer, principal accounting officer, controller or persons performing similar functions, or any officer or director, we will disclose the nature of such amendment or waiver on our website or in a current report on Form 8-K.

Section 16(a) Beneficial Ownership Reporting Compliance

Pursuant to Section 16(a) of the Securities Exchange Act, our directors and executive officers are required to file reports with the SEC indicating their holdings of and transactions in our equity securities. To our knowledge, based solely on our review of the copies of such reports furnished to us and written representations that our officers, directors and holders of more than 10% of our common stock complied with all applicable filing requirements during the fiscal year ended December 31, 2017.

ITEM 11. Executive Compensation

The information required by this item will be contained in the Proxy Statement and is incorporated in this Annual Report on Form 10-K by reference.

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

Except for the table regarding equity compensation plans, which is included in Part II Item 5 of this Annual Report on Form 10-K, the information required by this item will be contained in the Proxy Statement and is incorporated in this Annual Report on Form 10-K by reference.

ITEM 13. Certain Relationships and Related Party Transactions, and Director Independence

The information required by this item will be contained in the Proxy Statement and is incorporated in this Annual Report on Form 10-K by reference.

ITEM 14. Principal Accounting Fees and Services

The information required by this item will be contained in the Proxy Statement and is incorporated in this Annual Report on Form 10-K by reference.

ITEM 15. Exhibits, Financial Statement Schedules

(a) *Exhibits.* The following exhibits are filed as part of this registration statement:

Exhibit Number	Description of Document	Registrant's Form	Dated	Exhibit No.	Filed Herewith
3.1	Amended and Restated Certificate of Incorporation dated November 11, 2010.	S-1/A	11/16/2010	3.1	
3.2	Certificate of Amendment of the Third Amended and Restated Certificate of Incorporation, dated July 11, 2014.	8-K	7/10/2014	3.1	
3.3	Amended and Restated Bylaws dated January 24, 2018.	8-K	1/24/2018	3.1	
4.1	Specimen of Common Stock Certificate.	S-3	12/30/2015	4.1	
10.1*	Exclusive License Agreement dated September 3, 2013 by and between The Indiana University Research and Technology Corporation and Assembly Pharmaceuticals, Inc.	10-Q	11/17/2014	10.29	
10.2*	License and Collaboration Agreement dated November 8, 2013, by and between Ventrus Biosciences, Inc. and Therabiome, LLC.	10-K	3/31/2014	10.22	
10.3*	Research, Development, Collaboration and License Agreement dated January 6, 2017 between Assembly Biosciences, Inc. and Allergan Pharmaceuticals International Limited.	10-Q	5/8/2017	10.1	
10.4#	Employment Agreement, dated July 11, 2014, between Ventrus Biosciences, Inc. and Derek A. Small.	8-K	07/14/2014	10.24	
10.5#	Employment Agreement dated January 15, 2014 and effective December 22, 2013, by and between Ventrus Biosciences, Inc. and David J. Barrett.	8-K	01/16/2014	10.21	
10.6*	Employment Agreement, dated December 17, 2015 and effective January 5, 2016, between Assembly Biosciences, Inc. and Richard Colonno, Ph.D.	10-Q	11/9/2016	10.1	
10.7#	Employment Agreement, dated July 11, 2014, between Ventrus Biosciences, Inc. and Uri A. Lopatin.	8-K	07/14/2014	10.25	
10.8#	Employment Offer Letter dated January 9, 2016 between Assembly Biosciences, Inc. and Thomas E. Rollins.	10-K	3/2/2017	10.12	
10.9#	2010 Equity Incentive Plan	S-1/A	10/4/2010	10.14	
10.10#	Assembly Biosciences, Inc. Amended and Restated 2014 Stock Incentive Plan.	8-K	6/6/2016	10.1	
10.11#	Assembly Biosciences, Inc. 2017 Inducement Award Plan (the 2017 Inducement Award Plan).	10-Q	08/09/2017	10.1	

Exhibit Number	Description of Document	Registrant's Form	Dated	Exhibit No.	Filed Herewith
10.12#	Form of Notice of Stock Option Grant and Stock Option Agreement under the 2017 Inducement Award Plan.	10-Q	08/09/2017	10.2	
10.13#	Form of Restricted Stock Unit Award Notice and Restricted Stock Unit Award Agreement under the 2017 Inducement Award Plan.	10-Q	08/09/2017	10.3	
10.14#	Form of Restricted Stock Award Notice and Restricted Stock Unit Award Agreement under the Amended and Restated 2014 Stock Incentive Plan.	10-Q	11/01/2017	10.1	
10.15#	Assembly Biosciences, Inc. 2018 Discretionary Bonus Plan.	8-K	12/12/2017	10.1	
10.16#	Consulting Agreement, dated October 30, 2017 and effective November 1, 2017, between Assembly Biosciences, Inc. and Miguel S. Barbosa, Ph.D.				X
10.17#	First Amendment to Stock Option Agreement, effective as of November 1, 2017, between Assembly Biosciences, Inc. and Miguel S. Barbosa, Ph.D.				X
21	List of Subsidiaries of Assembly Biosciences, Inc.				X
23.1	Consent of Ernst & Young LLP, Independent Registered Public Accounting Firm.				X
24	Power of Attorney (included on signature page)				X
31.1	Certification of the Chief Executive Officer Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.				X
31.2	Certification of the Chief Financial Officer Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.				X
32.1	Certification of the Chief Executive Officer Pursuant to 18 U.S. C. Section 1350 as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.				X
32.2	Certification of the Chief Financial Officer Pursuant to 18 U.S. C. Section 1350 as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.				X
101.INS	XBRL Instance Document.				
101.SCH	XBRL Taxonomy Extension Schema Document.				
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document.				
101.DEF	XBRL Taxonomy Extension Definitions Linkbase Document.				

Exhibit Number	Description of Document	Registrant's Form	Dated	Exhibit No.	Filed Herewith
101.LAB	XBRL Taxonomy Extension Label Linkbase Document.				
101.PRE	XBRL Taxonomy Extension Presentation Linkbase Document.				

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* Certain information in this exhibit has been omitted and filed separately with the Securities and Exchange Commission pursuant to a confidential treatment request.

\# Represents management contracts or compensatory plans or arrangements.

Item 16. Form 10-K Summary.

None

SIGNATURES

In accordance with Section 13 or 15(d) of the Securities Exchange Act, the Registrant caused this report to be signed on its behalf by the undersigned, thereunto duly authorized.

ASSEMBLY BIOSCIENCES, INC.

Date: March 7, 2018

By: /s/ Derek A. Small
Name: Derek A. Small
Title: President and Chief Executive Officer

KNOW ALL PERSONS BY THESE PRESENTS, that each person whose signature appears below constitutes and appoints Derek A. Small, David J. Barrett and Elizabeth H. Lacy, jointly and severally, his or her attorneys-in-fact, each with the power of substitution, for him or her in any and all capacities, to sign any amendments to this report, and to file the same, with exhibits thereto and other documents in connection therewith with the Securities and Exchange Commission, hereby ratifying and confirming all that each of said attorneys-in-fact, or his or her substitute or substitutes may do or cause to be done by virtue hereof.

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

Signature	Title	Date
/s/ Derek A. Small Derek A. Small	President, Chief Executive Officer and Director (Principal Executive Officer)	March 7, 2018
/s/ David J. Barrett David J. Barrett	Chief Financial Officer and Chief Operating Officer (Principal Financial and Accounting Officer)	March 7, 2018
/s/ Anthony E. Altig Anthony E. Altig	Director	March 7, 2018
/s/ Mark Auerbach Mark Auerbach	Director	March 7, 2018
/s/ Richard D. DiMarchi, Ph.D. Richard D. DiMarchi, Ph.D.	Director	March 7, 2018
/s/ Myron Z. Holubiak Myron Z. Holubiak	Director	March 7, 2018
/s/ Alan J. Lewis, Ph.D. Alan J. Lewis, Ph.D.	Director	March 7, 2018
/s/ Susan Mahony, Ph.D. Susan Mahony, Ph.D.	Director	March 7, 2018
/s/ William R. Ringo, Jr. William R. Ringo, Jr.	Director	March 7, 2018

ASSEMBLY BIOSCIENCES, INC.
FINANCIAL STATEMENTS

REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

To the Stockholders and the Board of Directors of Assembly Biosciences, Inc.

Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheets of Assembly Biosciences, Inc. and Subsidiaries, (the Company) as of December 31, 2017 and 2016, and the related consolidated statements of operations and comprehensive loss, changes in stockholders' equity and cash flows for each of the three years in the period ended December 31, 2017, and the related notes (collectively referred to as the "financial statements"). In our opinion, the financial statements present fairly, in all material respects, the consolidated financial position of the Company at December 31, 2017 and 2016, and the consolidated results of their operations and their cash flows for each of the three years in the period ended December 31, 2017, in conformity with U.S. generally accepted accounting principles.

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States) (PCAOB), the Company's internal control over financial reporting as of December 31, 2017, based on criteria established in Internal Control-Integrated Framework issued by the Committee of Sponsoring Organizations of the Treadway Commission (2013 framework), and our report dated March 7, 2018 expressed an unqualified opinion thereon.

Basis for Opinion

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

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

/s/ Ernst & Young LLP

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

Stamford, Connecticut
March 7, 2018

REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

To the Stockholders and the Board of Directors of Assembly Biosciences, Inc.

Opinion on Internal Control over Financial Reporting

We have audited Assembly Biosciences, Inc. and Subsidiaries' internal control over financial reporting as of December 31, 2017, based on criteria established in Internal Control — Integrated Framework issued by the Committee of Sponsoring Organizations of the Treadway Commission (2013 framework), (the COSO criteria). In our opinion, Assembly Biosciences, Inc. and subsidiaries (the Company) maintained, in all material respects, effective internal control over financial reporting as of December 31, 2017, based on the COSO criteria.

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States) (PCAOB), the consolidated balance sheets of the Company as of December 31, 2017 and 2016, and the related consolidated statements of operations and comprehensive loss, changes in stockholders' equity and cash flows for each of the three years in the period ended December 31, 2017, and the related notes and our report dated March 7, 2018 expressed an unqualified opinion thereon.

