

CONCERT PHARMACEUTICALS, INC.  
Form 10-K  
February 28, 2019  
UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION  
Washington, D.C. 20549

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FORM 10-K

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(Mark One)

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

For the fiscal year ended: December 31, 2018

or

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

For the transition period from \_\_\_\_\_ to \_\_\_\_\_

Commission file number: 001-36310

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CONCERT PHARMACEUTICALS, INC.  
(Exact name of registrant as specified in its charter)

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Delaware 20-4839882  
(State or other jurisdiction of (I.R.S. Employer  
incorporation or organization) Identification No.)  
65 Hayden Avenue, Suite 3000N  
Lexington, Massachusetts 02421  
(Address of principal executive offices) (Zip Code)  
Registrant's telephone number, including area code: (781) 860-0045  
Securities registered pursuant to Section 12(b) of the Act:

| Title of each class                       | Name of each exchange on which registered |
|---|---|
| Common Stock, par value \$0.001 per share | The NASDAQ Global 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 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 every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). Yes  No

Indicate by check mark if disclosure of delinquent filers pursuant to Item 405 of Regulation S-K (§229.405) is not contained herein, and will not be contained, to the best of registrant's knowledge, in definitive proxy or information statements incorporated by reference in Part III of this Form 10-K or any amendment to this Form 10-K.

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, a smaller reporting company or an emerging growth company. See the definitions of "large accelerated filer", "accelerated filer", "smaller reporting company" and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer  Accelerated filer

Non-accelerated filer  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 and non-voting common equity held by non-affiliates of the registrant as of June 30, 2018 was approximately \$257,489,000 based on the closing price of the registrant's common stock on the NASDAQ Global Market on that date.

The number of shares outstanding of the registrant's Common Stock as of February 22, 2019: 23,529,253

CONCERT PHARMACEUTICALS, INC.  
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#### References to Concert

Throughout this Annual Report on Form 10-K, the “Company,” “Concert,” “we,” “us,” and “our,” except where the context requires otherwise, refer to Concert Pharmaceuticals, Inc. and its consolidated subsidiary, and “our board of directors” refers to the board of directors of Concert Pharmaceuticals, Inc.

#### Forward-Looking Information

This Annual Report on Form 10-K contains forward-looking statements regarding, among other things, our future discovery and development efforts, our future operating results and financial position, our business strategy, and other objectives for our operations. The words “anticipate,” “believe,” “estimate,” “expect,” “intend,” “may,” “plan,” “predict,” “project,” “would” and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. You also can identify forward-looking statements by the fact that they do not relate strictly to historical or current facts. There are a number of important risks and uncertainties that could cause our actual results to differ materially from those indicated by forward-looking statements. These risks and uncertainties include those inherent in pharmaceutical research and development, such as adverse results in our drug discovery and clinical development activities, decisions made by the U.S. Food and Drug Administration and other regulatory authorities with respect to the development and commercialization of our drug candidates, our ability to obtain, maintain and enforce intellectual property rights for our drug candidates, our ability to obtain any necessary financing to conduct our planned activities and other risk factors. 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 section entitled “Risk Factors” in Part I that could cause actual results or events to differ materially from the forward-looking statements that we make. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, joint ventures or investments that we may make. Unless required by law, we do not undertake any obligation to publicly update any forward-looking statements.

Part I

Item 1. Business

OVERVIEW

We are a clinical stage biopharmaceutical company applying our extensive knowledge of deuterium chemistry to discover and develop novel small molecule drugs. Selective incorporation of deuterium into known molecules has the potential, on a case-by-case basis, to provide better pharmacokinetic or metabolic properties, thereby enhancing their clinical safety, tolerability or efficacy. Our approach typically starts with previously studied compounds, including approved drugs, which we believe may be improved with deuterium substitution. Our technology provides the opportunity to develop products that may compete with the non-deuterated drug in existing markets or to leverage its known activity to expand into new indications and may enable compounds not otherwise well-suited for human drug development to be clinically developed. Our deuterated chemical entity platform, or DCE Platform®, has broad potential across numerous therapeutic areas. The Company's pipeline includes multiple clinical-stage candidates and a number of preclinical compounds that it is currently assessing.

OUR STRATEGY

Our strategy is to apply our deuterium technology to previously studied molecules, including approved drugs, in which deuterium substitution has the potential to enhance clinical safety, tolerability, or efficacy. We select pipeline candidates based on the medical needs of patients, commercial opportunity, regulatory considerations, and competitive landscape.

Key elements of our strategy include:

- using deuterium technology to develop deuterated product candidates that we believe are promising in view of the known biology of previously studied compounds, including approved drugs, in which deuterium substitution has the potential to enhance clinical safety, tolerability or efficacy;

- developing our deuterated product candidates quickly through proof-of-concept clinical trials, which could be as early as Phase 1, and then determining whether to advance it, independently or with a partner; and

- commercializing product candidates on our own, or with a strategic partner.

DEUTERIUM

Due to its natural abundance, the average adult human body contains approximately two grams of deuterium. While essentially identical to hydrogen in size and shape, deuterium differs from hydrogen in that it contains an additional neutron. As a result, deuterium forms a more stable chemical bond with carbon than does hydrogen. The deuterium-carbon bond is typically six to nine times more stable than the hydrogen-carbon bond. This has important implications for drug development because drug metabolism often involves the breaking of hydrogen-carbon bonds.

Because deuterium forms more stable bonds with carbon, deuterium substitution can in some cases alter drug metabolism, including through improved metabolic stability, reduced formation of toxins, increased formation of desired active metabolites, or a combination of these effects. At the same time, these improvements in drug metabolism are possible without materially altering the intrinsic biological activity of a compound. Deuterated compounds with enhanced metabolic properties can generally be expected to retain biochemical potency and selectivity similar to their hydrogen analogs. The effects, if any, of deuterium substitution on metabolic properties are highly dependent on the specific molecular positions at which deuterium is substituted for hydrogen. In addition, the metabolic effects of deuterium substitution, if any, are unpredictable, even in compounds that have similar chemical structures.

Potential advantages of product candidates based on our DCE Platform

Using our DCE Platform, we create novel drugs designed to have superior properties - including enhanced clinical safety, tolerability or efficacy - based on compounds that have established pharmacological activity. In many instances, Phase 1 clinical evaluation has the potential to demonstrate whether there will be product differentiation.

Potential advantages of our DCE Platform include the following:

Improved metabolic profile. An improved metabolic profile may potentially reduce or eliminate unwanted side effects or undesirable drug interactions or increase efficacy. Metabolic profile refers to the relative amounts and exposure profile of the parent drug and its metabolites or metabolic by-products in the body.

Increased half-life. A longer half-life may decrease the number of doses that a patient is required to take per day or provide more consistent exposure of the compound in comparison to the corresponding non-deuterated compound, potentially improving the drug's therapeutic profile. Half-life is usually defined as the time it takes for the body to clear half of a given concentration of the drug from the plasma.

Avoidance of undesirable metabolism: By avoiding first pass metabolism, we may be able to improve oral bioavailability, which could potentially lead to better efficacy at a lower dose of drug. First pass metabolism is metabolism that occurs before the drug reaches the circulatory system.

## OUR PRODUCT CANDIDATES

Our pipeline is focused on leveraging our deuterium expertise and proprietary product platform to develop novel medications designed to enhance patient outcomes in diverse therapeutic areas including autoimmune diseases and central nervous systems (CNS) disorders. The discussion below highlights our most advanced development programs including those being developed by our collaborators.

CTP-543

Background on Alopecia Areata

Alopecia areata is a chronic autoimmune disease affecting approximately 650,000 Americans at any given time that results in partial or complete loss of hair on the scalp and/or body. Alopecia areata occurs when the immune system attacks the hair follicles and is characterized as non-scarring hair loss. It presents in a number of patterns including:

• Patchy: coin-sized or larger patch or patches of hair loss;

• Totalis: no hair on the head; and

• Universalis: no hair anywhere on the body.

Onset can occur at any age including childhood, and it affects both women and men equally. While the average age of onset is between 25-35 years, the disease does occur in children, and onset in the first two decades is associated with more severe disease. The emotional effect of alopecia areata can be considerable and may result in anxiety and depression or affect personal attributes such as self-esteem and confidence. Alopecia areata may also be associated with other autoimmune conditions such as thyroid disease, vitiligo, allergic rhinitis, asthma, lupus, rheumatoid arthritis, and ulcerative colitis. The most common form of treatment is corticosteroids including intralesional

injections or topical application. However, they often are not an effective treatment option. There are currently no FDA-approved treatments for alopecia areata.



#### CTP-543 Opportunity

CTP-543 was discovered by applying Concert's deuterium chemistry technology to modify ruxolitinib, a Janus kinase ("JAK") inhibitor, which is commercially available under the name Jakafi® in the United States for the treatment of certain blood disorders. Ruxolitinib has been used to treat alopecia areata in academic settings, including an investigator-sponsored clinical trial, and has been shown to promote hair growth in individuals with moderate-to-severe disease.

In January 2018, we announced that the FDA had granted Fast Track designation to CTP-543 for the treatment of alopecia areata.

#### Clinical Development of CTP-543

In 2016, we completed single and multiple ascending dose Phase 1 trials with our investigational treatment CTP-543, which enrolled a total of 77 healthy volunteers. The pharmacokinetic measurements showed increased exposure with increasing doses of CTP-543. CTP-543 was well-tolerated across all dose groups and there were no serious adverse events reported in subjects who received CTP-543. In the multiple ascending dose Phase 1 trial of CTP-543, pharmacodynamic analyses were performed to assess the inhibition of IL-6- and IFN-gamma-mediated JAK/STAT signaling. Consistent with the expected pharmacological activity of a JAK1/JAK2 inhibitor, CTP-543 demonstrated a dose-related reduction of IL-6-stimulated phosphorylation of STAT3 in an ex-vivo assay. Also, IFN-gamma-mediated STAT1 signaling, which is believed to play a key role in the pathogenesis of alopecia areata, was significantly inhibited in disease-relevant immune cell types at all doses evaluated.

We also conducted a Phase 1 crossover study evaluating the metabolite profiles of CTP-543 and ruxolitinib. In this study, except for the presence of deuterium, no new metabolites were observed with CTP-543.

A Phase 2a double-blind, randomized, sequential dose-ranging trial to evaluate three sequential doses of CTP-543 (4, 8 and 12 mg twice daily) and a placebo control in patients with moderate-to-severe alopecia areata is ongoing. The primary outcome measure will utilize the severity of alopecia tool (SALT) after 24 weeks of dosing. In November 2018, we announced interim topline results from the 4 mg and 8 mg twice daily cohorts of our Phase 2a trial. At 24 weeks, patients treated with an 8 mg twice-daily dose of CTP-543 met the primary efficacy endpoint vs. placebo ( $p < 0.001$ ), patients achieving 50% relative reduction in SALT between Week-24 and baseline. Regrowth of hair did not appear to plateau at Week 24. In the primary analysis, the response observed in the 8 mg twice-daily dose was significantly different than the 4 mg twice daily dose ( $p < 0.05$ ). Compared to placebo, a significant relative reduction in mean SALT score was first observed in the 8 mg cohort at Week 12 ( $p < 0.05$ ). The average baseline SALT score across all patients enrolled in the trial was approximately 88. Treatment with CTP-543 was generally well tolerated. The most common side effects in the trial were headache, upper respiratory tract infection, cough, acne and nausea. No serious adverse events were reported. The 12 mg twice-daily cohort of CTP-543 compared to placebo is ongoing. Results from the complete Phase 2a trial, including the 12 mg cohort, are expected in the third quarter of 2019.



Primary Analysis: Responders at Week 24

Responders by Visit: Patients with  $\geq 50\%$  Change in SALT Relative to Baseline

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## CTP-692

### Background on Schizophrenia

Schizophrenia is a chronic and devastating neuropsychiatric disorder that is a leading cause of disability worldwide. The disease afflicts nearly 1% of the world's population, affecting both men and women equally, and striking all ethnic and socioeconomic groups with a similar level of prevalence. The illness is characterized by multiple symptoms that are categorized into three clusters known as positive symptoms (hallucinations and delusional behaviors), negative symptoms (anhedonia, social withdrawal and apathy), and cognitive dysfunction (diminished capacity for learning, memory, and executive function). The underlying basis of the current antipsychotic therapy is that excessive dopaminergic neurotransmission and dysfunctional D2 receptor signaling plays a key pathophysiological role in the disease, and consequently all typical and atypical antipsychotics in clinical practice possess some level of D2 antagonist activity. Currently available antipsychotic drugs exhibit efficacy for positive symptoms, but have been limited in their capacity to treat negative symptoms and cognitive deficits.

There is an extensive body of evidence supporting N-methyl-D-aspartate, or NMDA, receptor hypofunction as a key underlying mechanism of schizophrenia. The NMDA receptor comprises two binding domains and, in addition to requiring glutamate binding, activation with a co-agonist such as D-serine or glycine is necessary for NMDA receptor activation. D-Serine is believed to be the most important human NMDA synaptic co-agonist. It has been postulated for some time that administration of NMDA co-agonists could benefit patients with schizophrenia since there is evidence that plasma and cerebral spinal fluid, or CSF, levels of endogenous D-serine are reduced in patients with schizophrenia. In addition, higher activity of the D-serine-metabolizing enzyme D-amino acid oxidase has been reported in post-mortem brain tissue of patients with schizophrenia than in normal individuals.

### CTP-692 Opportunity

CTP-692 is a selective deuterium-modified analog of the endogenous amino acid, D-serine. Based on published preclinical and clinical effects of D-serine, the Company believes that CTP-692 has the potential to help restore NMDA receptor activity in key areas of the brain to improve clinical outcomes in patients with schizophrenia. Clinical studies have shown that levels of D-serine measured in the plasma and CSF of patients with schizophrenia are significantly lower than healthy controls. Academic studies have demonstrated that oral dosing of D-serine can result in dose-dependent improvement in positive, negative, and cognitive symptoms in patients with schizophrenia when added to D2-modulating antipsychotics. However, preclinical studies have demonstrated that D-serine can cause nephrotoxicity in rats. In addition, in some patients who received high doses of D-serine, clinical findings suggesting renal impairment were observed. As a result, the clinical development of D-serine has historically been limited. In preclinical studies, CTP-692 has shown clear dose separation from D-serine in causing increased levels of serum creatinine and blood urea nitrogen, suggesting that CTP-692 could have reduced toxicity and a larger therapeutic window. CTP-692 also provides greater exposure than doses of D-serine in several preclinical species. It therefore may be better-suited for development as a human therapeutic agent. CTP-692 will initially be developed as an adjunctive therapy along with standard antipsychotic medicines in patients with schizophrenia.