Basis for Opinion

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

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

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

Definition and Limitations of Internal Control Over Financial Reporting

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

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

/s/ Ernst & Young LLP

Stamford, Connecticut
March 7, 2018

ASSEMBLY BIOSCIENCES, INC. AND SUBSIDIARIES

CONSOLIDATED BALANCE SHEETS

	As of December 31,	
	2017	**2016**
ASSETS		
Current assets		
Cash and cash equivalents	$ 82,033,209	$ 28,575,085
Marketable securities, at fair value	37,914,482	24,388,403
Accounts receivable from collaboration	2,273,421	—
Prepaid expenses and other current assets	897,400	611,176
Total current assets	123,118,512	53,574,664
Long-term assets		
Marketable securities, at fair value	3,347,213	2,435,753
Property, plant and equipment, net	860,026	214,687
Security deposits	339,558	255,366
Intangible assets	29,000,000	29,000,000
Goodwill	12,638,136	12,638,136
Total long-term assets	46,184,933	44,543,942
Total assets	**$ 169,303,445**	**$ 98,118,606**
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities		
Accounts payable	$ 2,123,939	$ 2,368,131
Accrued expenses	6,139,000	4,752,823
Deferred revenue – short-term	5,229,227	—
Total current liabilities	13,492,166	7,120,954
Long-term liabilities		
Deferred tax liabilities	2,135,802	11,119,651
Deferred revenue – long-term	40,555,708	—
Total long-term liabilities	42,691,510	11,119,651
Total liabilities	**56,183,676**	**18,240,605**
Commitments and contingencies		
Stockholders' equity		
Preferred stock, $0.001 par value; 5,000,000 shares authorized; 0 shares issued and outstanding	—	—
Common stock, $0.001 par value; 50,000,000 shares authorized; 20,137,974 and 17,246,754 shares issued and outstanding as of December 31, 2017 and December 31, 2016, respectively	20,138	17,247
Additional paid-in capital	364,528,037	288,688,990
Accumulated other comprehensive loss	(392,391)	(600,769)
Accumulated deficit	(251,036,015)	(208,227,467)
Total stockholders' equity	113,119,769	79,878,001
Total liabilities and stockholders' equity	**$ 169,303,445**	**$ 98,118,606**

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

ASSEMBLY BIOSCIENCES, INC. AND SUBSIDIARIES

CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS

	Year Ended December 31,		
	2017	2016	2015
Collaboration revenue	$ 9,018,750	$ —	$ —
Operating expenses:			
Research and development	44,225,021	33,092,977	18,357,937
General and administrative	17,020,607	12,185,484	11,297,693
Total operating expenses	61,245,628	45,278,461	29,655,630
Loss from operations	**(52,226,878)**	**(45,278,461)**	**(29,655,630)**
Other income (expenses)			
Interest and other income	983,209	1,539,088	1,228,830
Realized loss from marketable securities	(615,128)	(1,139,861)	(27,033)
Total other income	368,081	399,227	1,201,797
Loss before income taxes	**(51,858,797)**	**(44,879,234)**	**(28,453,833)**
Income tax benefit	9,050,249	617,672	—
Net loss	**$(42,808,548)**	**$(44,261,562)**	**$(28,453,833)**
Other comprehensive (loss) income			
Unrealized loss recognized in accumulated other comprehensive loss before reclassification, net of tax benefit of $82,245, $299,741 and $0, respectively	(258,105)	(481,981)	(848,618)
Reclassification adjustment of unrealized loss included in net loss, net of tax expense of $148,645, $437,064 and $0, respectively	466,483	702,797	27,033
Comprehensive loss	**$(42,600,170)**	**$(44,040,746)**	**$(29,275,418)**
Net loss per share, basic and diluted	$ (2.41)	$ (2.57)	$ (1.81)
Weighted average common shares outstanding, basic and diluted	17,750,380	17,226,245	15,702,646

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

ASSEMBLY BIOSCIENCES, INC. AND SUBSIDIARIES

CONSOLIDATED STATEMENTS OF CHANGES IN STOCKHOLDERS' EQUITY

	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balance as of December 31, 2014	**10,672,059**	**$10,672**	**$194,072,572**	**$ —**	**$(135,512,072)**	**$ 58,571,172**
Proceeds from common stock sold, net of underwriters' discounts and cost	6,388,888	6,389	81,008,600	—	—	81,014,989
Exercise of stock options	76,422	77	554,191	—	—	554,268
Cashless exercise of warrants	88,293	88	(88)	—	—	—
Stock-based compensation	—	—	7,876,584	—	—	7,876,584
Change in unrealized loss on marketable securities	—	—	—	(821,585)	—	(821,585)
Net loss	—	—	—	—	(28,453,833)	(28,453,833)
Balance as of December 31, 2015	**17,225,662**	**$17,226**	**$283,511,859**	**$(821,585)**	**$(163,965,905)**	**$118,741,595**
Exercise of stock options	21,200	21	152,619	—	—	152,640
Stock-based compensation	—	—	5,024,512	—	—	5,024,512
Change in unrealized loss on marketable securities, net of income tax expense of $137,323	—	—	—	220,816	—	220,816
Cancellation of common stock	(108)	—	—	—	—	—
Net loss	—	—	—	—	(44,261,562)	(44,261,562)
Balance as of December 31, 2016	**17,246,754**	**$17,247**	**$288,688,990**	**$(600,769)**	**$(208,227,467)**	**$ 79,878,001**
Proceeds from common stock sold, net of underwriters' discounts and cost	2,541,500	2,541	64,844,813	—	—	64,847,354
Proceeds from the exercise of stock options	349,720	350	2,392,792	—	—	2,393,142
Change in unrealized gain on marketable securities, net of income tax expense of $66,400	—	—	—	208,378	—	208,378
Stock-based compensation	—	—	8,601,442	—	—	8,601,442
Net loss	—	—	—	—	(42,808,548)	(42,808,548)
Balance as of December 31, 2017	**20,137,974**	**$20,138**	**$364,528,037**	**$(392,391)**	**$(251,036,015)**	**$113,119,769**

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

ASSEMBLY BIOSCIENCES, INC. AND SUBSIDIARIES

CONSOLIDATED STATEMENTS OF CASH FLOWS

	Year Ended December 31,		
	2017	2016	2015
Cash flows from operating activities			
Net loss	$(42,808,548)	$(44,261,562)	$(28,453,833)
Adjustments to reconcile net loss to net cash used in operating activities:			
Depreciation and amortization	219,225	80,251	64,989
Stock-based compensation	8,601,442	5,024,512	7,876,584
Realized loss from marketable securities	615,128	1,139,861	27,033
Deferred income tax benefit	(9,050,249)	(617,672)	—
Loss on sale of fixed assets	—	—	954
Changes in operating assets and liabilities:			
Accounts receivable from collaboration	(2,273,421)	—	—
Prepaid expenses and other current assets	(286,224)	93,111	(555,849)
Accounts payable	(244,192)	1,004,433	456,097
Accrued expenses	1,386,177	2,713,619	1,968,844
Deferred revenue	45,784,935	—	—
Security deposits	(84,192)	(58,208)	(82,153)
Net cash provided by (used in) operating activities	1,860,081	(34,881,655)	(18,697,334)
Cash flows from investing activities			
Purchases of fixed assets	(864,564)	(146,329)	(58,261)
Sale of fixed assets	—	—	150
Purchases of marketable securities	(48,234,454)	(7,951,257)	(69,781,176)
Redemptions of marketable securities	33,456,565	44,294,160	4,983,777
Net cash (used in) provided by investing activities	(15,642,453)	36,196,574	(64,855,510)
Cash flows from financing activities			
Proceeds from common stock sold, net of underwriters' discounts and cost	64,847,354	—	81,014,989
Proceeds from the exercise of stock options	2,393,142	152,640	554,268
Net cash provided by financing activities	67,240,496	152,640	81,569,257
Net increase (decrease) in cash and cash equivalents	53,458,124	1,467,559	(1,983,587)
Cash and cash equivalents at the beginning of the period	28,575,085	27,107,526	29,091,113
Cash and cash equivalents at the end of the period	**$ 82,033,209**	**$ 28,575,085**	**$ 27,107,526**
Supplemental disclosure of cash flow information:			
Change in unrealized gain (loss) on marketable securities available-for-sale, before tax expense	$ 274,778	$ 358,139	$ (821,585)
Cashless exercise of warrants	$ —	$ —	$ 88
Supplemental disclosure of non-cash activities:			
Assembly business combination			
Goodwill	$ —	$ —	$ 99,214
Accounts payable and accrued expenses	—	—	(99,214)
Cash acquired in business combination	$ —	$ —	$ —

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

Note 1 — Nature of Business

Overview

Assembly Biosciences, Inc., together with its subsidiaries (Assembly or the Company), is a clinical stage biotechnology company advancing two innovative platform programs: a new class of oral therapeutic candidates for the treatment of hepatitis B virus (HBV) infection and a novel class of oral synthetic live biotherapeutic candidates, which are designed to treat disorders associated with the microbiome.

Over 250 million people worldwide are chronically infected with HBV. The Company's HBV-cure program is pursuing multiple drug candidates that inhibit the HBV lifecyle and block the generation of covalently closed circular DNA (cccDNA), with the aim of increasing the current low cure rates for patients with HBV. Assembly has discovered multiple novel core protein Allosteric Modulators (CpAMs), which are small molecules that directly target and allosterically modulate the HBV core (HBc) protein.

The Company's Microbiome program consists of a fully integrated platform that includes a disease targeted strain identification and selection process, methods for strain isolation and growth under current Good Manufacturing Practices (cGMP) conditions, and a patent-pending delivery system that we call GEMICEL®, which is designed to allow for targeted oral delivery of live biologic and conventional therapies to the lower gastrointestinal (GI) tract. Using its microbiome platform, the Company is developing product candidates for various disease indications, including ulcerative colitis, Crohn's disease, irritable bowel syndrome, non-alcoholic steatohepatitis (NASH), immuno-oncology and Clostridium difficile infections (CDI), which we will develop either internally or in collaboration with partners.

On January 6, 2017, the Company entered into a Research, Development, Collaboration and License Agreement (the Collaboration Agreement) with Allergan Pharmaceuticals International Limited (Allergan) to develop and commercialize select microbiome gastrointestinal programs. Pursuant to the terms of the Collaboration Agreement, in connection with the closing of the transaction on February 10, 2017, Allergan paid the Company an upfront payment of $50 million. Additionally, the Company is eligible to receive up to approximately $630 million in payments related to seven development milestones and up to approximately $2.15 billion in payments related to 12 commercial development and sales milestones in connection with the successful development and commercialization of licensed compounds for up to six different indications (see Note 8). Allergan and the Company have agreed to share development costs up to an aggregate of $75 million through proof-of-concept (POC) studies on a ⅔, ⅓ basis, respectively, and Allergan has agreed to assume all post-POC development costs. Additionally, the Company has an option to co-promote the licensed programs in the United States and China, subject to certain conditions set forth in the Collaboration Agreement.

On November 1, 2017, the Company sold to various investors an aggregate of 2,210,000 shares of common stock in a public offering. The purchase price paid by investors was $27.25 per share and an aggregate of $64.8 million (net of underwriting discounts and commissions and offering expenses) was received, which includes proceeds received pursuant to the underwriters' exercise of their 30-day option to purchase of up to an additional 331,500 shares in full, which occurred on November 2, 2017.

Liquidity

The Company has not derived any revenue from product sales to date as it currently has no approved products. Once a product has been developed, it will need to be approved for sale by the U.S. Food and Drug Administration (FDA) or an applicable foreign regulatory agency. Since inception, the Company's operations have been financed primarily through the sale of equity securities, the proceeds from the exercise of warrants and stock options, the issuance of debt and an up-front payment related to the Collaboration Agreement. The Company has incurred losses from operations and negative cash flows from operating activities since inception and expects to continue to incur substantial losses for the next several years as it continues its product development efforts. Management believes the Company currently has sufficient funds

to meet its operating requirements for at least the next twelve months. If the Company cannot generate significant cash from its operations, it intends to obtain any additional funding it requires through strategic relationships, public or private equity or debt financings, grants or other arrangements. The Company cannot assure such funding will be available on reasonable terms, if at all.

Note 2 — Summary of Significant Accounting Policies and Recent Accounting Pronouncements

Basis of Presentation

The accompanying consolidated financial statements include the accounts of the Company and its subsidiaries. All intercompany balances and transactions have been eliminated in consolidation.

Segments

The Company operates in one operating segment and, accordingly, no segment disclosures have been presented herein.

Use of Estimates

The preparation of consolidated financial statements in conformity with the accounting principles generally accepted in the United States of America (U.S. GAAP) requires management to make estimates and assumptions that may affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the date of the financial statements and reported amounts of expenses during the reporting period. Actual results could differ from those estimates.

Significant estimates inherent in the preparation of the accompanying consolidated financial statements include recoverability and useful lives (indefinite or finite) of intangible assets, assessment of impairment of goodwill, provisions for income taxes, and the fair value of stock options and warrants granted to employees, consultants, directors, investors, licensors, placement agents and underwriters. In addition, with the Company entering into the Collaboration Agreement in 2017, the Company believes its consolidated financial statements are also impacted by the following accounting estimates and judgments: (i) identifying deliverables under collaboration agreements involving multiple elements and determining whether such deliverables are separable from other aspects of the contractual relationship; (ii) estimating the selling price of deliverables for the purpose of allocating arrangement consideration for revenue recognition; and (iii) estimating the periods over which the allocated consideration for deliverables is recognized.

The Company's estimates could be affected by external conditions, including those unique to the Company and general economic conditions. It is reasonably possible that these external factors could have an effect on the Company's estimates and could cause actual results to differ from those estimates and assumptions.

Cash and cash equivalents

All highly liquid investments with maturities of three months or less at the time of purchase are considered to be cash equivalents. All of the Company's cash equivalents have liquid markets and high credit ratings. The Company maintains its cash in bank deposits and other accounts, the balances of which, at times and at December 31, 2017 and 2016, exceed federally insured limits.

Marketable Securities

The Company has designated marketable securities as of December 31, 2017 and 2016 as available-for-sale securities and measures these securities at their respective fair values. Marketable securities are classified as short-term or long-term based on the maturity date and their availability to meet current operating requirements. Marketable securities that mature in one year or less are classified as short-term available-for-sale securities and are reported as a component of current assets. Marketable securities that are not considered available for use in current operations are classified as long-term available-for-sale securities and are reported as a component of long-term assets.

Securities that are classified as available-for-sale are measured at fair value with temporary unrealized gains and losses reported in other comprehensive loss, and as a component of stockholders' equity until their disposition. The Company reviews all available-for-sale securities at each period end to determine if they remain available-for-sale based on their current intent and ability to sell the security if it is required to do so. The cost of securities sold is based on the specific identification method.

Marketable securities are subject to a periodic impairment review. The Company may recognize an impairment charge when a decline in the fair value of investments below the cost basis is determined to be other-than-temporary. There were no marketable securities deemed to be impaired as of December 31, 2017 or 2016.

Accounts Receivable

As of December 31, 2017, the accounts receivable relates to the Company's collaboration with Allergan. All accounts receivable are deemed collectible. There were no accounts receivable as of December 31, 2016.

Goodwill and Other Intangible Assets

Goodwill is the excess of purchase price over the fair value of identified net assets of businesses acquired. The Company's intangible assets with an indefinite life are related to in-process research and development (IPR&D) programs acquired in the Merger, as the Company expects future research and development on these programs to provide the Company with substantial benefit for a period that extends beyond the foreseeable horizon. Intangible assets with indefinite useful lives are measured at their respective fair values as of the acquisition date. The Company does not amortize goodwill and intangible assets with indefinite useful lives. Intangible assets related to IPR&D projects are considered to be indefinite lived until the completion or abandonment of the associated R&D efforts. If and when development is complete, which generally occurs if and when regulatory approval to market a product is obtained, the associated assets would be deemed finite lived and would then be amortized based on their respective estimated useful lives at that point in time.

The Company reviews goodwill and indefinite-lived intangible assets at least annually for possible impairment. Goodwill and indefinite-lived intangible assets are reviewed for possible impairment between annual tests if an event occurs or circumstances change that would more likely than not reduce the fair value of the reporting unit or the indefinite-lived intangible assets below their carrying values. The Company tests its goodwill and indefinite-lived intangible assets each year on October 1. The Company has one reporting unit. The Company tests goodwill for impairment by utilizing a market capitalization approach which was based upon the closing price of the Company's stock price as of the beginning of the 4^{th} quarter. As of October 1, 2017 and 2016, the fair value of the Company's reporting unit was in excess of carrying value and goodwill was not deemed to be impaired.

On October 1, 2017, the Company elected to bypass the qualitative assessment and performed a quantitative impairment test. On October 1, 2016, the Company performed a qualitative assessment of IPR&D. Significant assumptions used in the model include the period in which material net cash inflows are expected to commence, anticipated material changes from historical pricing, margins and expense levels and an appropriate risk adjusted discount rate applied to the estimated cash flows. As of October 1, 2017 and 2016, IPR&D was not deemed to be impaired.

Impairment of Long-lived Assets

The Company monitors the carrying value of long-lived assets for potential impairment and tests the recoverability of such assets whenever events or changes in circumstances indicate that the carrying amounts may not be recoverable. If a change in circumstance occurs, the Company performs a test of recoverability by comparing the carrying value of the asset or asset group to its undiscounted expected future cash flows. If cash flows cannot be separately and independently identified for a single asset, the Company will determine whether impairment has occurred for the group of assets for which the Company can identify the projected cash flows. If the carrying values are in excess of undiscounted expected future cash flows, the Company measures any impairment by comparing the fair value of the asset or asset group to its carrying value. There were no indicators of impairment of long-lived assets during the year ended December 31, 2017 and 2016.

Property and Equipment

Property and equipment are stated at cost and consist of lab equipment and computer hardware and software. The Company computes depreciation under the straight-line method over the following estimated useful life of the related assets:

- Lab equipment 3 to 5 years
- Computer hardware and software 3 years
- Office equipment 7 years

Leasehold improvements are amortized over the remaining terms of the respective leases or the estimated useful life of the leasehold improvements, whichever is less. Maintenance and repair costs are expensed as incurred.

Fair Value Measurements

The Company follows accounting guidance on fair value measurements for financial instruments measured on a recurring basis, as well as for certain assets and liabilities that are initially recorded at their estimated fair values. Fair value is defined as the exit price, or the amount that would be received from selling an asset or paid to transfer a liability in an orderly transaction between market participants at the measurement date. The Company use the following three-level hierarchy that maximizes the use of observable inputs and minimizes the use of unobservable inputs to value its financial instruments:

Level 1: Observable inputs such as unadjusted quoted prices in active markets for identical instruments.

Level 2: Quoted prices for similar instruments that are directly or indirectly observable in the marketplace.

Level 3: Significant unobservable inputs which are supported by little or no market activity and that are financial instruments whose values are determined using pricing models, discounted cash flow methodologies, or similar techniques, as well as instruments for which the determination of fair value requires significant judgment or estimation.

Financial instruments measured at fair value are classified in their entirety based on the lowest level of input that is significant to the fair value measurement. The assessment of the significance of a particular input to the fair value measurement in its entirety requires us to make judgments and consider factors specific to the asset or liability. The use of different assumptions and/or estimation methodologies may have a material effect on estimated fair values. Accordingly, the fair value estimates disclosed or initial amounts recorded may not be indicative of the amount that the Company or holders of the instruments could realize in a current market exchange.

The carrying amounts of cash equivalents and marketable securities approximate their fair value based upon quoted market prices. Certain of the Company's financial instruments are not measured at fair value on a recurring basis, but are recorded at amounts that approximate their fair value due to their liquid or short-term nature, such as cash, accounts receivable, accounts payable and accrued expenses.