In December 2018, dosing in a Phase 1 clinical trial for CTP-692 was initiated. The Phase 1 program is expected to enroll approximately 80 healthy volunteers. It will include a crossover pharmacokinetic comparison of CTP-692 to D-serine and single- and multiple-ascending dose studies to assess the safety, tolerability and pharmacokinetic profile of CTP-692 in healthy volunteers. Initial Phase 1 data are expected in the first quarter of 2019.

We expect to advance CTP-692 into a Phase 2 trial in patients stable on existing antipsychotic medications in the fourth quarter of 2019.

### Preclinical Pipeline

We are currently assessing a number of preclinical assets as potential development candidates.



#### Collaboration Product Candidates

We have several collaborative arrangements with companies to develop deuterium-modified versions of their marketed products. In each of these collaborations, the deuterium-modified compound was independently discovered at Concert. Our collaborators are responsible for any future clinical development activities and disclosures associated with the following programs.

AVP-786 is a combination of deudextromethorphan and an ultra-low dose of quinidine that is being investigated for the treatment of neuro-psychiatric disorders under a development and license agreement with Avanir. In November 2015, Avanir announced the initiation of the Phase 3 clinical program to evaluate the safety and efficacy of AVP-786 for the treatment of agitation associated with dementia of the Alzheimer's type. It expects to enroll approximately 850 patients in two North American Phase 3 double blind, placebo controlled trials. These two Phase 3 trials are expected to be completed in 2019 and are expected to be part of the NDA package. Additionally, in October 2017, Avanir initiated a global Phase 3 trial which is expected to enroll approximately 400 patients to evaluate the safety and efficacy of AVP-786 for the treatment of agitation associated with dementia of the Alzheimer's type.

CTP-730 is a deuterated analog of apremilast that is being developed under a collaboration with Celgene. In January 2019, Celgene and Bristol-Myers Squibb Company announced that they have entered into a definitive merger agreement pursuant to which Bristol-Myers Squibb will acquire Celgene. Apremilast is a selective phosphodiesterase 4 (PDE4) inhibitor approved in various countries for the treatment of moderate to severe psoriasis and psoriatic arthritis. We have completed the Phase 1 clinical evaluation of CTP-730. Once daily dosing of 50 mg of CTP-730 administered for seven days in the Phase 1 clinical trial demonstrated similar steady state exposure to historical data for 30 mg of apremilast twice daily. Treatment with CTP-730 was generally well-tolerated and no serious adverse events were observed. Celgene is responsible for any development of CTP-730 beyond the completed Phase 1 clinical trials. Celgene is assessing the path forward for CTP-730. However, CTP-730 has not advanced into new trials at this time.

Jazz Pharmaceuticals is evaluating several formulation and technology options as part of its once nightly oxybate program. Jazz Pharmaceuticals initially evaluated JZP-386, a deuterium containing high sodium analog of Xyrem, which demonstrated favorable deuterium-related effects. However its current once-nightly development efforts are focused on lower sodium compounds. The collaboration with Jazz Pharmaceuticals provides for the evaluation of deuterium as an option for a once nightly sodium oxybate product (D-SXB).

#### ASSET PURCHASE AGREEMENT WITH VERTEX PHARMACEUTICALS FOR CTP-656

In July of 2017, we completed a previously announced Asset Purchase Agreement under which Vertex acquired worldwide development and commercialization rights to CTP-656 and other assets related to the treatment of cystic fibrosis (CF). CTP-656, now known as VX-561, is an investigational cystic fibrosis transmembrane conductance regulator (CFTR) potentiator that has the potential to be used as part of future once-daily combination regimens of CFTR modulators that treat the underlying cause of cystic fibrosis. We received \$160 million in cash upon closing, and we are eligible to receive up to \$90 million in additional milestones based on regulatory approval in the U.S. and agreement for reimbursement in the first of the U.K., Germany or France.

#### INTELLECTUAL PROPERTY

We protect our product candidates through the use of patents, trade secrets and careful monitoring of our proprietary know-how. Our patents and patent applications, if they issue as patents, for our lead programs expire between 2028 and 2038. The expected expiration dates are before any patent term extension to which we may be entitled under the Drug Price Competition and Patent Term Restoration Act of 1984 (commonly referred to as the Hatch-Waxman Amendments) or equivalent laws in other jurisdictions where we have issued patents.

#### CTP-543

We hold a U.S. patent covering the composition of matter of deuterated analogs of ruxolitinib and corresponding U.S. patent applications. The patent and the patent applications are expected to expire in 2033. We have corresponding patent applications in Europe and Japan, which if they issue as patents, are expected to expire in 2033. We have retained all of the CTP-543 patent rights. The Patent Trial and Appeal Board, or PTAB, instituted an inter partes review, or IPR, brought against our U.S. Patent No. 9,249,149 by Incyte Corporation (the "149 patent"). The '149 patent claims deuterated analogs of ruxolitinib including CTP-543. In October 2017, the PTAB initially denied the

petition to institute the IPR. In November 2017, Incyte filed a

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request for rehearing of the PTAB's decision. In April 2018, the PTAB granted the request and instituted the IPR. A written decision by the PTAB is expected by April 9, 2019.

#### CTP-692

We hold a U.S. patent application covering pharmaceutical compositions of CTP-692. The patent, if issued, is expected to expire in 2038. We have retained all of the CTP-692 patent rights.

#### AVP-786

We hold U.S. patents and pending applications covering the composition of matter and methods of use of deudextromethorphan and other deuterated dextromethorphan analogs, as well as a U.S. patent application covering methods of use of certain other dextromethorphan compounds. These patents and patent applications are expected to expire between 2028 and 2030. We have corresponding patents and patent applications in Europe, Japan, and other countries that are expected to expire in 2028. We have granted exclusive licenses under these patent rights to Avanir.

#### DEUTERATED SODIUM OXYBATE

We hold two U.S. patents, as well as a corresponding U.S. patent application, covering the composition of matter of deuterated analogs of sodium oxybate and methods of using them for treating certain diseases and disorders, including narcolepsy. These patents and patent applications are expected to expire in 2030. We hold a corresponding European patent that is expected to expire in 2030. We also have U.S. patents covering pharmaceutical compositions of deuterated sodium oxybate and methods of use of deuterated sodium oxybate for treating certain diseases and disorders, including narcolepsy, as well as patent applications in the United States, Europe and Japan, covering the composition of matter and methods of use of deuterated sodium oxybate, that are expected to expire in 2032. We have granted exclusive licenses under these patent rights to Jazz Pharmaceuticals.

#### CTP-730

We hold U.S. patents covering the composition of matter and methods of use of CTP-730. The patents are expected to expire in 2030. We also hold corresponding patents in Europe and Japan that are expected to expire in 2030. We have granted exclusive licenses under these patent rights to Celgene.

#### Other Product Candidates

We also have patent portfolios that are related to a number of other programs. These patent portfolios are wholly owned by us. These include issued patents or patent applications that claim deuterated analogs of more than 90 non-deuterated drugs and drug candidates.

The term of individual patents depends upon the legal term of the patents in the countries in which they are obtained. In the United States and other countries in which we file, the patent term is 20 years from the earliest date of filing a non-provisional patent application.

Under U.S. patent law, the patent term may be extended by patent term adjustment due to certain failures of the U.S. Patent and Trademark Office to act in a timely manner. The patent term of a patent that covers an FDA-approved drug may also be eligible for patent term extension, which permits patent term restoration as compensation for the patent term lost during the FDA regulatory review process. The Hatch-Waxman Amendments permit a patent term extension of up to five years beyond the expiration of the patent. The length of the patent term extension is related to the length of time the drug is under regulatory review. Patent extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval and only one patent applicable to an approved drug may be extended. Similar provisions are available in Europe and other non-U.S. jurisdictions to extend the term of a patent that covers an approved drug. In the future, if and when our pharmaceutical products receive FDA approval, we expect to apply for patent term extensions on patents that we believe are eligible for such extension. We also intend to seek patent term extensions in other jurisdictions where these are available. However, there is no guarantee that the applicable authorities, including the FDA, will agree with our assessment of whether such extensions should be granted, and even if granted, the length of such extensions.

We also rely on trade secrets and careful monitoring of our proprietary know-how to protect aspects of our business that are not amenable to, or that we do not consider appropriate for, patent protection, including our DCE Platform, such as:



our methods of evaluating candidate compounds for deuteration;

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our bioanalytical methods for identifying and measuring metabolites formed by the in vitro and in vivo metabolism of deuterated compounds;

our analytical methods for evaluating how selective deuterium substitution affects different pharmacokinetic and metabolic parameters in vitro and in vivo systems; and

our methods to determine the degree of deuterium substitution in compounds we manufacture.

#### MANUFACTURING AND SUPPLY

We currently rely, and expect to continue to rely, on third parties for the manufacture of product candidates for our clinical trials. We obtain these manufacturing services, including both the manufacture of the active pharmaceutical ingredients and finished drug product, on a purchase order basis and have not entered into long-term contracts with any of these third party manufacturers. We expect to rely on third parties for commercial manufacturing for any of our product candidates that receive marketing approval.

We believe that all of the deuterium that we use in manufacturing our product candidates is currently derived, directly or indirectly, from deuterium oxide. For most of our deuterium supply we rely on bulk supplies of deuterium oxide, which we currently source from multiple suppliers, including two located in North America, one of which is in the United States.

Certain of our manufacturing processes for our product candidates incorporate deuterium by using deuterated chemical intermediates or reagents that are derived from deuterium oxide. For the deuterated chemical intermediates and reagents, we may be subject to the license requirements applicable to deuterium oxide. In addition, the manufacturer of the deuterated chemical intermediate or reagent may themselves be required to obtain deuterium oxide under applicable licensing requirements. Most of the manufacturers of these deuterated chemical intermediates and reagents are not located in countries that produce bulk quantities of deuterium oxide. Therefore, our ability to source these deuterated chemical intermediates or reagents will depend on the ability of these manufacturers to obtain deuterium oxide from other countries.

We purchase our raw materials on a purchase order basis and have not entered into long-term contracts with any of these third party suppliers. We believe that the raw materials for our product candidates are readily available and that the cost of manufacturing for our product candidates will not preclude us from selling them profitably, if approved for sale.

#### COMMERCIALIZATION

We have not yet established a sales, marketing or product distribution infrastructure. We plan to use a combination of third party collaboration, licensing and distribution arrangements and a focused in-house commercialization capability to sell any of our products that receive marketing approval. With respect to the United States, we plan to seek to retain full commercialization rights for products that we can commercialize with a specialized sales force and to retain co-promotion or similar rights when feasible in indications requiring a larger commercial infrastructure. We plan to collaborate with third parties for commercialization in the United States of any products that require a large sales, marketing and product distribution infrastructure. We also plan to collaborate with third parties for commercialization outside the United States.

We plan to build a marketing and sales management organization to create and implement marketing strategies for any products that we market through our own sales organization and to oversee and support our sales force. We expect the responsibilities of the marketing organization would include developing educational initiatives with respect to approved products and establishing relationships with thought leaders in relevant fields of medicine.

#### COMPETITION

The development and commercialization of new drug products is highly competitive. We expect that we, and our collaborators, will face significant competition from major pharmaceutical companies, specialty pharmaceutical companies and biotechnology companies worldwide with respect to our product candidates that we, or they, may seek to develop or commercialize in the future. Specifically, there are a number of large pharmaceutical and biotechnology companies that currently market and sell products or are pursuing the development of product candidates for the

treatment of neurologic disorders, autoimmune disorders and inflammation, which are key indications for our development programs. Our competitors may succeed in developing, acquiring or licensing technologies and drug products that are more effective, simpler to use, have

fewer or more tolerable side effects or are less costly than any product candidates that we are currently developing or that we may develop or acquire, which could render our product candidates obsolete and noncompetitive.

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than any products that we, or our collaborators, may develop. Our competitors also may obtain FDA or other marketing approval for their products before we, or our collaborators, are able to obtain approval for ours, which could reduce our ability to utilize expedited regulatory pathways and could result in our competitors establishing a strong market position before we, or our collaborators, are able to enter the market.

Many of our existing and potential future competitors may have significantly greater financial resources and expertise in research and development, manufacturing, nonclinical testing, conducting clinical trials, obtaining marketing approvals and marketing approved products than we do. Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller or early stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs.

Many pharmaceutical and biotechnology companies have begun to cover deuterated analogs of their product candidates in patent applications and may develop these deuterated compounds. Some of these pharmaceutical and biotechnology companies may have significantly greater financial resources and expertise in research and development, manufacturing, nonclinical testing, conducting clinical trials, obtaining marketing approvals and marketing approved products than we do. In some cases, these competitors may be interested in developing deuterated compounds that we may be interested in developing for ourselves. Our competitors may succeed in obtaining patents that dominate our products, preventing our operational freedom. In addition, these competitors may enter into collaborative arrangements or business combinations that result in their ability to research and develop deuterated compounds more effectively than us. Our potential competitors also include academic institutions, government agencies and other public and private research organizations.

#### CTP-543

CTP-543 is a deuterated analog of ruxolitinib, which is being developed for the treatment of moderate-to-severe alopecia areata, an autoimmune disease that results in partial or complete loss of hair on the scalp and body. If CTP-543 receives marketing approval for this indication, it may face competition from a number of other product candidates that are being studied for alopecia areata. Ruxolitinib is a Janus kinase, or JAK, inhibitor. A number of companies are pursuing development of oral JAK inhibitors with a range of subtype selectivities for the treatment of alopecia areata, including Aclaris Therapeutics, Eli Lilly and Pfizer.

#### CTP-692

CTP-692 is a deuterated analog of D-serine, which is being developed for the adjunctive treatment of schizophrenia. There are a number of candidates in clinical development for adjunctive treatment of schizophrenia, exploring cognitive or negative symptoms of the disease, including SyneuRx International [Taiwan] Corp.

#### AVP-786

Avanir is developing AVP-786 for the treatment of agitation associated with dementia of the Alzheimer's type and other neuro-psychiatric disorders. There are competing marketed drugs and product candidates

in clinical development for each indication. Axsome Therapeutics, and Otsuka Pharmaceuticals and their partner Lundbeck, are also developing treatments for agitation associated with dementia of the Alzheimer's type.