Revenue Recognition

The Company recognizes revenue when each of the following four criteria is met: (i) persuasive evidence of an arrangement exists; (ii) products are delivered or services are rendered; (iii) the sales price is fixed or determinable; and (iv) collectability is reasonably assured.

The Company recognizes revenue under the Collaboration Agreement based on the relevant accounting literature. Under this guidance, multiple elements or deliverables may include (i) grants of licenses, or options to obtain licenses, to intellectual property, (ii) research and development services, (iii) participation on joint research and/or joint development committees, and/or (iv) manufacturing or supply of services. The payments entities may receive under these arrangements typically include one or more of the following: non-refundable, upfront license fees; option exercise fees; funding of research and/or development efforts; amounts due upon the achievement of specified objectives; and/or royalties on future product sales.

Multiple-element arrangements require the separability of deliverables included in an arrangement into different units of accounting and the allocation of arrangement consideration to the units of accounting. The evaluation of multiple-element arrangements requires management to make judgments about (i) the identification of deliverables, (ii) whether such deliverables are separable from the other aspects of the contractual relationship, (iii) the estimated selling price of each deliverable, and (iv) the expected period of performance for each deliverable.

To determine the units of accounting under a multiple-element arrangement, management evaluates certain separation criteria, including whether the deliverables have stand-alone value, based on the relevant facts and circumstances for each arrangement. Management then estimates the selling price for each unit of accounting and allocates the arrangement consideration to each unit using the relative selling price method. The relative selling price for each deliverable is determined using vendor specific objective evidence (VSOE), of selling price or third-party evidence of selling price if VSOE does not exist. If neither VSOE nor third-party evidence of selling price exists, we use our best estimate of the selling price for the deliverable. The allocated consideration for each unit of accounting is recognized based on the method most appropriate for that unit of accounting and in accordance with the revenue recognition criteria detailed above.

If there are deliverables in an arrangement that are not separable from other aspects of the contractual relationship, they are treated as a combined unit of accounting, with the allocated revenue for the combined unit recognized in a manner consistent with the revenue recognition applicable to the final deliverable in the combined unit. Payments received prior to satisfying the relevant revenue recognition criteria are recorded as deferred revenue in the accompanying consolidated balance sheets and recognized as revenue when the related revenue recognition criteria are met.

The Collaboration Agreement provides for non-refundable milestone payments. The Company recognizes revenue that is contingent upon the achievement of a substantive milestone in its entirety in the period in which the milestone is achieved. A milestone is considered substantive when the consideration payable to the Company for such milestone (i) is consistent with the Company's performance necessary to achieve the milestone or the increase in value to the collaboration resulting from the Company's performance, (ii) relates solely to the Company's past performance and (iii) is reasonable relative to all of the other deliverables and payments within the arrangement. In making this assessment, the Company considers all facts and circumstances relevant to the arrangement, including factors such as the scientific, regulatory, commercial and other risks that must be overcome to achieve the milestone, the level of effort and investment required to achieve the milestone and whether any portion of the milestone consideration is related to future performance or deliverables.

The Collaboration Agreement provides Allergan with options to license additional intellectual property rights, or purchase additional research, development, or supply services. The Company concluded that these were "substantive options" under the multiple-element arrangement guidance, and accordingly, associated fees have not been considered in allocating contract consideration among deliverables with stand-alone value. If Allergan exercises one or more of these options, the associated revenue would be recognized using the method most appropriate for the particular deliverable.

The Company will periodically review the estimated performance periods under the Collaboration Agreement, which provides for non-refundable upfront payments and fees. The Company will adjust the periods over which revenue should be recognized when appropriate to reflect changes in assumptions relating to the estimated performance periods. The Company could accelerate revenue recognition in the event of early termination of programs or if the Company's expectations change. Alternatively, the Company could decelerate revenue recognition if programs are extended or delayed. While such changes to the Company's estimates have no impact on the Company's consolidated cash flows, the amount of revenue recorded in future periods could be materially impacted.

The Company records revenues related to the reimbursement of costs incurred under the Collaboration Agreement where the Company acts as a principal, controls the research and development activities and bears credit risk. Under the Collaboration Agreement, the Company is reimbursed for associated out-of-pocket costs. The gross amount of these pass-through reimbursed costs is reported as revenue in the accompanying consolidated statements of operations, while the actual expenses for which the Company is

reimbursed are reflected as research and development costs. The Company has also accounted for the milestone payments under Accounting Standards Codification (ASC), 605 *Revenue Recognition — Milestone Method*. See Note 8 for further information.

Stock-Based Compensation

The Company expenses stock-based compensation to employees and Board members over the requisite service period based on the estimated grant-date fair value of the awards. Stock-based awards with graded-vesting schedules are recognized on a straight-line basis over the requisite service period for each separately vesting portion of the award. For stock-based compensation awards to non-employees, the Company remeasures the fair value of the non-employee awards at each reporting period prior to vesting and finally at the vesting date of the award. Changes in the estimated fair value of these non-employee awards are recognized as compensation expense in the period of change.

The Company estimates the fair value of stock option grants using the Black-Scholes option pricing model and the assumptions used in calculating the fair value of stock-based awards represent management's best estimates and involve inherent uncertainties and the application of management's judgment.

The fair value of restricted stock units is determined based on the number of shares granted and the quoted market price of the Company's common stock on the date of grant. The fair value of restricted stock units with performance conditions deemed probable of being achieved and vesting are amortized to expense over the requisite service period using the straight-line method of expense recognition.

Effective on January 1, 2017, the Company elected to account for forfeited awards as they occur as permitted by Accounting Standards Update ("ASU") 2016-09. Ultimately, the actual expenses recognized over the vesting period will be for those shares that vested. Prior to making this election, the Company estimated a forfeiture rate for awards at 0%, as the Company did not have a significant history of forfeitures.

Research and Development Costs

Research and development costs are expensed as incurred. Research and development costs include salaries and personnel-related costs, consulting fees, fees paid for contract research services, fees paid to clinical research organizations and other third parties associated with clinical trials, the costs of laboratory equipment and facilities, and other external costs.

Tax Assets and Liabilities and Income Taxes

The Company records income taxes using the asset and liability method. Deferred income tax assets and liabilities are recognized for the future tax effects attributable to temporary differences between the financial statement carrying amounts of existing assets and liabilities and their respective income tax bases, and operating loss and tax credit carryforwards. The Company establishes a valuation allowance if it is more likely than not that the deferred tax assets will not be recovered based on an evaluation of objective verifiable evidence. For tax positions that are more likely than not of being sustained upon audit, the Company recognizes the largest amount of the benefit that is greater than 50% likely of being realized. For tax positions that are not more likely than not of being sustained upon audit, the Company does not recognize any portion of the benefit.

The Financial Accounting Standards Board ("FASB") ASC Topic *740, Income Taxes* (ASC 740) also clarifies the accounting for uncertainty in income taxes recognized in an enterprise's financial statements and prescribes a recognition threshold and measurement process for financial statement recognition and measurement of a tax position taken or expected to be taken in a tax return. For those benefits to be recognized, a tax position must be more-likely-than-not to be sustained upon examination by taxing authorities. ASC 740 also provides guidance on derecoginition, classification, interest and penalties, accounting in interim period, disclosure and transition. Based on the Company's evaluation, it has been concluded that there are no significant uncertain tax positions requiring recognition in the Company's consolidated financial statements. The Company believes that its income tax positions and deductions would be sustained on audit and does not anticipate any adjustments that would result in a material changes to its financial position.

The Company's policy for recording interest and penalties associated with audits is to record such expense as a component of income tax expense. There were no amounts accrued for penalties or interest as of December 31, 2017 and 2016. Management is currently unaware of any issues under review that could result in significant payments, accruals or material deviations from its position.

On December 22, 2017, legislation commonly known as the Tax Cuts and Jobs Act, or the Tax Act, was signed in to law. The legislation significantly changes U.S. tax law by, among other things, lowering corporate income tax rates, implementing a territorial tax system and imposing a repatriation tax on deemed repatriated earnings of foreign subsidiaries. The Tax Act permanently reduces the U.S. corporate income tax rate to 21% from the existing applicable rate of 34%, effective January 1, 2018. As a result, the Company has recorded a decrease to its deferred tax assets of $24.7 million and to valuation allowance of $28.4 million, resulting in a net tax benefit of $3.7 million for the year ended December 31, 2017. The Tax Act also permits an indefinite carry forward of net operating losses generated in taxable years ending after December 31, 2017, subject to a utilization limitation of 80% of taxable income. Due to the change in the carry forward period for post-2017 net operating losses, the Company has determined that it would be able to use the deferred tax liability associated with certain in-process research and development as a source of income in determining the realizability of its deferred tax assets. As a result, the Company has recorded a $4.9 million income tax benefit from the reduction of its valuation allowance. For additional information, see Note 10.

On December 22, 2017, the SEC staff issued Staff Accounting Bulletin No. 118 ("SAB 118") to address the application of GAAP in situations when a registrant does not have the necessary information available, prepared, or analyzed (including computations) in reasonable detail to complete the accounting for certain income tax effects of the Tax Reform Act. The Company has recognized the provisional tax impacts related to the revaluation of deferred tax assets and liabilities and included these amounts in its consolidated financial statements for the year ended December 31, 2017. The ultimate impact may differ from these provisional amounts, possibly materially, due to, among other things, additional analysis, changes in interpretations and assumptions the Company has made, additional regulatory guidance that may be issued, and actions the Company may take as a result of the Tax Act. The accounting is expected to be complete when the 2017 U.S. corporate income tax return is filed in 2018.