#### GOVERNMENT REGULATIONS

Government authorities in the United States, at the federal, state and local level, and in other countries and jurisdictions, including the European Union, extensively regulate, among other things, the research, development, testing, manufacture, manufacturing changes, packaging, storage, recordkeeping, labeling, advertising, promotion, sales, distribution, marketing, and import and export of pharmaceutical products. The processes for obtaining regulatory approvals in the United States and in foreign countries and jurisdictions, along with subsequent compliance with applicable statutes and regulations and other regulatory authorities, require the expenditure of substantial time and financial resources.

### Review and Approval of Drugs in the United States

In the United States, the FDA regulates drugs under The Federal Food, Drug, and Cosmetic Act, or FDCA, 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 and/or sponsor to a variety of administrative or judicial sanctions, including refusal by the FDA to approve pending applications, withdrawal of an approval, imposition of a clinical hold, issuance of warning letters and other types of 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 investigations and penalties brought by the FDA and the Department of Justice or other governmental entities.

An applicant seeking approval to market and distribute a new drug product in the United States must typically undertake the following:

- completion of nonclinical laboratory tests, animal studies and formulation studies in compliance with the FDA's good laboratory practice, or GLP, regulations;

- production of well-characterized drug substance and drug product, and potentially matching placebos;

- submission to the FDA of an investigational new drug application, or IND application, which allows human clinical trials to begin unless the FDA otherwise informs the drug's sponsor within 30 days;

- agreement by clinical investigators and their clinical trial sites, followed by approval by an independent institutional review board, or IRB, representing each clinical site, before the clinical trial may be initiated at that site;

- performance of adequate and well-controlled human clinical trials in accordance with the FDA's current Good Clinical Practices, or GCPs, to establish the safety and efficacy of the proposed drug product for each indication;

- preparation and submission to the FDA of a New Drug Application, or NDA;

- review of the NDA by an FDA advisory committee, where appropriate or if applicable;

- satisfactory completion of one or more FDA inspections of the manufacturing facility or facilities at which the drug product, and the active pharmaceutical ingredient or active ingredients thereof, are produced to assess compliance with current good manufacturing practices and to assure that the facilities, methods and controls are adequate to ensure the product's identity, strength, quality and purity;

- payment of user fees and securing FDA approval of the NDA; and

- compliance with any post-approval requirements, including a risk evaluation and mitigation strategy, or REMS, and post-approval studies required by the FDA.

### Nonclinical Studies and an IND

Nonclinical studies can include in vitro and animal studies to assess the potential for efficacy and adverse events and, in some cases, to establish a rationale for human therapeutic use. The conduct of nonclinical studies is subject to federal regulations and requirements, including GLP regulations. Other studies include laboratory evaluation of the purity, stability and physical form of the manufactured drug substance or active pharmaceutical ingredient and the physical properties, stability and reproducibility of the formulated drug or drug product. An IND sponsor must submit the results of the relevant nonclinical tests, including all tests conducted under GLP conditions, together with manufacturing information, analytical data, any available clinical data or literature and plans for clinical studies,

among other things, to the FDA as part of an IND. Some nonclinical testing, such as longer-term toxicity testing, animal tests of reproductive adverse events and carcinogenicity, may continue after the IND is submitted. An IND automatically becomes effective 30 days after receipt by the FDA, unless before that time the FDA raises concerns or questions related to a proposed clinical trial and places the trial on clinical hold or partial clinical hold. In such a case, 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.

Following commencement of a clinical trial under an IND, the FDA may place a clinical hold on that trial. A clinical hold is an order issued by the FDA to the sponsor to delay a proposed clinical investigation or to suspend an ongoing investigation. A

partial clinical hold is a delay or suspension of only part of the clinical work requested under the IND. For example, a specific protocol or part of a protocol is not allowed to proceed, while other protocols may do so. No more than 30 days after imposition of a clinical hold or partial clinical hold, the FDA will provide the sponsor a written explanation of the basis for the hold. Following issuance of a clinical hold or partial clinical hold, an investigation may only resume after the FDA has notified the sponsor that the investigation may proceed. The FDA will base that determination on information provided by the sponsor correcting the deficiencies previously cited or otherwise satisfying the FDA that the investigation can proceed.

#### Human Clinical Studies in Support of an NDA

Clinical trials involve the administration of the investigational 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 representing 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 and reapprove the study at least annually. 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 NIH for public dissemination on their ClinicalTrials.gov website. Human clinical trials are typically conducted in three sequential phases, which may overlap or be combined:

The product candidate is initially introduced into healthy human subjects or patients with the target disease or  
Phase 1: condition and tested for safety, dosage tolerance, absorption, metabolism, distribution, excretion and, if possible, to gain an early indication of its effectiveness.

The product candidate is administered to a limited patient population to identify possible adverse effects and  
Phase 2: safety risks, to preliminarily evaluate the efficacy of the product for specific targeted diseases and to determine dosage tolerance and dosage for Phase 3 studies.

The product candidate is administered to an expanded patient population, generally at geographically  
Phase 3: dispersed clinical trial sites, in well-controlled clinical trials to generate enough data to statistically evaluate 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 and written IND safety reports must be submitted to the FDA and investigators for serious and unexpected suspected adverse events, or any findings from animal or in vitro testing that suggests a significant risk for human subjects. 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, or an institution it represents, if the clinical trial is not being conducted in accordance with the IRB's requirements or if the drug has been associated with unexpected serious harm to patients. Additionally, some clinical trials are overseen by an independent group of qualified experts organized by the clinical trial sponsor, known as the data monitoring committee (DMC) or board. This group provides authorization for whether or not a trial may move forward at designated check points based on review of certain data from the trial. The FDA will often inspect one or more clinical sites in late-stage clinical trials to assure compliance with GCP and the integrity of the clinical data submitted.

#### Submission of an NDA to the FDA



Assuming successful completion of required clinical testing and other requirements, the results of the nonclinical and clinical studies, 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 requesting approval to market the drug product for one or more indications. Under federal law, the submission of most NDAs is additionally subject to a number of application and user fees.

Under certain circumstances, the FDA will waive the application fee for the first human drug application that a small business, defined as a company with less than 500 employees, or its affiliate submits for review. An affiliate is defined as a business entity that has a relationship with a second business entity if one business entity controls, or has the power to control, the other business entity, or a third party controls, or has the power to control, both entities.

The FDA conducts a preliminary review of an NDA within 60 days of its receipt and informs the sponsor by the 74th day after the FDA's receipt of the submission to determine whether the application is sufficiently complete to permit substantive review. The FDA may request additional information rather than accept an NDA 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 process of NDAs. Most such applications are meant to be reviewed within ten months from the date of filing, and most applications for "priority review" products are meant to be reviewed within six months of filing. The review process may be extended by the FDA for three additional months to consider new information or clarification provided by the applicant to address an outstanding deficiency identified by the FDA following the original submission.

Before approving an NDA, 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. Additionally, before approving an NDA, the FDA will typically inspect one or more clinical sites to assure compliance with GCP.

The FDA also may require submission of a REMS plan to mitigate any identified or suspected serious risks. The REMS plan could include medication guides, physician communication plans, assessment plans, and elements to assure safe use, such as restricted distribution methods, patient registries, or other risk minimization tools.

The FDA may also refer an application for a novel drug to an advisory committee or explain why such referral was not required. Typically, an advisory committee is a panel of independent experts, including clinicians and other scientific experts, that reviews, evaluates and provides a recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions.

The FDA's Decision on an NDA

On the basis of the FDA's evaluation of the NDA and accompanying information, including the results of the 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 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, 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.

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 the drug's safety after approval, require testing and surveillance programs to monitor the product after commercialization, or impose other conditions, including distribution restrictions or other risk management mechanisms, including REMS, which can materially affect the potential market and profitability of the product. 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.

The product may also be subject to official lot release, meaning that the manufacturer is required to perform certain tests on each lot of the product before it is released for distribution. If the product is subject to official release, the manufacturer must submit samples of each lot, together with a release protocol showing a summary of the history of manufacture of the lot and the results of all of the manufacturer's tests performed on the lot, to the FDA. The FDA may in addition perform certain confirmatory tests on lots of some products before releasing the lots for distribution.

Finally, the FDA will conduct laboratory research related to the safety and effectiveness of drug products.

Expedited development and review programs

The FDA has various programs, including Fast Track Designation, Breakthrough Designation, priority review and accelerated approval, which are intended to expedite or facilitate the development and review of new drugs that meet certain criteria and/or provide for approval on the basis of surrogate endpoints.

New drugs are eligible for Fast Track designation if they are intended to treat a serious or life-threatening condition and demonstrate the potential to address an unmet medical need for the condition. Fast Track designation is intended to facilitate early and frequent meetings between the FDA and the sponsor company during development and the FDA may agree to review sections of an NDA on a rolling basis before the complete NDA is submitted. A drug may be eligible for Breakthrough Designation if the drug is intended to treat a serious or life-threatening disease and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies. Breakthrough Designation provides for frequent meetings between the sponsor and the FDA, involving senior and experience review staff, as appropriate, in a collaborative, cross-functional review and the assignment of an FDA project lead to facilitate efficient review of the development program and serve as a scientific liaison with the sponsor. Although Fast Track and Breakthrough designation do not affect the regulatory standards for approval, the frequent interactions with the FDA may facilitate a more efficient development program. In addition, the NDAs for drugs granted Fast Track and Breakthrough Designation may become eligible for priority review. Priority review is designed for drug candidates that offer significant improvements in safety or effectiveness or fill an unmet medical need and provides for an initial review within six months of acceptance of the NDA for filing, as compared to a standard review of ten months after acceptance for filing. Accelerated approval provides an earlier approval of drugs that treat serious diseases, and that fill an unmet medical need, based on a surrogate endpoint that FDA determines is reasonably likely to predict a clinical benefit. As a condition of approval, the FDA may require that the sponsor of a drug receiving accelerated approval perform post-marketing confirmatory clinical trials. Even if a drug candidate qualifies for one or more of these programs, the FDA may later decide that the drug no longer meets the conditions for qualification or that the time period for FDA review will not be shortened.

#### Section 505(b)(2) NDAs

NDAs for most new drug products are based on two adequate and well-controlled clinical trials which must contain substantial evidence of the safety and efficacy of the proposed new product. These applications are generally submitted under Section 505(b)(1) of the FDCA. The FDA is, however, authorized to approve an alternative type of NDA under Section 505(b)(2) of the FDCA. This latter type of application allows the applicant to rely, in part, on the FDA's previous findings of safety and efficacy for a similar reference product, or may rely on published literature. Specifically, Section 505(b)(2) applies to NDAs for a drug for which the applicant relies, as part of its application, on investigations made to show whether or not the drug is safe and effective for use "that were not conducted by or for the applicant and for which the applicant has not obtained a right of reference or use from the person by or for whom the investigations were conducted."

Thus, Section 505(b)(2) authorizes the FDA to approve an NDA based on safety and effectiveness data that were not developed by the applicant. NDAs filed under Section 505(b)(2) may provide an alternate and potentially more expeditious pathway to FDA approval for new or improved formulations or new uses of previously approved products. If the 505(b)(2) applicant can establish that reliance on the FDA's previous approval is scientifically appropriate, the applicant may eliminate the need to conduct certain nonclinical or clinical studies of the new product. The FDA may also require companies to perform additional studies or measurements to support the change from the approved product. The FDA may then approve the new drug candidate for all or some of the label indications for which the referenced product has been approved, as well as for any new indication sought by the Section 505(b)(2) applicant.

If our partners submit NDAs for approval of deuterated analogs of marketed compounds for which they are the NDA holder, we believe that in certain cases the FDA may allow referencing of data from the non-deuterated compound in support of the application for approval of the deuterated product. Since this referencing by our partners would involve use of their own data and not require the use of another party's data, it would constitute a Section 505(b)(1) application.

#### Post-Approval Requirements

Drugs manufactured or distributed pursuant to FDA approvals are 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. There also are continuing, annual user fee requirements for any marketed products and the establishments at which such products are manufactured, as well as new application fees for supplemental applications with clinical data.

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 the sponsor and any third-party manufacturers that the sponsor may decide to use. Accordingly, manufacturers must continue to expend time, money, and effort in the area 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 or problems with manufacturing processes of unanticipated severity or frequency, 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 REMS 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 NDAs or supplements to approved NDAs, or suspension or revocation of product license approvals;

- product seizure or detention, or refusal to permit the import or export of products;

- or

- 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 may be promoted only for the approved indications and in accordance with the provisions of the approved label. 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.

In addition, the distribution of prescription pharmaceutical products is subject to the Prescription Drug Marketing Act, or PDMA, which regulates the distribution of drugs and drug samples at the federal level, and sets minimum standards for the registration and regulation of drug distributors by the states. Both the PDMA and state laws limit the distribution of prescription pharmaceutical product samples and impose requirements to ensure accountability in distribution.

#### Abbreviated New Drug Applications for Generic Drugs

In 1984, with passage of the Hatch-Waxman Amendments to the FDCA, Congress authorized the FDA to approve generic drugs that are the same as drugs previously approved by the FDA under the NDA provisions of the statute. To obtain approval of a generic drug, an applicant must submit an abbreviated new drug application, or ANDA, to the agency. In support of such applications, a generic manufacturer may rely on the nonclinical and clinical testing previously conducted for a drug product previously approved under an NDA, known as the reference listed drug, or RLD. To reference that information, however, the ANDA applicant must demonstrate, and the FDA must conclude, that the generic drug does, in fact, perform in the same way as the RLD it purports to copy.

Specifically, in order for an ANDA to be approved, the FDA must find that the generic version is identical to the RLD with respect to the active ingredients, the route of administration, the dosage form, and the strength of the drug. At the same time, the FDA must also determine that the generic drug is “bioequivalent” to the innovator drug. Under the statute, a generic drug is bioequivalent to a RLD if “the rate and extent of absorption of the generic drug do not show a significant difference from the rate and extent of absorption of the reference listed drug. . . .”