Net Loss per Share of Common Stock

Basic net loss per common share excludes dilution and is computed by dividing net loss by the weighted average number of common shares outstanding during the period. Diluted net loss per common share reflects the potential dilution that could occur if securities or other contracts to issue common stock were exercised or converted into common stock or resulted in the issuance of common stock that then shared in the earnings of the entity unless inclusion of such shares would be anti-dilutive. Since the Company has only incurred losses, basic and diluted net loss per share is the same. Securities that could potentially dilute loss per share in the future that were not included in the computation of diluted loss per share at December 31, 2017, 2016 and 2015 are as follows:

	Year Ended December 31,		
	2017	2016	2015
Warrants to purchase common stock	15,296	16,909	16,909
Options to purchase common stock	4,551,819	4,457,251	3,367,784
Restricted stock units to purchase common stock	120,000	—	—
Total	**4,687,115**	**4,474,160**	**3,384,693**

Comprehensive Loss

Comprehensive loss is comprised of net loss and adjustments for the change in unrealized gains and losses on investments in available-for-sale marketable securities. The Company displays comprehensive loss and its components in the consolidated statements of operations and comprehensive loss.

Concentrations of Credit Risk

Financial instruments which potentially subject the Company to credit risk consist primarily of cash, cash equivalents and marketable securities. The Company holds these investments in highly rated financial

institutions, and, by policy, limits the amounts of credit exposure to any one financial institution. These amounts at times may exceed federally insured limits. The Company has not experienced any credit losses in such accounts and does not believe it is exposed to any significant credit risk on these funds. The Company has no off-balance sheet concentrations of credit risk, such as foreign currency exchange contracts, option contracts or other hedging arrangements.

Adoption of Recent Accounting Pronouncements

In March 2016, the FASB issued ASU 2016-09, *Compensation — Stock Compensation (Topic 718): Improvements to Employee Share-Based Payment Accounting*. Under ASU 2016-09, companies will no longer record excess tax benefits and certain tax deficiencies in additional paid-in capital (APIC). Instead, they will record all excess tax benefits and tax deficiencies as income tax expense or benefit in the income statement and the APIC pools will be eliminated. In addition, ASU 2016-09 eliminates the requirement that excess tax benefits be realized before companies can recognize them. ASU 2016-09 also requires companies to present excess tax benefits as an operating activity on the statement of cash flows rather than as a financing activity. Furthermore, ASU 2016-09 will increase the amount an employer can withhold to cover income taxes on awards and still qualify for the exception to liability classification for shares used to satisfy the employer's statutory income tax withholding obligation. An employer with a statutory income tax withholding obligation will now be allowed to withhold shares with a fair value up to the amount of taxes owed using the maximum statutory tax rate in the employee's applicable jurisdiction(s). ASU 2016-09 requires a company to classify the cash paid to a tax authority when shares are withheld to satisfy its statutory income tax withholding obligation as a financing activity on the statement of cash flows. Under current GAAP, it is not specified how these cash flows should be classified. In addition, companies will now have to elect whether to account for forfeitures on share-based payments by (1) recognizing forfeitures of awards as they occur or (2) estimating the number of awards expected to be forfeited and adjusting the estimate when it is likely to change, as is currently required. The Company adopted ASU 2016-09 on January 1, 2017 and elected to account for forfeited awards as they occur. The adoption did not have a material impact on the Company's consolidated financial statements and related disclosures.

In January 2017, the FASB issued ASU 2017-04, *Intangibles — Goodwill and Other (Topic 350)*: Simplifying the Accounting for Goodwill Impairment. ASU 2017-04 removes Step 2 of the goodwill impairment test, which requires a hypothetical purchase price allocation. A goodwill impairment will now be the amount by which a reporting unit's carrying value exceeds its fair value, not to exceed the carrying amount of goodwill. This standard, which will be effective for the Company beginning in the first quarter of fiscal year 2021, is required to be applied prospectively. Early adoption is permitted for interim or annual goodwill impairment tests performed on testing dates after January 1, 2017. The Company adopted the new standard on October 1, 2017. The adoption of ASU 2017-04 did not have a material impact on its consolidated financial statements and related disclosures.

Accounting Pronouncements to Be Adopted

In May 2014, the FASB issued ASU 2014-09, *Revenue from Contracts with Customers (Topic 606),* as modified by ASU 2015-14, *Revenue from Contracts with Customers (Topic 606): Deferral of the Effective Date*, ASU 2016-08, *Revenue from Contracts with Customers (Topic 606): Principal versus Agent Considerations (Reporting Revenue Gross versus Net),* ASU 2016-10, *Revenue from Contracts with Customers (Topic 606): Identifying Performance Obligations and Licensing,* and ASU 2016-12, *Revenue from Contracts with Customers (Topic 606): Narrow-Scope Improvements and Practical Expedients.* The revenue recognition principle in ASU 2014-09 is that an entity should recognize revenue to depict the transfer of goods or services to customers in an amount that reflects the consideration to which the entity expects to be entitled in exchange for those goods or services. In addition, new and enhanced disclosures will be required. Companies may adopt the new standard either using the full retrospective approach, a modified retrospective approach with practical expedients, or a cumulative effect upon adoption approach. The Company will adopt the new standard on January 1, 2018, using the modified retrospective approach. The adoption of ASU 2014-09 will not have a material impact on the Company's consolidated financial statements and related disclosures.

In January 2016, the FASB issued ASU 2016-01, *Recognition and Measurement of Financial Assets and Financial Liabilities*. ASU 2016-01 requires equity investments to be measured at fair value with changes in

fair value recognized in net income; simplifies the impairment assessment of equity investments without readily determinable fair values by requiring a qualitative assessment to identify impairment; eliminates the requirement for public business entities to disclose the method(s) and significant assumptions used to estimate the fair value that is required to be disclosed for financial instruments measured at amortized cost on the balance sheet; requires public business entities to use the exit price notion when measuring the fair value of financial instruments for disclosure purposes; requires an entity to present separately in other comprehensive income the portion of the total change in the fair value of a liability resulting from a change in the instrument-specific credit risk when the entity has elected to measure the liability at fair value in accordance with the fair value option for financial instruments; requires separate presentation of financial assets and financial liabilities by measurement category and form of financial assets on the balance sheet or the accompanying notes to the financial statements and clarifies that an entity should evaluate the need for a valuation allowance on a deferred tax asset related to available-for-sale securities in combination with the entity's other deferred tax assets. ASU 2016-01 is effective for financial statements issued for fiscal years beginning after December 15, 2017, and interim periods within those fiscal years. The Company will adopt the new standard on January 1, 2018. The adoption of ASU 2016-01 will not have a material impact on its consolidated financial statements and related disclosures.

In August 2016, the FASB issued ASU 2016-15, *Statement of Cash Flows — Classification of Certain Cash Receipts and Cash Payments,* which addresses eight specific cash flow issues with the objective of reducing the existing diversity in practice in how certain cash receipts and cash payments are presented and classified in the statement of cash flows. The standard is effective for fiscal years beginning after December 15, 2017, including interim periods within those fiscal years. Early adoption is permitted, including adoption in an interim period. The Company will adopt the new standard on January 1, 2018. The adoption of ASU 2016-15 will not have an impact on its consolidated statements of cash flows and related disclosures.

In May 2017, the FASB issued ASU 2017-09, *Compensation-Stock Compensation (Topic 718): Scope of Modification Accounting*. ASU 2017-09 provides clarity and reduces both (1) diversity in practice and (2) cost and complexity when applying the guidance in Topic 718, to a change to the terms or conditions of a share-based payment award. The amendments in ASU 2017-09 should be applied prospectively to an award modified on or after the adoption date. This ASU is effective for fiscal years beginning after December 15, 2017, including interim periods within those years. The Company will adopt the new standard on January 1, 2018. The adoption of ASU 2017-09 will not have an impact on its consolidated financial statements and related disclosures.

In February 2016, the FASB issued ASU 2016-02, *Leases (Topic 842)* which supersedes FASB Topic 840, *Leases (Topic 840)* and provides principles for the recognition, measurement, presentation and disclosure of leases for both lessees and lessors. The new standard requires lessees to apply a dual approach, classifying leases as either finance or operating leases based on the principle of whether or not the lease is effectively a financed purchase by the lessee. This classification will determine whether lease expense is recognized based on an effective interest method or on a straight-line basis over the term of the lease, respectively. A lessee is also required to record a right-of-use asset and a lease liability for all leases with a term of greater than twelve months regardless of classification. Leases with a term of twelve months or less will be accounted for similar to existing guidance for operating leases. In January 2018, the FASB issued ASU 2018-01, *Leases (Topic 842) Land Easement Practical Expedient for Transition to Topic 842*, which amends ASU 2016-02 to provide entities an optional transition practical expedient to not evaluate under Topic 842 existing or expired land easements that were not previously accounted for as leases under the current leases guidance in Topic 842. An entity that elects this practical expedient should evaluate new or modified land easements under Topic 842 beginning at the date that the entity adopts Topic 842. The standard will be effective for annual and interim periods beginning after December 15, 2018, with early adoption permitted upon issuance. The Company is currently evaluating the impact on its consolidated financial statements and related disclosures.

In June 2016, the FASB issued ASU 2016-13, *Financial Instruments — Credit Losses: Measurement of Credit Losses on Financial Instruments*. ASU 2016-13 requires that expected credit losses relating to financial assets measured on an amortized cost basis and available-for-sale debt securities be recorded through an allowance for credit losses. ASU 2016-13 limits the amount of credit losses to be recognized for

available-for-sale debt securities to the amount by which carrying value exceeds fair value and also requires the reversal of previously recognized credit losses if fair value increases. The new standard will be effective on January 1, 2020. Early adoption will be available on January 1, 2019. The Company is currently evaluating the effect that the updated standard will have on its consolidated financial statements and related disclosures.

In February 2018, the FASB issued ASU 2018-02, *Income Statement — Reporting Comprehensive Income*, *(Topic 220): Reclassification of Certain Tax Effects from Accumulated Other Comprehensive Income,* which allows a reclassification from accumulated other comprehensive income to retained earnings for stranded tax effects resulting from the newly enacted federal corporate income tax rate under the Tax Cuts and Jobs Act. The amount of the reclassification would be the difference between the historical corporate income tax rate and the newly enacted 21% corporate income tax rate. The new standard is effective for fiscal years, including interim periods within those fiscal years, beginning after December 15, 2018 with early adoption in any interim period permitted. The Company is currently evaluating the effect that the updated standard will have on its consolidated financial statements and related disclosures.