Upon approval of an ANDA, the FDA indicates that the generic product is “therapeutically equivalent” to the RLD and it assigns a therapeutic equivalence rating to the approved generic drug in its publication “Approved Drug Products with Therapeutic Equivalence Evaluations,” also referred to as the “Orange Book.” Physicians and pharmacists consider the therapeutic equivalence rating to mean that a generic drug is fully substitutable for the RLD. In addition, by operation of certain state laws and numerous health insurance programs, the FDA’s designation of a therapeutic equivalence rating often results in substitution of the generic drug without the knowledge or consent of either the prescribing physician or patient.

Under the Hatch-Waxman Amendments, the FDA may not approve an ANDA until any applicable period of non-patent exclusivity for the RLD has expired. The FDCA provides a period of five years of data exclusivity for new drug containing a new chemical entity. For the purposes of this provision, a new chemical entity is a drug that contains no active moiety that has been previously approved by FDA in any other NDA. An active moiety is the molecule or ion responsible for the physiological or pharmacological action of the drug substance. In cases where such new chemical entity exclusivity has been granted, an ANDA may not be filed with the FDA until the expiration of five years unless the submission is accompanied by a Paragraph IV certification, in which case the applicant may submit its application four years following the original product approval.

The FDCA also provides for a period of three years of exclusivity if the NDA includes reports of one or more new clinical investigations, other than bioavailability or bioequivalence studies, that were conducted by or for the applicant and are essential to the approval of the application. This three-year exclusivity period often protects changes to a previously approved drug product, such as a new dosage form, route of administration, combination or indication. Three year exclusivity would be available for a drug product that contains a previously approved active moiety, provided the statutory requirement for a new clinical investigation is satisfied. Unlike five year new chemical entity exclusivity, an award of three year exclusivity does not block the FDA from accepting ANDAs seeking approval for generic versions of the drug as of the date of approval of the original drug product.

**Hatch-Waxman Patent Certification and the 30 Month Stay**

NDA sponsors are required to list with the FDA each patent with claims that cover the applicant's product or a method of using the product. Each of the patents listed by the NDA sponsor is published in the Orange Book. When an ANDA applicant files its application with the FDA, the applicant is required to certify to the FDA concerning any patents listed for the reference product in the Orange Book, except for patents covering methods of use for which the ANDA applicant is not seeking approval.

Specifically, the applicant must certify with respect to each patent that:

- the required patent information has not been filed;
- the listed patent has expired;
- the listed patent has not expired, but will expire on a particular date and approval is sought after patent expiration; or
- the listed patent is invalid, unenforceable or will not be infringed by the new product.

A certification that the new product will not infringe the already approved product's listed patents or that such patents are invalid or unenforceable is called a Paragraph IV certification. If the applicant does not challenge the listed patents or indicate that it is not seeking approval of a patented method of use, the ANDA application will not be approved until all the listed patents claiming the referenced product have expired.

If the ANDA applicant has provided a Paragraph IV certification to the FDA, the applicant must also send notice of the Paragraph IV certification to the NDA and patent holders once the ANDA has been accepted for filing by the FDA. The NDA and patent holders may then initiate a patent infringement lawsuit in response to the notice of the Paragraph IV certification. The filing of a patent infringement lawsuit within 45 days after the receipt of a Paragraph IV certification automatically prevents the FDA from approving the ANDA until the earlier of 30 months, expiration of the patent, settlement of the lawsuit or a decision in the infringement case that is favorable to the ANDA applicant. To the extent that the Section 505(b)(2) applicant is relying on studies conducted for an already approved product, the applicant is required to certify to the FDA concerning any patents listed for the approved product in the Orange Book to the same extent that an ANDA applicant would. As a result, approval of a 505(b)(2) NDA can be stalled until all the listed patents claiming the referenced product have expired, until any non-patent exclusivity, such as exclusivity for obtaining approval of a new chemical entity, listed in the Orange Book for the referenced product has expired, and, in the case of a Paragraph IV certification and subsequent patent infringement suit, until the earlier of 30 months, settlement of the lawsuit or a decision in the infringement case that is favorable to the Section 505(b)(2) applicant.

**Pediatric Studies and Exclusivity**

Under the Pediatric Research Equity Act of 2003, an NDA or supplement thereto must contain data that are adequate to assess the safety and effectiveness of the drug 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. With enactment of the Food and Drug Administration Safety and Innovation Act, or FDASIA, in 2012, sponsors must also submit pediatric study plans within sixty days of an end-of-phase 2 meeting, or as may be agreed between the sponsor and FDA. Those plans must contain an outline of the proposed pediatric study or studies the applicant plans to conduct, including study objectives and design, any deferral or waiver requests, and other information required by regulation. The applicant, the FDA, and the FDA's internal review committee must then review the information submitted, consult with each other, and agree upon a final plan. The FDA or the applicant may request an amendment to the plan at any time.



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 efficacy and safety has been established in adults, or full or partial waivers from the pediatric data requirements. Additional requirements and procedures relating to deferral requests and requests for extension of deferrals are contained in

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FDASIA. Unless otherwise required by regulation, the pediatric data requirements do not apply to products with orphan designation.

Pediatric exclusivity is another type of non-patent marketing exclusivity in the United States and, if granted, provides for the attachment of an additional six months of marketing protection to the term of any existing regulatory exclusivity, including the non-patent and orphan exclusivity. This six-month exclusivity may be granted if an NDA sponsor submits pediatric data that fairly respond to a written request from the FDA for such data. The data do not need to show the product to be effective in the pediatric population studied; rather, if the clinical trial is deemed to fairly respond to the FDA's request, the additional protection is granted. If reports of requested pediatric studies are submitted to and accepted by the FDA within the statutory time limits, whatever statutory or regulatory periods of exclusivity or patent protection cover the product are extended by six months. This is not a patent term extension, but it effectively extends the regulatory period during which the FDA cannot accept or approve another application.

#### Patent Term Restoration and Extension

A patent claiming a new drug product may be eligible for a limited patent term extension under the Hatch-Waxman Amendments. Those Amendments permit a patent restoration of up to five years for patent term lost during product development and the FDA regulatory review. The restoration period granted is typically one-half the time between the effective date of an IND and the submission date of a NDA, plus the time between the submission date of a NDA and ultimate approval. Patent term restoration cannot be used to extend the remaining term of a patent past a total of 14 years from the product's approval date. Only one patent applicable to an approved drug product is eligible for the extension, and the application for the extension must be submitted prior to the expiration of the patent in question. The U.S. Patent and Trademark Office reviews and approves the application for any patent term extension or restoration in consultation with the FDA.

#### Review and Approval of Drug Products in the European Union

In order to market any product outside of the United States, a company must also comply with numerous and varying regulatory requirements of other countries and jurisdictions regarding quality, safety and efficacy and governing, among other things, clinical trials, marketing authorization, commercial sales and distribution of its products. Whether or not it obtains FDA approval for a product, the company would need to obtain the necessary approvals by the comparable foreign regulatory authorities before it can commence clinical trials or marketing of the product in those countries or jurisdictions. The approval process ultimately varies between countries and jurisdictions and can involve additional product testing and additional administrative review periods. The time required to obtain approval in other countries and jurisdictions might differ from and be longer than that required to obtain FDA approval. Regulatory approval in one country or jurisdiction does not ensure regulatory approval in another, but a failure or delay in obtaining regulatory approval in one country or jurisdiction may negatively impact the regulatory process in others. Pursuant to the European Clinical Trials Directive, a system for the approval of clinical trials in the European Union has been implemented through national legislation of the member states. Under this system, an applicant must obtain approval from the competent national authority of a European Union member state in which the clinical trial is to be conducted. Furthermore, the applicant may only start a clinical trial after a competent ethics committee has issued a favorable opinion. Clinical trial applications must be accompanied by an investigational medicinal product dossier with supporting information prescribed by the European Clinical Trials Directive and corresponding national laws of the member states and further detailed in applicable guidance documents.

To obtain marketing approval of a drug under European Union regulatory systems, an applicant must submit a marketing authorization application, or MAA, either under a centralized or decentralized procedure.

The centralized procedure provides for the grant of a single marketing authorization by the European Commission that is valid for all European Union member states. The centralized procedure is compulsory for specific products, including for medicines produced by certain biotechnological processes, products designated as orphan medicinal products, advanced therapy products and products with a new active substance indicated for the treatment of certain diseases. For products with a new active substance indicated for the treatment of other diseases and products that are highly innovative or for which a centralized process is in the interest of patients, the centralized procedure may be optional.

Under the centralized procedure, the Committee for Medicinal Products for Human Use, or the CHMP, established at the European Medicines Agency, or EMA, is responsible for issuing an Opinion following the initial assessment of an MAA. Under the centralized procedure, the maximum timeframe for the evaluation of an MAA is 210 days, excluding clock stops, when additional information or written or oral explanation is to be provided by the applicant in response to questions of the

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CHMP. Accelerated evaluation might be granted by the CHMP in exceptional cases, when a medicinal product is of major interest from the point of view of public health and in particular from the viewpoint of therapeutic innovation. In this circumstance, the EMA ensures that the opinion of the CHMP is given within 150 days. Following a positive Opinion by the CHMP the final authorization is issued by the European Commission.

The decentralized procedure is available to applicants who wish to market a product in various European Union member states where such product has not received marketing approval in any European Union member states before. The decentralized procedure provides for approval by one or more other, or concerned, member states of an assessment of an application performed by one member state designated by the applicant, known as the reference member state. Under this procedure, an applicant submits an application based on identical dossiers and related materials to the reference member state and concerned member states. The reference member state prepares a draft assessment report and drafts of the related materials within 120 days after receipt of a valid application. Within 90 days of receiving the reference member state's assessment report and related materials, each concerned member state must decide whether to approve the assessment report and related materials.

If a member state cannot approve the assessment report and related materials on the grounds of potential serious risk to public health, the disputed points are subject to a dispute resolution mechanism and may eventually be referred to the European Commission, whose decision is binding on all member states.

#### Data and Market Exclusivity in the European Union

In the European Union, new chemical entities qualify for eight years of data exclusivity upon marketing authorization and an additional two years of market exclusivity. This data exclusivity, if granted, prevents regulatory authorities in the European Union from referencing the innovator's data to assess a generic (abbreviated) application for eight years, after which generic marketing authorization can be submitted, and the innovator's data may be referenced, but not approved for two years. The overall ten-year period will be extended to a maximum of eleven years if, during the first eight years of those ten years, the marketing authorization holder obtains an authorization for one or more new therapeutic indications which, during the scientific evaluation prior to their authorization, are held to bring a significant clinical benefit in comparison with existing therapies. Even if a compound is considered to be a new chemical entity and the sponsor is able to gain the prescribed period of data exclusivity, another company nevertheless could also market another version of the drug if such company can complete a full MAA with a complete database of pharmaceutical test, nonclinical tests and clinical trials and obtain marketing approval of its product.

#### Pharmaceutical Coverage, Pricing and Reimbursement

Significant uncertainty exists as to the coverage and reimbursement status of products approved by the FDA and other government authorities. Sales of products will depend, in part, on the extent to which third-party payors, including government health programs in the United States such as Medicare and Medicaid, commercial health insurers and managed care organizations, provide coverage, and establish adequate reimbursement levels, for such products. The process for determining whether a payor will provide coverage for a product may be separate from the process for setting the price or reimbursement rate that the payor will pay for the product once coverage is approved. Third-party payors are increasingly challenging the prices charged for medical products and services and imposing controls to manage costs. Third-party payors may limit coverage to specific products on an approved list, or formulary, which might not include all of the approved products for a particular indication. In order to secure coverage and reimbursement for any product that might be approved for sale, a company may need to conduct expensive pharmacoeconomic studies in order to demonstrate the medical necessity and cost-effectiveness of the product, in addition to the costs required to obtain FDA or other comparable regulatory approvals. 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 maintain price levels high enough to realize an appropriate return on our investment in product development.

The containment of healthcare costs has also become a priority of federal, state and foreign governments, and the prices of drugs have been a focus in this effort. Governments have shown significant interest in implementing cost-containment programs, including price controls, restrictions on reimbursement and requirements for substitution of generic products, and re-importation of products sold outside of the United States. Adoption of price controls and cost-containment measures, and adoption of more restrictive policies in jurisdictions with existing controls and

measures, could adversely affect our net revenue and results.

Outside of the United States, ensuring adequate coverage and payment for products remains challenging. Pricing of prescription pharmaceuticals is subject to governmental control in many countries. Pricing negotiations with governmental authorities can extend well beyond the receipt of regulatory marketing approval for a product and may require us to conduct a

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clinical trial that compares the cost effectiveness of our product candidates or products to other available therapies. The conduct of such a clinical trial could be expensive and result in delays in our commercialization efforts. As a result, the marketability of any product which receives 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 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, as amended by the Health Care and Education Affordability Reconciliation Act, 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 that receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

In the European Union, 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 it may instead adopt a system of direct or indirect controls on the profitability of the company 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 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. Any country that has price controls or reimbursement limitations for drug products may not allow favorable reimbursement and pricing arrangements for any of our products.

#### Healthcare Law and Regulation

Healthcare providers, physicians and third-party payors will play a primary role in the recommendation and prescription of drug products that are granted marketing approval. Arrangements with third-party payors and customers are subject to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we market, sell and distribute our products for which we obtain marketing approval. Restrictions under applicable federal and state healthcare laws and regulations, include the following:

the federal healthcare Anti-Kickback Statute prohibits, among other things, persons and entities from knowingly and willfully soliciting, offering, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward either the referral of an individual for, or the purchase, order or recommendation of, any good or service, for which payment may be made, in whole or in part, under a federal healthcare program such as Medicare and Medicaid;

the federal civil and criminal false claims laws, including the False Claims Act, which imposes civil monetary penalties, and provides for 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 or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government;

the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which imposes federal criminal and civil liability for, among other things, knowingly and willingly executing, or attempting to execute, a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters;

HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act and its implementing regulations, also imposes obligations, including mandatory contractual terms, with respect to safeguarding the privacy, security and transmission of individually identifiable health information;

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 Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, or the Affordable Care Act, which requires certain manufacturers of drugs,

devices, biologics and medical supplies to report to the Department of Health and Human Services information related to payments and other transfers of value to physicians and teaching hospitals and physician ownership and investment interests; and

analogous state and foreign laws and regulations, such as state anti-kickback and false claims laws, which may apply to healthcare items or services that are reimbursed by non-governmental third-party payors, including private insurers. Some state laws require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government in addition to requiring drug manufacturers to report information related to payments to physicians and other health care providers or marketing expenditures. State and foreign laws also govern the privacy and security of health information in some circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

#### Regulation of Deuterium Oxide

We believe that all of the deuterium that we use in manufacturing our product candidates is currently derived, directly or indirectly, from deuterium oxide. For most of our deuterium supply we rely on bulk supplies of deuterium oxide, which we currently source from multiple suppliers, including two located in North America, one of which is located in the United States. In order to internationally transport any deuterium oxide that we purchase from foreign suppliers, we, or our U.S. supplier, may be required to obtain an export license from the country of origin and we may be required to obtain an International Import Certificate from the country of destination. We are also generally required to obtain an export license from the Nuclear Regulatory Commission before shipping deuterium oxide from the United States to any contract manufacturer in another country. Each of these documents specifies the maximum amount of deuterium oxide that we, or our suppliers, are permitted to either import or export. We have obtained a license from the Nuclear Regulatory Commission, or NRC, for the export of 20,000 kilograms of heavy water over the life of the license, which is valid until January 2020. We have obtained an additional export license from the NRC for the export of 20,000 kilograms of heavy water over the life of the license, which is valid until March 2020. In addition, in order to obtain additional supplies of deuterium oxide from one of the foreign suppliers from which we have previously purchased deuterium oxide, the supplier will be required to obtain an additional export license from the country of origin and, as part of the export license application process, we may be required to obtain a U.S. import certificate. While we and our suppliers have obtained similar licenses and certificates in the past, we or our suppliers may not be able to obtain them in the future in a timely manner or at all. We have not obtained an export license from the country in which our potential future foreign supplier is located. In addition, if any of our product candidates is approved by the FDA, then the FDA will also have regulatory jurisdiction over the manufacture and use of deuterium oxide in such product.

#### EMPLOYEES

As of December 31, 2018, we had 71 employees, 49 of whom were primarily engaged in research and product development activities. A total of 17 employees have Ph.D. degrees. None of our employees are represented by a labor union and we believe our relations with our employees are good.

#### FACILITIES

In the third quarter of 2018, Concert relocated its offices to a new location in Lexington, Massachusetts, consisting of approximately 55,500 square feet of leased office and laboratory space. The term of the new lease expires in 2029.

#### RESEARCH AND DEVELOPMENT

We have dedicated a significant portion of our resources to our efforts to develop our pipeline and product candidates. We incurred research and development expenses of \$43.1 million, \$30.2 million, and \$37.0 million during the years ended December 31, 2018, 2017 and 2016, respectively. We anticipate that a significant portion of our operating expenses in future periods will continue to be related to research and development as we continue to advance our product candidates through clinical development.



LEGAL PROCEEDINGS

The PTAB instituted an IPR brought against our U.S. Patent No. 9,249,149 by Incyte Corporation. The '149 patent claims deuterated analogs of ruxolitinib including CTP-543. In October 2017, the PTAB initially denied the petition to institute the IPR. In November 2017, Incyte filed a request for rehearing of the PTAB's decision. In April 2018, the PTAB granted the request and instituted the IPR. A written decision by the PTAB is expected by April 9, 2019.

#### AVAILABLE INFORMATION

We file reports and other information with the Securities and Exchange Commission, or SEC, as required by the Securities Exchange Act of 1934, as amended, which we refer to as the Exchange Act. You can review our electronically filed reports and other information that we file with the SEC on the SEC's web site at <http://www.sec.gov>.

We were incorporated under the laws of the State of Delaware on April 12, 2006 as Concert Pharmaceuticals, Inc. Our principal executive offices are located at 65 Hayden Avenue, Suite 3000N, Lexington, Massachusetts, 02421, and our telephone number is (781) 860-0045. Our Internet website is <http://www.concertpharma.com>. We make available free of charge through our website our Annual Report on Form 10-K, quarterly reports on Form 10-Q, current reports on Form 8-K and amendments to those reports filed or furnished pursuant to Sections 13(a) and 15(d) of the Exchange Act. We make these reports available through our website as soon as reasonably practicable after we electronically file such reports with, or furnish such reports to, the SEC. In addition, we regularly use our website to post information regarding our business, product development programs and governance, and we encourage investors to use our website, particularly the information in the section entitled "Investors," as a source of information about us. The foregoing references to our website are not intended to, nor shall they be deemed to, incorporate information on our website into this Annual Report on Form 10-K by reference.

Item 1A. Risk Factors.

Our business is subject to numerous risks. The following important factors, among others, could cause our actual results to differ materially from those expressed in forward-looking statements made by us or on our behalf in this Quarterly Report on Form 10-Q and other filings with the Securities and Exchange Commission, or the SEC, press releases, communications with investors and oral statements. Actual future results may differ materially from those anticipated in our forward-looking statements. We undertake no obligation to update any forward-looking statements, whether as a result of new information, future events or otherwise.

RISKS RELATED TO OUR FINANCIAL POSITION AND NEED FOR ADDITIONAL CAPITAL

We have incurred significant losses since inception, expect to incur losses for at least the next several years and may never sustain profitability.

As of December 31, 2018, we had an accumulated deficit of \$116.5 million. We have not generated any revenues from product sales and have financed our operations to date primarily through the public offering of our common stock, private placements of our preferred stock, debt financings and funding from collaborations or other agreements. We have not completed development of any product candidate and have devoted substantially all of our financial resources and efforts to research and development, including nonclinical studies and our clinical development programs. We expect to continue to incur significant expenses and increasing operating losses for at least the next several years. Our net losses may fluctuate significantly from quarter to quarter and year to year. Net losses and negative cash flows have had, and will continue to have, an adverse effect on our stockholders' equity and working capital.

We anticipate that our expenses will increase substantially if and as we:

- continue to develop and conduct nonclinical studies and clinical trials with respect to our product candidates;
- seek to identify additional product candidates;
- in-license or acquire additional product candidates;
- seek marketing approvals for our product candidates that successfully complete clinical trials;
- establish sales, marketing, distribution and other commercial infrastructure in the future to commercialize various products for which we may obtain marketing approval;
- require the manufacture of larger quantities of product candidates for clinical development and potentially commercialization;
- maintain, expand and protect our intellectual property portfolio;
- hire additional personnel;
- add equipment and physical infrastructure to support our research and development; and
- continue to implement the infrastructure necessary to support our product development and help us comply with our obligations as a public company.

Our ability to become and remain profitable depends on our ability to generate revenue. We do not expect to generate significant revenue unless and until we are, or one of our collaborators is, able to successfully commercialize one or more of our product candidates. This will require success in a range of challenging activities, including completing clinical trials of our product candidates, obtaining marketing approval for these product candidates, manufacturing, marketing and selling those products for which we, or our collaborators, may obtain marketing approval, satisfying any post-marketing requirements and obtaining reimbursement for our products from private insurance or government payors. We, and our collaborators, may never succeed in these activities and, even if we do, or one of our collaborators does, we may never generate revenues that are large enough for us to achieve profitability. Even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would decrease the value of our Company and could impair our ability to raise capital, expand our business, maintain our research and development efforts, diversify our pipeline of product candidates or continue our operations. A decline in the value of our Company could cause our stockholders to lose all or part of their investments in us.

We have a limited operating history and no history of commercializing pharmaceutical products, which may make it difficult to evaluate the prospects for our future viability.

We began operations in April 2006. Our operations to date have been limited to financing and staffing our Company, developing our technology and product candidates and establishing collaborations. We have not yet demonstrated an ability to successfully conduct an international multi-center clinical trial, conduct a large-scale pivotal clinical trial, obtain marketing approvals, manufacture product on a commercial scale or arrange for a third party to do so on our behalf, or conduct sales and

marketing activities necessary for successful product commercialization. Consequently, predictions about our future success or viability may not be as accurate as they could be if we had a longer operating history or a history of successfully developing and commercializing pharmaceutical products.

We will need substantial additional funding. If we are unable to raise capital when needed, we could be forced to delay, reduce or eliminate our product development programs or commercialization efforts.

Developing pharmaceutical products, including conducting nonclinical studies and clinical trials, is a very time-consuming, expensive and uncertain process that takes years to complete. We expect our expenses to increase in connection with our ongoing activities, particularly as we initiate new clinical trials of, initiate new research and nonclinical development efforts for and seek marketing approval for, our product candidates, or if we in-license or acquire product candidates. In addition, if we obtain marketing approval for any of our product candidates, we may incur significant commercialization expenses related to product sales, marketing, manufacturing and distribution to the extent that such sales, marketing and distribution are not the responsibility of one of our collaborators. In particular, the costs that we may be required to incur for the manufacture of any product candidate that receives marketing approval may be substantial. Manufacturing a drug at commercial scale may require specialized facilities, processes and materials. Furthermore, we will continue to incur 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 may be forced to delay, reduce or eliminate our research and development programs or any future commercialization efforts.

In any event, our existing cash and cash equivalents and investments will not be sufficient to fund all of the efforts that we plan to undertake or to fund the completion of development of any of our product candidates. Accordingly, we will be required to obtain further funding through public or private equity offerings, debt financings, collaborations and licensing arrangements or other sources. Adequate additional financing may not be available to us on acceptable terms, or at all. Our failure to raise capital when needed would have a negative impact on our financial condition and our ability to pursue our business strategy.

We believe our existing cash and cash equivalents and investments as of December 31, 2018 will enable us to fund our operating expenses and capital expenditure requirements into the second half of 2020. Our estimate as to how long we expect our cash and cash equivalents and investments to be able to continue to fund our operations is based on assumptions that may prove to be wrong, and we could use our available capital resources sooner than we currently expect. Changing circumstances could cause us to consume capital significantly faster than we currently anticipate, and we may need to spend more money than currently expected because of circumstances beyond our control. Our future funding requirements, both short-term and long-term, will depend on many factors, including:

- the progress, timing, costs and results of clinical trials of, and research and nonclinical development efforts for, our product candidates and potential product candidates, including current and future clinical trials;
- our current collaboration agreements and achievement of milestones under these agreements;
- our ability to enter into and the terms and timing of any additional collaborations, licensing, product acquisition or other arrangements that we may establish;
- the number of product candidates that we pursue and their development requirements;
- the outcome, timing and costs of seeking regulatory approvals;
- our headcount growth and associated costs as we expand our research and development and establish a commercial infrastructure;
- the costs of preparing, filing and prosecuting patent applications, maintaining and protecting our intellectual property rights and defending against intellectual property related claims;
- potential litigation costs; and
- the costs of operating as a public company.

Raising additional capital may cause dilution to our stockholders or require us to relinquish rights to our technologies or product candidates.

Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs through a combination of public or private equity offerings, debt financings, additional collaborations and licensing

arrangements, and other sources. We do not have any committed external source of funds, other than potential milestone payments under the Asset Purchase Agreement with Vertex, as well as potential milestone payments and royalties under our existing license agreements, each of which is subject to the achievement of development, regulatory and/or sales-based milestones with respect to our product candidates. To the extent that we raise additional capital through the sale of common stock, convertible securities or other equity securities, the ownership interests of our stockholders may be materially diluted, and the terms of these securities could include liquidation or other preferences and anti-dilution protections that could adversely affect the rights of our

stockholders. In addition, debt financing, if available, would result in increased fixed payment obligations and may involve agreements that include restrictive covenants that limit our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends, that could adversely impact our ability to conduct our business.

If we raise additional funds through collaborations or marketing, distribution or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams or product candidates, or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds 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.

Any future indebtedness could adversely affect our ability to operate our business.

We could in the future incur indebtedness containing financial obligations and restrictive covenants, which could have significant adverse consequences, including:

- requiring us to dedicate a portion of our cash resources to the payment of interest and principal, reducing money available to fund working capital, capital expenditures, product development and other general corporate purposes;
- increasing our vulnerability to adverse changes in general economic, industry and market conditions;
- subjecting us to restrictive covenants that may reduce our ability to take certain corporate actions or obtain further debt or equity financing;
- limiting our flexibility in planning for, or reacting to, changes in our business and the industry in which we compete; and
- placing us at a competitive disadvantage compared to our competitors that have less debt or better debt servicing options.

Any financial obligations or restrictive covenants could negatively impact our ability to conduct our business.

#### RISKS RELATED TO THE DISCOVERY, DEVELOPMENT AND COMMERCIALIZATION OF OUR PRODUCT CANDIDATES

Clinical drug development involves a lengthy and expensive process with an uncertain outcome.

Clinical testing is expensive, time-consuming and uncertain as to outcome. We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, if at all. The clinical development of our product candidates is susceptible to the risk of failure inherent at any stage of drug development, including failure to demonstrate efficacy in a clinical trial or across a broad or definable population of patients, the occurrence of severe or medically or commercially unacceptable adverse events, fraudulent conduct by clinical investigators, failure to comply with protocols, applicable regulatory requirements or other determinations made by the Food and Drug Administration, or FDA, or any comparable foreign regulatory authority that a drug product is not approvable. It is possible that even if one or more of our product candidates has a beneficial effect, that effect will not be detected during clinical evaluation as a result of one or more of a variety of factors, including the size, duration, design, measurements, conduct or analysis of our clinical trials. Conversely, as a result of the same factors, our clinical trials may indicate an apparent positive effect of a product candidate that is greater than the actual positive effect, if any. Similarly, in our clinical trials, we may fail to detect toxicity of or intolerability caused by our product candidates, or mistakenly believe that our product candidates are toxic or not well tolerated when that is not in fact the case.

While we believe that our DCE Platform may enable drug discovery and clinical development that is more efficient and less expensive than conventional small molecule drug research and development, we may not be able to realize the advantages that we expect. In addition, while a key element of our drug discovery and development strategy involves utilizing existing information regarding non-deuterated compounds to assist the discovery and development of deuterated analogs of those compounds, not all of the product candidates that we develop are based on drugs or drug candidates that progressed into advanced clinical development. Particularly in these situations, existing

information regarding the corresponding non-deuterated compound may not be sufficient to mitigate drug development risks.

In addition to the risk of failure inherent in drug development, certain of the deuterated compounds that we, and our collaborators, are developing and may develop in the future may be particularly susceptible to failure to the extent they are based on compounds that others have previously studied or tested, but did not progress in development due to safety, tolerability or efficacy concerns or otherwise. Deuteration of these compounds may not be sufficient to overcome the problems experienced with the corresponding non-deuterated compound.



We may not be able to continue further clinical development of our wholly owned development programs, including CTP-543 and CTP-692. If we are unable to develop, obtain marketing approval for or commercialize our wholly owned development programs, ourselves or through a collaboration, or experience significant delays in doing so, our business could be materially harmed.