Note 3 — Marketable Securities

Marketable securities consisted of the following as of December 31, 2017 and 2016:

	December 31, 2017			
	Amortized Cost	Gross Unrealized Gain[(1)]	Gross Unrealized Loss[(1)]	Fair Value
Short-term available-for-sale securities				
Corporate bonds	$38,092,585	$5,635	$(183,738)	$37,914,482
Long-term available-for-sale securities				
Corporate bonds	3,357,778	—	(10,565)	3,347,213
Total	$41,450,363	$5,635	$(194,303)	$41,261,695

	December 31, 2016			
	Amortized Cost	Gross Unrealized Gain[(1)]	Gross Unrealized Loss[(1)]	Fair Value
Short-term available-for-sale securities				
Corporate bonds	$22,032,191	$3,190	$(473,056)	$21,562,325
Government and agency obligations	1,225,000	661	—	1,225,661
Municipal bonds	1,596,160	4,257	—	1,600,417
	24,853,351	8,108	(473,056)	24,388,403
Long-term available-for-sale securities				
Corporate bonds	2,434,251	1,502	—	2,435,753
	2,434,251	1,502	—	2,435,753
Total	$27,287,602	$9,610	$(473,056)	$26,824,156

(1) Gross unrealized gain (loss) is pre-tax.

The contractual term to maturity of short-term marketable securities held by the Company as of December 31, 2017 is less than one year. The contractual term to maturity of long-term marketable securities held by the Company as of December 31, 2017 is from 1.1 to 1.2 years.

The proceeds from sales of available-for-sale securities were $33.5 million, $44.3 million and $5.0 million as of December 31, 2017, 2016 and 2015, respectively. Gross realized loss that has been included in net loss as a result of those sales were approximately $0.6 million, $1.1 million and $27,000 for the years ended December 31, 2017, 2016 and 2015, respectively.

The fair value of marketable securities was classified into fair value measurement categories as follows:

	As of December 31,	
	2017	2016
Quoted prices in active markets for identical assets (Level 1)	$ —	$ —
Quoted prices for similar assets observable in the marketplace (Level 2)	41,261,695	26,824,156
Significant unobservable inputs (Level 3)	—	—
Total	$41,261,695	$26,824,156

The fair values of marketable securities are determined using quoted market prices from daily exchange traded markets based on the closing price as of December 31, 2017 and 2016, and are classified as Level 2.

There were no transfers between levels 1, 2 or 3 for the years ended December 31, 2017 and 2016.

The following table shows the Company's investments' gross unrealized losses (pre-tax) and fair value, aggregated by investment category and length of time that individual securities have been in a continuous unrealized loss position, at December 31, 2017.

	Less than 12 Months		12 Months or More		Total	
	Fair Value	Unrealized Losses	Fair Value	Unrealized Losses	Fair Value	Unrealized Losses
Corporate bonds	$36,825,138	$(194,303)	$—	$—	$36,825,138	$(194,303)
Total	**$36,825,138**	**$(194,303)**	**$—**	**$—**	**$36,825,138**	**$(194,303)**

The Company has determined that the unrealized losses are deemed to be temporary impairments as of December 31, 2017. The Company believes that the unrealized losses generally are caused by increases in the risk premiums required by market participants rather than an adverse change in cash flows or a fundamental weakness in the credit quality of the issuer or underlying assets. Because the Company has the ability and intent to hold these investments until a recovery of fair value, which may be maturity, it does not consider the investment in corporate bonds to be other-than-temporarily impaired at December 31, 2017.

Note 4 — Goodwill and Intangible Assets

Goodwill

In July 2014, the Company completed its merger with Assembly Pharmaceuticals. The fair value of consideration paid, common stock and assumed options, totaled $29,823,096, which, net of amounts allocated to assets and liabilities acquired at fair value, resulted in an allocation to goodwill (as adjusted) of $12,638,136. The Company only has one reporting unit.

Goodwill is recorded as an indefinite-lived asset and is not amortized for financial reporting purposes but is tested for impairment on an annual basis or more frequently when indications of impairment exist. No goodwill impairment losses have been recognized. Goodwill is not deductible for income tax purposes since the tax basis is $0. The Company performed impairment tests of the carrying value of the Company's goodwill at October 1, 2017 and 2016, and deemed there was no goodwill impairment.

Intangible Assets

In July 2014, the Company completed its acquisition of Assembly Pharmaceuticals. The Company acquired in-process research and development related to Assembly Pharmaceuticals' technology which is an indefinite lived intangible asset. The Company performed its annual impairment test at October 1, 2017 and 2016, and deemed there was no impairment of long-lived assets.

Note 5 — Property, Plant and Equipment, Net

Property, plant and equipment, consists of the following:

	Useful life (Years)	As of December 31, 2017	As of December 31, 2016
Computer hardware and software	3	$ 104,968	$ 86,228
Lab equipment	3 to 5	369,827	253,735
Office equipment	7	25,354	1,109
Leasehold improvement	1 to 3.25	773,700	68,213
Total property, plant and equipment		1,273,849	409,285
Less: Accumulated depreciation and amortization		(413,823)	(194,598)
Property, plant and equipment, net		$ 860,026	$ 214,687

Depreciation expense for the years ended December 31, 2017, 2016 and 2015 was approximately $219,000, $80,000 and $65,000, respectively, and was recorded in both research and development expense and general and administrative expense in the consolidated statements of operations and comprehensive loss.

Note 6 — Accrued Expenses

Accrued expenses consist of the following:

	As of December 31, 2017	As of December 31, 2016
Accrued expenses:		
Salaries, bonuses and employee benefits	$4,518,128	$2,884,000
Accrued severance expenses	—	241,737
Research and development expenses	674,686	916,674
General and administrative expenses	946,186	710,412
Total accrued expenses	$6,139,000	$4,752,823

Note 7 — Stockholders' Equity

Common and Preferred Stock Transactions

2015 Activity

On March 19, 2015, the Company sold to various investors an aggregate of 5,555,555 shares of common stock in a public offering. The purchase price paid by the investors was $13.50 per share and an aggregate of $70.4 million in net proceeds were received, after deducting underwriting discounts and commissions and estimated offering expenses. In addition, the Company granted the underwriters a 30-day option to purchase up to an additional 833,333 shares of common stock.

On April 6, 2015, the underwriters exercised in full their option to purchase an additional 833,333 shares of common stock at the public offering price of $13.50 per share, less underwriting discounts and commissions and offering expenses. The closing of the option exercise resulted in net proceeds of approximately $10.6 million. Exercise of the underwriters' option increased the net proceeds (net of underwriting discounts and commissions) of the public offering, from $70.4 million to $81.0 million.

On December 30, 2015, the Company filed a registration statement on Form S-3 with the SEC using a "shelf" registration process, file number 333-208806, which became effective January 19, 2016. Under this shelf registration process, the Company may from time to time sell any combination of the securities described in the registration statement in one or more offerings for an aggregate offering price of up to

$150,000,000. On November 6, 2017, the Company closed an offering of an aggregate offering price of approximately $69.3 million of common stock. As a result, securities with an aggregate offering price of approximately $80.7 million remain available under this registration statement.

2016 Activity

On June 29, 2016, the Company cancelled 108 shares of common stock, which represented the aggregate number of fractional shares that were cashed out as a result of the reverse stock split effected in July 2014.

2017 Activity

For the year ended December 31, 2017, the Company issued an aggregate of 349,720 shares of common stock and received gross proceeds of approximately $2.4 million from the exercise of options.

On November 1, 2017, the Company sold to various investors an aggregate of 2,210,000 shares of common stock in a public offering. The purchase price paid by investors was $27.25 per share and an aggregate of $64.8 million (net of underwriting discounts and commissions and offering expenses) was received, which includes proceeds received pursuant to the underwriters' exercise of their 30-day option to purchase of up to an additional 331,500 shares in full, which occurred on November 2, 2017.

On December 29, 2017, the Company filed a registration statement on Form S-3 with the SEC using a "shelf" registration process, file number 333-222366, which became effective January 10, 2018. Under this shelf registration process, the Company may from time to time sell any combination of the securities described in the registration statement in one or more offerings for an aggregate offering price of up to $250,000,000. In connection with the filing of this registration statement, the Company entered into a sales agreement under which the Company may offer and sell shares of its common stock having an aggregate offering price of up to $75.0 million under this registration statement through "at the market offerings."

Options, Warrants and Restricted Stock Units

Options

In July 2010, the stockholders approved the 2010 Equity Incentive Plan (the 2010 Plan) and on May 19, 2011, the stockholders approved an amendment to the 2010 Plan increasing the authorized shares thereunder to 793,440, on a post-Split Effective Time basis. As of December 31, 2017, there were outstanding options to purchase an aggregate of 595,334 shares of common stock. Effective on June 2, 2016, the 2010 Plan was frozen and no further grants will be made under the 2010 Plan. Shares that are forfeited under the 2010 Plan on or after June 2, 2016 will become available for issuance under the Amended and Restated 2014 Plan (as defined below).

In July 2014, the stockholders approved the 2014 Stock Incentive Plan (the 2014 Plan). On June 2, 2016, at the 2016 Annual Meeting of Stockholders, the stockholders of the Company approved the amendment and restatement of the Company's 2014 Plan (the Amended and Restated 2014 Plan). Pursuant to the terms of the Amended and Restated 2014 Plan, the maximum number of shares reserved for issuance thereunder is 4,160,000. As of December 31, 2017, there were outstanding options to purchase an aggregate of 3,216,246 shares of common stock and outstanding restricted stock units to purchase 120,000 shares of common stock (as below), and 587,391 shares available for grant under the Amended and Restated 2014 Plan, which includes 73,876 shares of common stock forfeited under the 2010 Plan.