We currently have no products approved for sale. The success of our wholly owned development programs will depend on several factors, including:

- in the case of CTP-543, our ability to safely and effectively treat moderate-to-severe alopecia areata;
- in the case of CTP-692, our ability to safely and effectively treat schizophrenia in patients concurrently receiving antipsychotic medication;
- successful and timely completion of clinical trials;
- receipt of marketing approvals from applicable regulatory authorities;
- the performance of our future collaborators, if any, for our programs;
- the extent of any required post-marketing approval commitments to applicable regulatory authorities;
- establishment of supply arrangements with third party raw materials suppliers and manufacturers;
- our ability to manufacture or arrange for the manufacture of our active pharmaceutical ingredients and drug products with sufficient quality, quantity, and reproducibility to support clinical trials and potential future commercialization;
- establishment of arrangements with third party manufacturers to obtain finished drug products that are appropriately packaged for sale;
- obtaining and maintaining patent, trade secret protection, regulatory exclusivity, and freedom to operate, both in the United States and internationally;
- amount of commercial sales, if and when approved;
- a continued acceptable safety profile of our programs following any marketing approval; and
- agreement by third party payors to reimburse patients for the costs of treatment with our products, and the terms of such reimbursement.

If we are unable to successfully develop, receive marketing approval for, and commercialize our wholly owned development programs, or experience delays as a result of any of these factors or otherwise, our business could be materially harmed.

If clinical trials of our product candidates fail to satisfactorily demonstrate safety and efficacy to the FDA and other regulators, we, or our collaborators, may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of these product candidates.

We, or our collaborators, must complete nonclinical development and then conduct extensive clinical trials to demonstrate the safety and efficacy of our product candidates in humans in order to obtain marketing approval from regulatory authorities for the sale of our product candidates. Clinical testing is expensive, difficult to design and implement, can take many years to complete and is inherently uncertain as to outcome. Further, the outcome of nonclinical studies and early clinical trials may not be predictive of the success of later clinical trials, and interim results of a clinical trial do not necessarily predict final results. Moreover, nonclinical and clinical data are often susceptible to varying interpretations and analyses. Many companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in late-stage clinical trials after achieving positive results in earlier development, and we cannot be certain that we will not face similar setbacks.

Any inability to successfully complete nonclinical and clinical development could result in additional costs to us, or our collaborators, and impair our ability to generate revenues from product sales, regulatory and commercialization milestones and royalties. In addition, if (1) we, or our collaborators, are required to conduct additional or larger clinical trials or other testing of our product candidates beyond the trials and testing that we, or they, contemplate, (2) we, or our collaborators, are unable to successfully complete clinical trials of our product candidates or other testing, (3) the results of these trials or tests are unfavorable, uncertain or are only modestly favorable, or (4) there are unacceptable safety concerns associated with our product candidates, we, or our collaborators, in addition to incurring additional costs, may:

- be delayed in obtaining marketing approval for our product candidates;
- not obtain marketing approval at all;
- obtain approval for indications or patient populations that are not as broad as intended or desired;
- obtain approval with labeling that includes significant use or distribution restrictions or significant safety warnings, including boxed warnings;
- be subject to additional post-marketing testing or other requirements; or
- be required to remove the product from the market after obtaining marketing approval.

Even if we, or our collaborators, believe that the results of clinical trials for our product candidates warrant marketing approval, the FDA or comparable foreign regulatory authorities may disagree and may not grant marketing approval of our product candidates.

If we, or our collaborators, experience any of a number of possible unforeseen events in connection with clinical trials of our product candidates, potential marketing approval or commercialization of our product candidates could be delayed or prevented.

We, or our collaborators, may experience numerous unforeseen events during, or as a result of, clinical trials that could delay or prevent marketing approval of our product candidates, including:

- toxicity or serious adverse effects may be observed in our nonclinical studies causing us to delay or abandon clinical trials;

- clinical trials of our product candidates may produce unfavorable or inconclusive results;

- unexpectedly high placebo response rates;

- inter-rater variability in the assessment of clinical endpoints;

- we, or our collaborators, may decide, or regulators may require us or them, to conduct additional clinical trials and or develop and or validate new clinical endpoints for our clinical trials, or abandon product development programs;

- the number of patients required for clinical trials of our product candidates may be larger than we, or our

- collaborators, anticipate, patient enrollment in these clinical trials may be slower than we, or our collaborators, anticipate or participants may drop out of these clinical trials at a higher rate than we, or our collaborators, anticipate;

- our third party contractors or those of our collaborators, including those manufacturing our product candidates or components or ingredients thereof or conducting clinical trials on our behalf or on behalf of our collaborators, may fail to comply with regulatory requirements or meet their contractual obligations to us or our collaborators in a timely manner or at all;

- regulators or institutional review boards may not authorize us, our collaborators or our or their investigators to commence a clinical trial or conduct a clinical trial at a prospective trial site;

- we, or our collaborators, may have delays in reaching or fail to reach agreement on acceptable clinical trial contracts or clinical trial protocols with prospective trial sites;

- patients that enroll in a clinical trial may misrepresent their eligibility to do so or may otherwise not comply with the clinical trial protocol, resulting in the need to drop the patients or the sites from the clinical trial, increase the needed enrollment size for the clinical trial, extend the clinical trial's duration or cause spurious results;

- investigators may provide inaccurate or false data, resulting in spurious clinical results, an inadequate data set or regulators' unwillingness to approve a product;

- regulators, institutional review boards or data monitoring committees may require that we, or our collaborators, or our or their investigators suspend or terminate clinical research for various reasons, including noncompliance with

- regulatory requirements or their standards of conduct, a finding that the participants are being exposed to unacceptable health risks, undesirable side effects or other unexpected characteristics of the product candidate or findings of undesirable effects caused by a chemically or mechanistically similar drug or drug candidate;

- the FDA or comparable foreign regulatory authorities may disagree with our or our collaborators' clinical trial design or our or their interpretation of data from nonclinical studies and clinical trials;

- the FDA or comparable foreign regulatory authorities may change their requirements for approvability for a given product or for an indication after we have initiated work based on their previous guidance;

- the supply or quality of raw materials or manufactured product candidates or other materials necessary to conduct clinical trials of our product candidates may be insufficient, inadequate or not available at an acceptable cost, or we may experience interruptions in supply;

- we, or our manufacturing vendors, may not produce, or may not consistently produce material that meets necessary specifications for commercialization;

- the FDA or comparable foreign regulatory authorities may determine that our, or our manufacturing vendors, manufacturing or quality control processes fail to meet their specifications or guidelines; and

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the approval policies or regulations of the FDA or comparable foreign regulatory authorities may significantly change in a manner rendering our clinical data insufficient to obtain marketing approval.

Product development costs for us, or our collaborators, will increase if we, or they, experience delays in testing or pursuing marketing approvals and we, or they, may be required to obtain additional funds to complete clinical trials and prepare for possible commercialization of our product candidates. We, and our collaborators, do not know whether any nonclinical tests or clinical trials will begin as planned, will need to be restructured or will be completed on schedule, or at all. Significant nonclinical or clinical trial delays also could shorten any periods during which we, or our collaborators, may have the exclusive right to commercialize our product candidates or allow our competitors, or the competitors of our collaborators, to bring

products to market before we, or our collaborators, do and impair our ability, or the ability of our collaborators, to successfully commercialize our product candidates and may harm our business and results of operations. In addition, many of the factors that cause, or lead to, clinical trial delays may ultimately lead to the denial of marketing approval of any of our product candidates.

If we, or our collaborators, experience delays or difficulties in the enrollment of patients in clinical trials, our, or their, receipt of necessary regulatory approvals could be delayed or prevented.

We, or our collaborators, may not be able to initiate or continue clinical trials for any of our product candidates if we, or they, are unable to locate and enroll a sufficient number of eligible patients to participate in clinical trials as required by the FDA or comparable foreign regulatory authorities, such as the European Medicines Agency. Patient enrollment is a significant factor in the timing of clinical trials, and is affected by many factors, including:

- the size and nature of the patient population;
- the severity of the disease under investigation;
- the proximity of patients to clinical sites;
- the eligibility criteria for the trial;
- the design of the clinical trial, including any requirement to halt current therapy in connection with the trial;
- the potential need to discontinue investigational treatment at the completion of the study;
- access to relevant clinical trial sites;
- efforts to facilitate timely enrollment;
- competing clinical trials;
- support by relevant industry or patient organizations with influence over clinical trial sites; and
- clinicians' and patients' perceptions as to the potential advantages and risks of the drug being studied in relation to other available therapies, including any new drugs that may be approved or used for the indications we are investigating.

Our inability, or the inability of our collaborators, to enroll a sufficient number of patients for our, or their, clinical trials could result in significant delays or may require us or them to abandon one or more clinical trials altogether. Enrollment delays in our, or their, clinical trials may result in increased development costs for our product candidates, delay or halt the development of and approval processes for our product candidates and jeopardize our, or our collaborators', ability to commence sales of and generate revenues from our product candidates, which could cause the value of our Company to decline and limit our ability to obtain additional financing, if needed.

Fast Track designation by the FDA may not lead to a faster development, regulatory review or approval.

Although CTP-543 has been granted Fast Track designation by FDA for the treatment of alopecia areata, Fast Track designation does not necessarily lead to a faster development pathway or regulatory review process, and does not increase the likelihood of regulatory approval. The FDA may later withdraw the designation if they believe the designation is no longer supported by the data from our clinical development program.

Serious adverse events, undesirable side effects or other unexpected properties of our product candidates, including those that we have licensed to collaborators, may be identified during development that could delay or prevent the product candidate's marketing approval.

All of our product candidates are in nonclinical and clinical development stages and their risk of failure is high. Serious adverse events or undesirable side effects caused by our product candidates, or competitor products with similar mechanisms of action, could cause us, one of our collaborators, an institutional review board, data monitoring committee, or regulatory authorities to interrupt, amend, delay or halt clinical trials of one or more of our product candidates and could result in a more restrictive label or the delay or denial of marketing approval by the FDA or comparable foreign regulatory authorities. A dose of a deuterated compound could, in comparison to an equal dose of the corresponding non-deuterated compound, result in altered exposure levels, distribution and half-life in the body and alter the levels of particular metabolites that are present in the body. These changes may cause serious adverse events or undesirable side effects that we or our collaborators did not anticipate, whether based on the characteristics of the corresponding non-deuterated compound or otherwise. If any of our product candidates is associated with serious adverse events or undesirable side effects or have properties that are unexpected, we, or our collaborators, may

need to abandon development or limit development of that product candidate to certain uses or subpopulations in which the undesirable side effects or other characteristics are less prevalent, less severe or more acceptable from a risk-benefit perspective. Many compounds that initially showed promise in clinical or earlier stage testing have later been found to cause undesirable or unexpected side effects that prevented further development of the compound. In addition, unexpected adverse clinical effects of a deuterated product candidate, including either those identified by us or deuterated

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analogs of approved drugs being developed by any third parties, may create general concerns regarding deuteration technology that could delay the development of our product candidates.

The increasing use of social media platforms presents risks and challenges.

The increasing use of social media platforms presents risks and challenges. Social media increasingly is being used by third parties to communicate about our drug candidates and the diseases they are designed to treat. We believe that members of the Alopecia Areata community may be more active on social media as compared to other patient populations due to the demographics of this patient population. Social media practices in the pharmaceutical and biotechnology industries are evolving, which creates uncertainty and risk of noncompliance with regulations applicable to our business. For example, patients in clinical trials may use social media platforms to comment on the effectiveness of, or adverse experiences with, a drug candidate which could result in reporting obligations. In addition, there is a risk of inappropriate disclosure of sensitive information or negative or inaccurate posts or comments about us on any social networking website. If any of these events were to occur or we otherwise fail to comply with applicable regulations, we could incur liability, face restrictive regulatory actions or incur other harm to our business. Even if one of our product candidates receives marketing approval, it may fail to achieve the degree of market acceptance by physicians, patients, third party payors and others in the medical community necessary for commercial success and the market opportunity for the product candidate may be smaller than we estimate.

Even if one of our product candidates, including those licensed to our collaborators, is approved by the appropriate regulatory authorities for marketing and sale, it may nonetheless fail to gain sufficient market acceptance by physicians, patients, third party payors, and formulary decision-makers, and others in the medical or patient communities. For example, physicians are often reluctant to switch their patients from existing therapies even when new and potentially more effective or convenient treatments enter the market. Further, patients often acclimate to the therapy that they are currently taking and do not want to switch unless their physicians recommend switching products or they are required to switch therapies due to lack of reimbursement for existing therapies. If any of our product candidates receive negative publicity, patients may choose not to request them even if approved, or may not comply with taking them as prescribed.

Efforts to educate the medical community, and formulary decision-makers, and third party payors on the benefits of our product candidates may require significant resources and may not be successful. If any of our product candidates is approved but does not achieve an adequate level of market acceptance, we may not generate significant revenues and we may not become profitable. The degree of market acceptance of our product candidates, including those licensed to our collaborators, if approved for commercial sale, will depend on a number of factors, including:

- the efficacy and safety of the product;
- the potential advantages of the product compared to alternative treatments;
- the prevalence and severity of any side effects;
- the clinical indications for which the product is approved;
- whether the product is designated under physician treatment guidelines as a first-line therapy or as a second- or third-line therapy;
- limitations or warnings, including distribution or use restrictions or burdensome prescription requirements contained in the product's approved labeling;
- our ability, or the ability of our collaborators, to offer the product for sale at commercially acceptable prices;
- the product's convenience and ease of administration compared to alternative treatments;
- the willingness of the target patient population to try, and of physicians to prescribe, the product;
- the strength of sales, marketing and distribution support;
- the approval of other new products for the same indications;
- the extent and success of counter-detailing efforts by our competitors;
- the organization stability of our collaborators, if any;

• changes in the standard of care for the targeted indications for the product;  
• the timing of market introduction of our approved products as well as competitive products; and  
• availability and amount of reimbursement from government payors, managed care plans and other third party payors. The potential market opportunities for our product candidates are difficult to precisely estimate. Our estimates of the potential market opportunities are predicated on many assumptions including industry knowledge and publications, third party research reports and other surveys. While we believe that our internal assumptions are reasonable, these assumptions involve the exercise of significant judgment on the part of our management, are inherently uncertain and the reasonableness of these



assumptions has not been assessed by an independent source. If any of the assumptions proves to be inaccurate, the actual markets for our product candidates could be smaller than our estimates of the potential market opportunities. If any of our product candidates receives marketing approval and we, or others, later discover that the drug is less pure, homogeneous, or stable than believed, less effective than previously believed, or causes undesirable side effects that were not previously identified or at a higher rate than was projected during clinical development, our ability to market the drug, or that of our collaborators, could be compromised.