On April 3, 2017, the Company's Board of Directors adopted the Assembly Biosciences, Inc. 2017 Inducement Award Plan (the Inducement Plan) pursuant to which the Company reserved 800,000 shares of common stock for issuance under the Inducement Plan. The only persons eligible to receive grants of awards under the Inducement Plan are individuals who satisfy the standards for inducement grants under Nasdaq Marketplace Rule 5635(c)(4) and the related guidance under Nasdaq IM-5635-1. An "Award" is any right to receive Assembly common stock pursuant to the Inducement Plan, consisting of nonstatutory stock options, stock appreciation rights, dividend equivalent rights, restricted stock awards, restricted stock unit awards, or any other stock award. As of December 31, 2017, there were outstanding options to purchase an aggregate of 189,000 shares of common stock and 611,000 shares available for grant under the Inducement Plan.

A summary of the Company's option activity and related information for the years ended December 31, 2017, 2016 and 2015 is as follows:

	Number of Shares	Weighted Average Exercise Price	Total Intrinsic Value
Outstanding as of December 31, 2015	3,367,784	$ 7.16	$ 3,971,205
Granted	1,196,500	$ 7.46	$ 5,676,405
Exercised	(21,200)	$ 7.20	—
Forfeited	(85,833)	$12.18	—
Outstanding as of December 31, 2016	4,457,251	$ 7.14	$ 23,258,604
Granted	706,800	$24.16	$ 15,792,854
Exercised	(353,612)	$ 7.03	—
Forfeited	(258,620)	$ 9.51	—
Outstanding as of December 31, 2017	4,551,819	$ 9.66	$162,002,439
Options vested and exercisable	3,128,885	$ 6.80	$120,316,959

The Company expects that all outstanding unvested options will vest. The fair value of the options granted for the year ended December 31, 2017, 2016 and 2015, was based on the following assumptions:

	Year Ended December 31,		
	2017	2016	2015
Exercise price	$12.81 – $44.28	$5.84 – $13.21	$7.88 – $16.55
Expected stock price volatility	81.2% – 87.0%	85.8% – 91.8%	88.62% – 95.55%
Risk-free rate of interest	2.02% – 2.29%	1.36% – 2.18%	1.49% – 2.27%
Term (years)	5.5 – 7.0	5.3 – 7.0	5.0 – 8.2

Estimated future stock-based compensation expense relating to unvested stock options is as follows:

	Future Stock Option Compensation Expenses
Year Ended December 31, 2018	$5,755,478
Year Ended December 31, 2019	1,760,834
Year Ended December 31, 2020	634,516
Year Ended December 31, 2021	54,968
Total	$8,205,796

The weighted average remaining amortization period is approximately 1.2 years at December 31, 2017. The weighted average remaining contractual term of exercisable options is approximately 6.8 years at December 31, 2017.

Warrants

On April 17, 2015, the Company issued an aggregate of 88,293 shares of common stock from the cashless exercise of 120,265 warrants. The Company did not receive any proceeds from this cashless exercise.

During the year ended December 31, 2015, 133,587 warrants to purchase common stock expired unexercised.

There was no warrant activity for the years ended December 31, 2016.

During the year ended December 31, 2017, 1,613 warrants to purchase common stock expired unexercised.

A summary of the Company's warrant activity and related information is as follows:

	Warrants	Weighted Average Exercise Price
Outstanding as of December 31, 2015	16,909	$30.81
Outstanding as of December 31, 2016	16,909	$30.81
Expired	(1,613)	—
Outstanding as of December 31, 2017	15,296	$30.00

The weighted average remaining contractual life of outstanding warrants at December 31, 2017 is approximately 2.7 years.

Restricted Stock Units

On December 8, 2017, the Company issued 120,000 restricted stock units to its Chief Scientific Officer, Richard Colonno. The RSUs will vest upon the occurrence of the performance milestones.

A summary of the Company's restricted stock units and related information is as follows:

	Number of units	Weighted average grant price
Unvested as of December 31, 2016	—	$ —
Granted	120,000	44.28
Unvested as of December 31, 2017	120,000	$44.28

As of December 31, 2017, the Company had unrecognized stock-based compensation expense related to all unvested restricted stock units of $3.0 million, which is expected to be recognized over the remaining weighted-average vesting period of 0.6 years.

Stock-based compensation expenses for the years ended December 31, 2017, 2016 and 2015 were as follows:

	Year Ended December 31,		
	2017	2016	2015
Research and development	$5,422,731	$3,025,178	$3,257,732
General and administrative	3,178,711	1,999,334	4,618,852
Total stock-based compensation expense	$8,601,442	$5,024,512	$7,876,584

Note 8 — License Agreements

Allergan

On January 6, 2017, the Company entered into the Collaboration Agreement with Allergan to develop and commercialize select microbiome gastrointestinal programs. Pursuant to the Collaboration Agreement, the Company granted Allergan an exclusive worldwide license to certain of its intellectual property, including its intellectual property arising under the Collaboration Agreement, to develop and commercialize licensed compounds for ulcerative colitis (UC), Crohn's disease, and irritable bowel syndrome (IBS).

Under the Collaboration Agreement, Allergan and the Company will collaborate on research and development activities with respect to the licensed compounds in accordance with a mutually agreed upon research and development plan.

Pursuant to the terms of the Collaboration Agreement, in connection with the closing of the transaction on February 10, 2017, Allergan paid the Company an upfront payment of $50 million. Additionally, the Company is eligible to receive up to approximately $630 million in payments related to seven development milestones and up to approximately $2.15 billion in payments related to 12 commercial development and sales milestones in connection with the successful development and commercialization of licensed compounds for up to six different indications. At the time of execution of the Collaboration

Agreement, there was significant uncertainty as to whether the stated milestones would be achieved. In conjunction with this uncertainty, the Company has determined that the milestones are substantive in nature as they are commensurate with the enhancement of value of the delivered license as they relate to clinical success and advancement within the FDA product development platform. In addition, the Company is eligible to receive tiered royalties at rates ranging from the mid-single digits to the mid-teens based on net sales. Allergan and the Company have agreed to share development costs up to an aggregate of $75 million through proof-of-concept (POC) studies on a ⅔, ⅓ basis, respectively, and Allergan has agreed to assume all post-POC development costs. In the event any pre-POC development costs exceed $75 million in the aggregate, the Company may elect either (a) to fund ⅓ of such costs in excess of $75 million or (b) to allow Allergan to deduct from future development milestone payments ⅓ of the development costs funded by Allergan in excess of $75 million plus a premium of 25%. The Company has an option to co-promote the licensed programs in the U.S. and China, subject to certain conditions set forth in the Collaboration Agreement. In 2017, total POC development costs were approximately $7.2 million. The Company shared approximately $2.4 million of POC development costs and recorded it in research and development expense in the consolidated statements of operations and comprehensive loss for the year ended December 31, 2017.

Allergan may terminate the Collaboration Agreement for convenience at any time upon either 90 days' (prior to the initiation of the first POC trial of a licensed product) or 120 days' (after the initiation of the first POC trial of a licensed product), as applicable, advance written notice to the Company. The Collaboration Agreement also contains customary provisions for termination by either party, including in the event of breach of the Collaboration Agreement, subject to cure.

The Collaboration Agreement meets the definition of a collaborative arrangement and a multiple-element arrangement. The Company concluded that there were two significant deliverables under the Collaboration Agreement for each of four indicators — the licenses and the research and development services — but that the license does not have stand-alone value as Allergan cannot obtain value from the license without the research and development services, which the Company is uniquely able to perform. The deferred revenue will be amortized over a 10-year service period. As such, the Company recognized the upfront payment received of $50.0 million as approximately $5.0 million in short-term deferred revenue and $45.0 million in long-term deferred revenue as of the closing date. Given the early stage of development, the Company has determined the relative selling price for each of the four indicators to be $12.5 million and expects the elements to deliver over similar times. For the year ended December 31, 2017, the Company recorded approximately $4.4 million in revenue related to the amortization of deferred revenue. Expense reimbursements will be recognized as collaboration revenue when the related expenses are incurred. The reimbursable expenses incurred in connection with the Collaboration Agreement during the year ended December 31, 2017 were approximately $4.6 million and recorded in collaboration revenue in the consolidated statement of operations and comprehensive loss. In the consolidated balance sheets, $2.3 million is recorded as accounts receivable from collaboration as of December 31, 2017.

Note 9 — Milestones and Research Agreements

HBV Research Agreement with Indiana University

The Company, through its wholly-owned subsidiary, Assembly Pharmaceuticals, is party to a license agreement with Indiana University Research and Technology Corporation (IURTC) from whom it has licensed aspects of the Company's HBV program held by IURTC. The license agreement requires the Company to make milestone payments based upon the successful accomplishment of clinical and regulatory milestones. The aggregate amount of all performance milestone payments under the IURTC license agreement, should all milestones through development be met, is $825,000. As of December 31, 2017, no performance milestone payments have been made. The Company also is obligated to pay IURTC royalty payments based on net sales of the licensed technology. The Company is also obligated to pay diligence maintenance fees (starting at $25,000 in 2014 and rising to $100,000 in the year following first commercial sale of licensed product) each year to the extent that the royalty, sublicensing, and milestone payments to IURTC are less than the diligence maintenance fee for that year.

Microbiome Targeted Colonic Delivery Platform

On November 8, 2013, the Company entered into a License and Collaboration Agreement with Therabiome, LLC (Therabiome), for all intellectual property and know-how owned or controlled by Therabiome relating to the oral delivery of pharmaceutical drugs to specific sites in the intestine, using a pH sensitive controlled release capsule-in-capsule technology. Under the agreement, Therabiome granted to the Company the exclusive worldwide license, with rights to sublicense, to develop the intellectual property for commercialization (a) in the use of bacteria, viruses, proteins and small molecules by oral delivery in (i) gastrointestinal dysbiosis, including but not limited to C. *difficile*, irritable bowel syndrome-constipation and inflammatory bowel disease, (ii) auto-immune disorders and autism, including but not limited to as controlled by bacteria or virus, and (iii) orally delivered vaccines, including viral and bacterial, and (b) any oral delivery of small molecules using the licensed intellectual property. The Company will be solely responsible for all research and development activities with respect to any product it develops under the license.

For the license, the Company paid Therabiome an upfront non-refundable license fee of $300,000. The Company must pay Therabiome clinical and regulatory milestones for each product or therapy advanced from the platform for U.S. regulatory milestones. The Company also must pay Therabiome lesser amounts for foreign regulatory milestones, which vary by country and region. The Company also must pay Therabiome royalties on annual net sales of a product in the low to mid-single digit percentages plus, once annual net sales exceed two certain thresholds, a one-time cash payment upon reaching each threshold.

Therabiome must pay the Company royalties on annual net sales of any product it develops, using the intellectual property, in the low double to mid-double digit percentages, depending on the level of development or involvement the Company had in the product.

Note 10 — Income Taxes

There was no current income tax provision for the years ended December 31, 2017, 2016 and 2015. There was a deferred income tax benefit of $9,050,249 and $617,672 for the years ended December 31, 2017 and 2016, respectively. There was no deferred income tax benefit for the year ended December 31, 2015.

The Company's deferred tax assets as of December 31, 2017 and 2016 consist of the following:

	As of December 31,	
	2017	2016
Deferred tax assets:		
Net-operating loss carryforward	$ 43,577,000	$ 59,926,000
Stock-based compensation	5,726,000	9,279,000
In-Process R&D	2,259,000	4,050,000
Deferred revenue	11,064,000	—
R&D credit	5,604,000	3,962,000
Change in unrealized loss on marketable securities	46,000	178,000
Other	314,000	316,000
Total Deferred Tax Assets	68,590,000	77,711,000
Valuation allowance	(63,718,000)	(77,711,000)
Deferred Tax Asset, Net of Allowance	$ 4,872,000	$ —
In-process research and development (Assembly Merger)	7,007,802	11,119,651
Net Deferred Tax Liability	$ 2,135,802	$ 11,119,651

On December 22, 2017, the Tax Act, was signed into law. Among other items, the Tax Act reduces the federal corporate tax rate to 21% from the existing applicable rate of 34%, effective January 1, 2018. As a result, the Company has recorded a decrease to its deferred tax assets of $24.7 million and to valuation allowance of $28.4 million, resulting in a net tax benefit of $3.7 million.

The Tax Act also permits an indefinite carry forward of net operating losses generated in taxable years ending after December 31, 2017, subject to a utilization limitation of 80% of taxable income. Due to the change in the carryforward period for post-2017 net operating losses, the Company has determined that it would be able to use the deferred tax liability associated with certain in-process research and development as a source of income in determining the realizability of its deferred tax assets. As a result, the Company recorded a $4.9 million income tax benefit from the reduction of its valuation allowance.

The Company's income tax benefit for the year ended December 31, 2017 of $9.1 million includes a tax benefit of $8.6 million related to the Tax Act.

The Company maintains a valuation allowance on deferred tax assets due to the uncertainty regarding the ability to utilize these deferred tax assets in the future. The deferred tax liability was recorded in connection with the acquisition of Assembly Pharmaceuticals in 2014 and relates to the difference between the carrying amount of in-process research and development for financial statement purposes relative to the amount used for income tax purposes.

At December 31, 2017, the Company had potentially utilizable gross Federal net operating loss carryforwards of approximately $153.2 million, State net operating loss carry-forwards of approximately $165.0 million and research and development credit carry forward of approximately $5.6 million, all of which expire between 2027 and 2037.

Sections 382 and 383 of the Internal Revenue Code of 1986, as amended (IRC), subject the future utilization of net operating losses and certain other tax attributes to an annual limitation in the event of certain ownership changes, as defined. The Company has performed an ownership change study through December 31, 2016 and has determined that a "change in ownership" as defined by IRC Section 382 and the rules and regulations promulgated thereunder, did occur in December 2010, January 2013 and October 2014. The Company is in the process of performing an ownership change study through December 31, 2017.

The following is a reconciliation of the U.S. federal statutory rate to the effective income tax rates for the year ended December 31, 2017, 2016 and 2015:

	As of December 31,		
	2017	2016	2015
Statutory Federal Income Tax Rate	(34.0)%	(34.0)%	(34.0)%
State Taxes, Net of Federal Tax Benefit	(2.6)%	(4.3)%	(11.0)%
Merger Cost	—%	—%	0.9%
Stock based Compensation	—%	3.4%	16.7%
Credits	(2.1)%	(1.8)%	—%
Federal Rate Change	47.7%	—%	—%
State Rate Change	0.2%	7.5%	—%
Other	0.3%	2.3%	0.1%
Change in Valuation Allowance	(27.0)%	25.5%	27.3%
Income Taxes Provision (Benefit)	(17.5)%	(1.4)%	—%

Note 11 — Commitments and Contingencies

Real Property Leases and Equipment Leases

The Company leases office space for corporate functions in Carmel, Indiana under a lease agreement that expires in August 2023. The leased location in Carmel, Indiana supports both the HBV-cure and Microbiome programs. The Company leases office and laboratory space in San Francisco, California under a sublease that expires on December 31, 2018 unless the Company requests a six-month extension. The Company also conducted research activities for the HBV-cure program at laboratory space leased from Indiana University at Bloomington, Indiana until May 2017. The Company transferred the activities that it performed at Indiana University to its Carmel, Indiana and San Francisco, California locations. The Company also conducts research activities for the Microbiome program at office and laboratory space in Groton, Connecticut under a lease that expires in March 2019. The Company ceased leasing office and laboratory space from the University of Florida Research Foundation in Alachua, Florida in May 2017.

The total real estate leasing expenses for the year ended December 31, 2017, 2016 and 2015 were approximately $1.3 million, $1.2 million and $0.7 million, respectively.

Pursuant to a Master Lease agreement dated November 25, 2014, the Company is leasing certain laboratory equipment. The equipment lease expense for the year ended December 31, 2017, 2016 and 2015 amounted to approximately $758,000, $705,000 and $107,000, respectively. These equipment leases begin to expire in 2017, with the final lease expiring in 2021. The sum of all future payments through termination is approximately $2.9 million.

Future minimum rental payments for real estate and equipment required as of December 31, 2017 are as follows:

Year Ended December 31, 2018	$2,974,537
Year Ended December 31, 2019	1,193,126
Year Ended December 31, 2020	827,511
Year Ended December 31, 2021	254,136
Year Ended December 31, 2022	212,625
Thereafter	106,312
	$5,568,247

Employment Agreements

The Company has employment agreements with its executive officers, which agreements set forth the terms of their employment, including severance arrangements. The Chief Executive Officer and Chief Financial Officer/Chief Operating Officer were paid an aggregate annual base salary of approximately $0.9 million in 2017, $0.8 million in 2016 and $0.8 million in 2015, respectively.

Litigation

The Company is not a party to any material legal proceedings and is not aware of any claims or actions pending or threatened against it. From time to time, the Company may be subject to various legal proceedings and claims that arise in the ordinary course of its business activities.

Note 12 — Selected Quarterly Financial Data (Unaudited)

The following table contains quarterly financial information for fiscal years 2017 and 2016. The Company believes that the following information reflects all normal recurring adjustments necessary for a fair statement of the information for the periods presented.

	First Quarter	Second Quarter	Third Quarter	Fourth Quarter
2017				
Collaboration revenue	$ 684,369	$ 2,359,311	$ 2,659,613	$ 3,315,457
Operating expenses	$ 14,614,198	$ 15,926,562	$ 15,109,793	$ 15,595,075
Interest and other income	$ 136,484	$ 239,858	$ 241,326	$ 365,541
Realized loss from marketable securities	$ (137,248)	$ (340,984)	$ (99,068)	$ (37,828)
Net loss	$(13,930,593)	$(13,598,864)	$(12,272,019)	$ (3,007,072)
Basic and diluted net loss per common share	$ (0.81)	$ (0.78)	$ (0.71)	$ (0.16)
2016				
Operating expenses	$ 11,277,152	$ 10,454,130	$ 11,678,925	$ 11,868,254
Interest and other income	$ 490,421	$ 444,605	$ 378,381	$ 225,681
Realized loss from marketable securities	$ (201,827)	$ (142,675)	$ (273,573)	$ (521,786)
Net loss	$(10,988,558)	$(10,152,200)	$(11,574,117)	$(11,546,687)
Basic and diluted net loss per common share	$ (0.64)	$ (0.59)	$ (0.67)	$ (0.67)

CORPORATE INFORMATION

Directors

Anthony E. Altig
Former Chief Financial Officer, Biotix Holdings, Inc.

Mark Auerbach
Former Non-Executive Chairman of the Board and Chairman of the Audit Committee for RCS Capital Corporation; Former Lead Independent Director and Chairman of the Audit Committee of Optimer Pharmaceuticals, Inc.

Richard D. DiMarchi, Ph.D.
Cox Distinguished Professor of Biochemistry and Gill Chair in Biomolecular Sciences and Vice President of Research, Novo Nordisk Research Labs

Myron Z. Holubiak
President and Chief Executive Officer, Citius Pharmaceuticals, Inc.

Helen S. Kim
Former Executive Vice President of Business Development, Kite Pharma, Inc.

Alan J. Lewis, Ph.D.
Chief Executive Officer, DiaVacs, Inc.

Susan Mahony, Ph.D.
Senior Vice President and President of Lilly Oncology, Eli Lilly and Company

William R. Ringo, Jr.
Director of Sangamo BioSciences, Inc., Immune Design Corp., Dermira, Inc. and Five Prime Therapeutics, Inc.

Derek A. Small
President and Chief Executive Officer, Assembly Biosciences, Inc.

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Stock Listing
Assembly Biosciences, Inc. common stock is listed on the Nasdaq Capital Market and quoted under the symbol "ASMB"

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President and Chief Executive Officer

Graham Cooper
Chief Financial Officer and Chief Operating Officer

Richard J. Colonno, Ph.D.
Executive Vice President and Chief Scientific Officer of Virology Operations

Uri A. Lopatin, M.D.
Chief Medical Officer and Vice President of Research and Development

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