Clinical trials of our product candidates are conducted in carefully defined subsets of patients who have agreed to enter into clinical trials. Consequently, it is possible that these individuals are not representative of the actual patient population or that our clinical trials may indicate an apparent positive effect of a product candidate that is greater than the actual positive effect, if any, or alternatively fail to identify undesirable side effects. If, following approval of a product candidate, we, or others, discover that the drug is less effective than previously believed or causes undesirable side effects that were not previously identified, any of the following adverse events could occur:

- regulatory authorities may withdraw their approval of the drug and/or seize the drug;
- we, or our collaborators, may be required to recall the drug or change the way the drug is administered;
- additional restrictions may be imposed on the marketing of, or the manufacturing processes for, the particular drug, including the addition of labeling statements, such as a “black box” warning or a contraindication;
- we may be subject to fines, injunctions or the imposition of civil or criminal penalties;
- we, or our collaborators, may be required to operate under a REMS;
- we, or our collaborators, could be sued and held liable for harm caused to patients; and
- the drug may become less competitive.

Any of these events could have a material and adverse effect on our operations and business and could adversely impact our stock price.

If we are unable to establish sales, marketing and distribution capabilities or enter into sales, marketing and distribution arrangements with third parties, we may not be successful in commercializing any product candidates that we develop if and when those product candidates are approved.

We do not have a sales, marketing or distribution infrastructure and as a company have no experience in the sale, marketing or distribution of pharmaceutical products. To achieve commercial success for any approved product, we must either develop a sales and marketing organization or outsource these functions to third parties. We expect to use a combination of third party collaboration, licensing and distribution arrangements and a focused in-house commercialization capability to sell any products that receive marketing approval.

We generally plan to seek to retain full commercialization rights for the United States for products that we can commercialize with a specialized sales force and to retain co-promotion or similar rights for the United States when feasible in indications requiring a larger commercial infrastructure. The development of sales, marketing and distribution capabilities will require substantial resources, will be time-consuming and could delay any product launch. If the commercial launch of a product candidate for which we recruit a sales force and establish marketing and distribution capabilities is delayed or does not occur for any reason, we could have prematurely or unnecessarily incurred these commercialization costs. This may be costly, and our investment could be lost if we cannot retain or reposition our sales and marketing personnel. In addition, we may not be able to hire or retain a sales force in the United States that is sufficient in size or has adequate expertise in the medical markets that we plan to target. If we are unable to establish or retain a sales force and marketing and distribution capabilities, our operating results may be adversely affected. If a potential partner has development or commercialization expertise that we believe is particularly relevant to one of our products, then we may seek to collaborate with that potential partner even if we believe we could otherwise develop and commercialize the product independently.

We currently expect to collaborate with third parties for commercialization in the United States of any products that require a large sales, marketing and product distribution infrastructure. We also expect to commercialize our product candidates outside the United States through collaboration, licensing and distribution arrangements with third parties, if at all. As a result of entering into arrangements with third parties to perform sales, marketing and distribution services, our product revenues or the profitability of these product revenues may be lower, perhaps substantially

lower, than if we were to directly market and sell products in those markets. Furthermore, we may be unsuccessful in entering into the necessary arrangements with third parties or may be unable to do so on terms that are favorable to us. In addition, we may have little or no control over such third parties, and any of them may fail to devote the necessary resources and attention to sell and market our products effectively, or may actively sell a competing product at the expense of selling ours.

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If we do not establish sales and marketing capabilities, either on our own or in collaboration with third parties, we will not be successful in commercializing any of our product candidates that receive marketing approval.

We face substantial competition from other pharmaceutical and biotechnology companies and our operating results may suffer if we fail to compete effectively.

The development and commercialization of new drug products is highly competitive. We expect that we, and our collaborators, will face significant competition from major pharmaceutical companies, specialty pharmaceutical companies and biotechnology companies worldwide with respect to our product candidates that we, or they, may seek to develop or commercialize in the future. Specifically, there are a number of large pharmaceutical and biotechnology companies that currently market and sell products or are pursuing the development of product candidates for the treatment of neurologic disorders, autoimmune disorders and inflammation, which are key indications for our development programs. Our competitors may succeed in developing, acquiring or licensing technologies and drug products that attain preferred reimbursement by payors or are more effective, simpler to use, have fewer or more tolerable side effects or are less costly than any product candidates that we are currently developing or that we may develop or acquire, or which are marketed more effectively, which could render our product candidates obsolete and noncompetitive.

Avanir, a subsidiary of Otsuka, is developing AVP-786 for the treatment of agitation associated with Alzheimer's disease and other neurologic or psychological disorders. There are competing marketed drugs and product candidates in clinical development for each indication. Intra-Cellular Therapies, Axsome Therapeutics, and Otsuka Pharmaceuticals and their partner Lundbeck, are developing treatments for agitation in patients with Alzheimer's disease.

We are developing CTP-543 as an oral agent for the treatment of moderate-to-severe alopecia areata. If CTP-543 receives marketing approval for this indication, it may face competition from a number of other product candidates that are being studied for alopecia areata. A number of companies are pursuing development of oral JAK inhibitors with a range of subtype selectivities for the treatment of alopecia areata, including Aclaris Therapeutics, Eli Lilly and Pfizer.

We are developing CTP-692 as an adjunctive treatment of schizophrenia. There are a number of companies pursuing development for adjunctive treatment of schizophrenia, exploring cognitive or negative symptoms of the disease, including SyneuRx International [Taiwan] Corp.

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than any products that we, or our collaborators, may develop. Our competitors also may obtain FDA or other marketing approval for their products before we, or our collaborators, are able to obtain approval for ours, which could reduce our ability to utilize expedited regulatory pathways and could result in our competitors establishing a strong market position before we, or our collaborators, are able to enter the market.

Many of our existing and potential future competitors have significantly greater financial resources and expertise in research and development, manufacturing, nonclinical testing, conducting clinical trials, obtaining marketing approvals and marketing approved products than we do. Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller or early stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs. We also face competition in the development of deuterated compounds.

Many pharmaceutical and biotechnology companies have begun to cover deuterated analogs of their product candidates in patent applications and may develop these deuterated compounds. Some of these pharmaceutical and biotechnology companies may have significantly greater financial resources and expertise in research and development, manufacturing, nonclinical testing, conducting clinical trials, obtaining marketing approvals and

marketing approved products than we do. In addition, other companies are utilizing deuterium substitution for drug development, including Alkeus Pharmaceuticals, Inc. and DeuteRx LLC. In some cases, these competitors may be interested in developing deuterated compounds that we may be interested in developing for ourselves. In addition, these competitors may enter into collaborative arrangements or business combinations that result in their ability to research and develop deuterated compounds more effectively than us. Our potential competitors also include academic institutions, government agencies and other public and private research organizations.

If our competitors in the development of deuterated compounds are able to grow their intellectual property estates and create new and successful deuterated compounds more effectively than us, our ability to identify additional compounds for nonclinical and clinical development and obtain product revenues in future periods could be compromised, which could result in significant harm to our operations and financial position.

If the FDA or comparable foreign regulatory authorities approve generic versions of any of our products that receive marketing approval, or such authorities do not grant our products appropriate periods of data exclusivity before approving generic versions of our products, the sales of our products could be adversely affected.

Once an NDA is approved, the product covered thereby becomes a “reference listed drug” in the FDA’s publication, “Approved Drug Products with Therapeutic Equivalence Evaluations.” Manufacturers may seek approval of generic versions of reference listed drugs through submission of abbreviated new drug applications, or ANDAs, in the United States. In support of an ANDA, a generic manufacturer need not conduct clinical studies. Rather, the applicant generally must show that its product has the same active ingredient(s), dosage form, strength, route of administration and conditions of use or labeling as the reference listed drug and that the generic version is bioequivalent to the reference listed drug, meaning it is absorbed in the body at the same rate and to the same extent. Generic products may be significantly less costly to bring to market than the reference listed drug and companies that produce generic products are generally able to offer them at lower prices. Thus, following the introduction of a generic drug, a significant percentage of the sales of any branded product or reference listed drug is typically lost to the generic product.

The FDA may not approve an ANDA for a generic product until any applicable period of non-patent exclusivity for the reference listed drug has expired. The Federal Food, Drug, and Cosmetic Act, or FDCA, provides a period of five years of non-patent exclusivity for a new drug containing a new chemical entity. Specifically, in cases where such exclusivity has been granted, an ANDA may not be filed with the FDA until the expiration of five years unless the submission is accompanied by a Paragraph IV certification that a patent covering the reference listed drug is either invalid or will not be infringed by the generic product, in which case the applicant may submit its application four years following approval of the reference listed drug. While we believe that our product candidates contain active ingredients that would be treated as new chemical entities by the FDA and, therefore, if approved, should be afforded at least five years of data exclusivity, the FDA may disagree with that conclusion and may approve generic products after a period that is less than five years. Manufacturers may seek to launch these generic products following the expiration of the applicable marketing exclusivity period, even if we still have patent protection for our product. Competition that our products may face from generic versions of our products could materially and adversely impact our future revenue, profitability and cash flows and substantially limit our ability to obtain a return on the investments we have made in those product candidates.

To the extent we, or our collaborators, market products that are deuterated analogs of generic drugs that are approved or will be approved while we market our products in territories in which the generic drug is available, our products may compete against these generic products and the sales of our products could be adversely affected.

We anticipate that some of the products that we, or our collaborators, may develop will be deuterated analogs of approved drugs that are or will then be available on a generic basis. In addition, if we develop a product that is a deuterated analog of a non-generic approved drug, the FDA or comparable foreign regulatory authorities may also approve generic versions of the corresponding non-deuterated drug. If approved, we expect that our deuterated products will compete against these generic non-deuterated compounds if they are used in the same indications. Even if the approved indications are different for the deuterated and non-deuterated drugs, the generic non-deuterated drug may be used off-label, negatively affecting sales of our product. Efforts to educate the medical community and third party payors on the benefits of any product that we develop as compared to the corresponding non-deuterated compound, or generic versions of it, may require significant resources and may not be successful. If physicians, rightly or wrongly, do not believe that a product that we, or our collaborators, develop offers substantial advantages over the corresponding non-deuterated compound, or generic versions of the corresponding non-deuterated compound, or that the advantages offered by our product as compared to the corresponding non-deuterated compound, or its generic versions, are not sufficient to merit the increased price over the corresponding non-deuterated compound, or its generic versions, that we, or our collaborators, would seek, physicians might not prescribe that product. In addition,

third party payors may refuse to provide reimbursement for a product that we, or our collaborators, develop when the corresponding non-deuterated compound, or generic versions of the corresponding non-deuterated compound, offer a cheaper alternative therapy in the same indication, or may otherwise encourage use of the corresponding non-deuterated compound, or generic versions of the corresponding non-deuterated compound, over our product, even if our product possesses favorable pharmaceutical properties or is labeled for a different indication.

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Competition that our product candidates may face from any generic non-deuterated product on which our product candidate is based or a later-approved generic version of a branded non-deuterated product on which our product is based, could materially and adversely impact our future revenue, profitability and cash flows and substantially limit our ability to obtain a return on the investments we have made in those product candidates.

Even if we, or our collaborators, are able to commercialize any product candidate that we, or they, develop, the product may become subject to unfavorable pricing regulations, third party payor reimbursement practices or healthcare reform initiatives that could harm our business.

The commercial success of our product candidates will depend substantially, both domestically and abroad, on the extent to which the costs of our product candidates will be paid by health maintenance, managed care, pharmacy benefit and similar healthcare management organizations, or reimbursed by government health administration authorities, private health coverage insurers and other third party payors. Government authorities and third party payors, such as private health insurers and health maintenance organizations, decide which medications they will cover and establish reimbursement levels. The healthcare industry is acutely focused on cost containment, both in the United States and elsewhere. Government authorities and third party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications, which could affect our ability or that of our collaborators to sell our product candidates profitably. These payors may not view our products, if any, as cost-effective, and coverage and reimbursement may not be available to our customers, or those of our collaborators, or may not be sufficient to allow our products, if any, to be marketed on a competitive basis. Cost-control initiatives could cause us, or our collaborators, to decrease the price we, or they, might establish for products, which could result in lower than anticipated product revenues. If reimbursement is not available, or is available only to limited levels, we, or our collaborators, may not be able to successfully commercialize our product candidates. Even if coverage is provided, the approved reimbursement amount may not be high enough to allow us, or our collaborators, to establish or maintain pricing sufficient to realize a sufficient return on our or their investments.

There is significant uncertainty related to third party payor coverage and reimbursement of newly approved drugs. Marketing approvals, pricing and reimbursement for new drug products vary widely from country to country. Some countries require approval of the sale price of a drug 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, or our collaborators, might obtain marketing approval for a product in a particular country, but then be subject to price regulations that delay commercial launch of the product, possibly for lengthy time periods, which may negatively impact the revenues we are able to generate from the sale of the product in that country. Adverse pricing limitations may hinder our ability or the ability of our collaborators to recoup our or their investment in one or more product candidates, even if our product candidates obtain marketing approval.

Third party payor coverage of newly approved drugs may be more limited than the indications for which the drugs are approved by the FDA or comparable foreign regulatory authorities. Moreover, eligibility for reimbursement does not imply that any drug will be paid for in all cases or at a rate that covers our costs, including research, development, manufacture, sale and distribution. Reimbursement rates may vary, by way of example, according to the use of the drug and the clinical setting in which it is used. Reimbursement rates may also be based on reimbursement levels already set for lower cost drugs or may be incorporated into existing payments for other services.

In addition, increasingly, third party payors are requiring higher levels of evidence of the benefits and clinical outcomes of new technologies, requiring burdensome comparison studies with currently approved drugs and challenging the prices charged. We, and our collaborators, cannot be sure that coverage will be available for any product candidate that we, or they, commercialize and, if available, that the reimbursement rates will be adequate. Further, the net reimbursement for drug products may be subject to additional reductions if there are changes to laws that presently restrict imports of drugs from countries where they may be sold at lower prices than in the United States. An inability to promptly obtain coverage and adequate payment rates from both government-funded and private payors for any our product candidates for which we, or our collaborators, obtain marketing approval could have a material adverse effect on our operating results, our ability to raise capital needed to commercialize products and our overall financial condition.

We may not be successful in our efforts to identify or discover additional potential product candidates. A significant portion of our research involves the development of new deuterated compounds using our DCE Platform. These efforts may not be successful in creating compounds that have commercial value or therapeutic utility beyond the corresponding non-deuterated compound, or at all. Our research programs may initially show promise in creating potential product candidates, yet fail to yield viable product candidates for clinical development for a number of reasons, including:



deuterated analogs of existing non-deuterated compounds or newly designed deuterated compounds may not demonstrate satisfactory efficacy or other benefits, such as convenience of dosing, increased tolerability, enhanced formation of desirable active metabolites or reduced formation of toxic metabolites; potential product candidates may, on further study, be shown to have harmful side effects or other characteristics that indicate that they are unlikely to be products that will receive marketing approval and achieve market acceptance; and pharmaceutical and biotechnology companies have begun to claim deuterated analogs of their compounds in patent filings, resulting in otherwise promising deuterated product candidates already being covered by patents or patent applications.

If we are unable to identify suitable additional compounds for nonclinical and clinical development, our ability to develop product candidates and obtain product revenues in future periods could be compromised, which could result in significant harm to our financial position and adversely impact our stock price.

Product liability lawsuits against us could divert our resources, cause us to incur substantial liabilities and limit commercialization of any products that we may develop.

We face an inherent risk of product liability claims as a result of the clinical testing of our product candidates despite obtaining appropriate informed consents from our clinical trial participants. We will face an even greater risk if we or our collaborators commercially sell any product that we may or they may develop. If we cannot successfully defend ourselves against product liability claims, we may incur substantial liabilities or be required to limit commercialization of our product candidates. Regardless of the merits or eventual outcome, liability claims may result in:

- decreased demand for our product candidates or products that we may develop;
- injury to our reputation and significant negative media attention;
- withdrawal of clinical trial participants;
- significant costs to defend litigation;
- distraction to our management diverting focus from business operations and strategy;
- initiation of investigations by regulators;
- product recalls, withdrawals or labeling, marketing or promotional restrictions;
- substantial monetary awards to trial participants or patients;
- loss of revenue; and
- the inability to commercialize any products that we may develop.

Although we maintain product liability insurance coverage, it may not fully cover potential liabilities that we may incur. The cost of any product liability litigation or other proceeding, even if resolved in our favor, could be substantial. We will need to increase our insurance coverage if and when we begin selling any product candidate that receives marketing approval. In addition, insurance coverage is becoming increasingly expensive. If we are unable to obtain or maintain sufficient insurance coverage at an acceptable cost or to otherwise protect against potential product liability claims, it could prevent or inhibit the development and commercial production and sale of our product candidates, which could adversely affect our business, financial condition, results of operations and prospects.

#### RISKS RELATED TO OUR DEPENDENCE ON THIRD PARTIES

We depend on collaborations with third parties for the development and commercialization of some of our product candidates and expect to continue to do so in the future. Our prospects with respect to those product candidates will depend in significant part on the success of those collaborations.

We have entered into collaborations for the development and commercialization of certain of our product candidates and expect to enter into additional collaborations in the future. We have limited control over the amount and timing of resources that our collaborators dedicate to the development or commercialization of our product candidates and our ability to generate revenues from these arrangements will depend on our collaborators' abilities to successfully perform the functions assigned to them in these arrangements. In addition, our collaborators have the right to abandon research or development projects and terminate applicable agreements, including funding obligations, prior to or upon the expiration of the agreed upon terms.

Collaborations involving our product candidates pose a number of risks, including:

- collaborators have significant discretion in determining the efforts and resources that they will apply to these collaborations;
- collaborators may not perform their obligations as expected;

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collaborators may not pursue development and commercialization of our product candidates or may elect not to continue or renew development or commercialization programs, based on clinical trial results, changes in the collaborators' strategic focus or available funding or external factors, such as an acquisition, that divert resources or create competing priorities;

collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a product candidate, repeat or conduct new clinical trials or require a new formulation of a product candidate for clinical testing;

- collaborators may conduct their clinical trials poorly or inadequately, harming our products, including our products' development in other territories;

product candidates developed in collaboration with us, including in particular product candidates based on deuteration of a collaborator's marketed drugs or advanced clinical candidates, may be viewed by our collaborators as competitive with their own product candidates or products, which may cause collaborators to cease to devote resources to the commercialization of our product candidates;

a collaborator with marketing and distribution rights to one or more products may not commit sufficient resources to the marketing and distribution of such product or products;

disagreements with collaborators, including disagreements over proprietary rights, contract interpretation or the preferred course of development, might cause delays or termination of the research, development or commercialization of product candidates, might lead to additional responsibilities for us with respect to product candidates, or might result in litigation or arbitration, any of which would be time-consuming and expensive;

collaborators may steal our trade secrets or may hire valuable employees from us;

collaborators may fail to protect our trade secrets;

collaborators may not properly maintain or defend our intellectual property rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential litigation;

collaborators may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability; and

collaborations may be terminated and, if terminated, may result in a need for additional capital to pursue further development or commercialization of the applicable product candidates.

Collaboration agreements may not lead to development or commercialization of product candidates in the most efficient manner or at all. If a collaborator of ours is involved in a business combination, it could decide to delay, diminish or terminate the development or commercialization of any product candidate licensed to it by us.

We expect to seek to establish additional collaborations, and if we are not able to establish them on commercially reasonable terms, we may have to alter our development and commercialization plans.

Our drug development programs and the potential commercialization of our product candidates will require substantial additional cash to fund expenses. We may seek one or more collaborators for the development and commercialization of one or more of our product candidates.

We face significant competition in seeking appropriate collaborators. Whether we reach a definitive agreement for collaboration will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator's evaluation of a number of factors. Those factors may include the potential differentiation of our product candidate from its corresponding non-deuterated analog, design or results of clinical trials, the likelihood of approval by the FDA or comparable foreign regulatory authorities and the regulatory pathway for any such approval, the potential market for the product candidate, the proposed collaborator's perception of our freedom to operate in a particular market or markets without challenge, the costs and complexities of manufacturing and delivering the product to patients and the potential of competing products. The collaborator may also consider alternative product candidates or technologies that may be available for collaboration and whether such collaboration could be more attractive than the one with us for our product candidate. Collaborations are complex and time-consuming to negotiate and document. In addition, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators. We are also restricted under the terms of certain of our existing collaboration

agreements from entering into collaborations regarding or otherwise developing specified compounds that are similar to the compounds that are subject to those agreements and collaboration agreements that we enter into in the future may contain further restrictions on our ability to enter into potential collaborations or to otherwise develop specified compounds.

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We may not be able to negotiate collaborations for our product candidates on a timely basis, on acceptable terms, or at all. If we are unable to do so, we may have to limit the development of the product candidate for which we are seeking to collaborate, reduce or delay its development program or one or more of our other development programs, delay its potential commercialization or reduce the scope of any sales or marketing activities, or increase our expenditures and undertake development or commercialization activities at our own expense. If we elect to increase our expenditures to fund development or commercialization activities on our own, we may need to obtain additional capital, which may not be available to us on acceptable terms or at all. If we do not have sufficient funds, we may not be able to further develop our product candidates or bring them to market and generate product revenue. In cases where we seek a collaborator for a product compound that is a deuterated analog of a compound that has been previously developed, failure to enter into a collaboration with the developer of the corresponding non-deuterated compound may result in a loss of the potential to obtain clearance from the FDA to follow expedited development programs that reference and rely on findings previously obtained from the developer's prior nonclinical or clinical studies of the corresponding non-deuterated compound.

We rely on third parties to conduct our clinical trials and some aspects of our research and nonclinical testing. If they terminate their relationships with us or do not perform satisfactorily, our business may be materially harmed.

We do not independently conduct clinical trials of any of our product candidates. We rely on third parties, such as contract research organizations, clinical data management organizations, medical institutions and clinical investigators, to conduct these clinical trials and expect to rely on these third parties to conduct clinical trials of any other product candidate that we develop. We also rely on third parties to conduct some aspects of our research and nonclinical testing and expect to rely on these third parties in the future. Any of these third parties may terminate their engagements with us under certain circumstances. If any of our relationships with these third parties terminate, we may not be able to enter into arrangements with alternative third parties on commercially reasonable terms, or at all. Switching to or adding additional third parties would involve additional cost and require management time and focus. In addition, there is a natural transition period when a new third party commences work, which could result in delays in our product development activities. Although we seek to carefully manage our relationships with our contract research organizations, any such challenges or delays could have a material adverse impact on our business, financial condition and prospects.

Our reliance on these third parties for clinical development activities limits our control over these activities but we remain responsible for ensuring that each of our studies is conducted in accordance with the applicable protocol, legal, regulatory and scientific standards. For example, notwithstanding the obligations of a contract research organization for a trial of one of our product candidates, we remain responsible for ensuring that each of our clinical trials is conducted in accordance with the general investigational plan and protocols for the trial. Moreover, the FDA requires us to comply with standards, commonly referred to as current Good Clinical Practices, or GCPs, for conducting, recording and reporting the results of clinical trials to assure that data and reported results are credible and accurate and that the rights, integrity and confidentiality of trial participants are protected. The FDA enforces these GCPs through periodic inspections of trial sponsors, principal investigators, clinical trial sites and institutional review boards. If we or our third party contractors fail to comply with applicable GCPs, the clinical data generated in our clinical trials may be deemed unreliable and the FDA may require us to perform additional clinical trials before approving our product candidates, which would delay the marketing approval process. We cannot be certain that, upon inspection, the FDA will determine that any of our clinical trials comply with GCPs.

Furthermore, these third parties are not our employees, and except for remedies available to us under our agreements with such contractors, we cannot control whether or not they devote sufficient time, skill and resources to our ongoing development programs. These contractors may also have relationships with other commercial entities, including our competitors, which could impede their ability to devote appropriate time to our clinical programs. If these third parties do not successfully carry out their contractual duties, meet expected deadlines or conduct their services in accordance with our contracts, regulatory requirements or our stated protocols, we may not be able to obtain, or may be delayed in obtaining, marketing approvals for our product candidates. If that occurs, we will not be able to, or may be delayed in our efforts to, successfully commercialize our product candidates. In such an event, our financial results and the commercial prospects for any product candidates that we seek to develop could be harmed, our costs could increase

and our ability to generate revenues could be delayed, impaired or foreclosed.

We also rely on other third parties to store, label and distribute drug supplies for our clinical trials. Any performance failure on the part of our distributors could delay clinical development or marketing approval of our product candidates or commercialization of any resulting products, producing additional losses and depriving us of potential product revenue.

We are also required to register clinical trials and post the results of completed clinical trials on a government-sponsored database, such as ClinicalTrials.gov, within certain timeframes. Failure to do so can result in the inability to report our clinical results in certain publications, fines, adverse publicity and civil and criminal sanctions.

Because there are limited sources of deuterium, we, and our collaborators, are exposed to a number of risks and uncertainties associated with our deuterium supply.

We believe that all of the deuterium that we use in manufacturing our product candidates is currently derived, directly or indirectly, from deuterium oxide. For most of our deuterium supply, we rely on bulk supplies of deuterium oxide which we currently source from multiple suppliers, including two located in North America, one of which is in the United States.

In order to internationally transport any deuterium oxide that we purchase from our current or potential future foreign suppliers, we, or our suppliers, may be required to obtain an export license from the country of origin and we may be required to obtain an International Import Certificate or other governmental approvals or assurances from the country of destination. We are also required to obtain an export license from the Nuclear Regulatory Commission before shipping deuterium oxide from the United States to any contract manufacturer in another country. Export licenses and certain other required documents may specify the maximum amount of deuterium oxide that we, or our suppliers, are permitted to either import or export. In order for us to obtain supplies of deuterium oxide from foreign suppliers, they may be required to obtain an export license from the country of origin and we may be required to obtain domestic governmental approvals or assurances. In addition, our current U.S. export licenses may be insufficient to meet our future requirements. We, or our suppliers, may not be able to obtain such licenses, approvals or assurances in a timely manner or at all.

Certain of our manufacturing processes for our product candidates incorporate deuterium by using deuterated chemical intermediates or reagents that are derived from deuterium oxide. For the deuterated chemical intermediates and reagents, we are not subject to the license requirements applicable to deuterium oxide; however the manufacturer of the deuterated chemical intermediate or reagent may themselves be required to obtain deuterium oxide under applicable licensing requirements. Most of the manufacturers of these deuterated chemical intermediates and reagents are not located in countries that produce bulk quantities of deuterium oxide. Therefore, our ability to source these deuterated chemical intermediates will depend on the ability of these manufacturers to obtain deuterium oxide from other countries. Certain countries may also limit or prohibit the export of deuterium-containing products or intermediates. In the future we may arrange for supplies of deuterated chemical intermediates or reagents from manufacturers located in countries from which they can source deuterium oxide in bulk. However, contract manufacturers in these countries may not represent a viable alternative to our current suppliers. We do not have long-term agreements with our suppliers of deuterated chemical intermediates or reagents and we obtain some of these deuterated chemical intermediates or reagents from single sources, putting us at risk of uncontrolled cost increases or supply interruptions if we cannot establish alternative sourcing arrangements. Deuterated chemical intermediates may be expensive or difficult to obtain or may be produced by specialized techniques that are not widely practiced and we may not be able to enter into arrangements for larger scale supply of deuterated chemical intermediates on acceptable terms, or at all.

We estimate that our current sources of deuterium oxide will be sufficient to meet our anticipated requirements; however, we do not have long-term agreements with our current suppliers. If we are not able to establish or maintain supply arrangements, or any relevant foreign governments decide to withhold authorizations for the export of deuterium oxide that we seek, we may be unable to secure alternative sources. If we are unable to obtain sufficient supplies of deuterium oxide from our current suppliers or our potential future foreign supplier, we would be forced to either seek alternative suppliers of deuterium oxide, likely in other countries, or alternative sources of deuterium. Such alternative supplies may not be available to us on acceptable terms, or at all.

If we are unable to obtain sufficient supplies of deuterium, our ability to produce our product candidates would be impeded and our business, financial condition and prospects could be harmed. In particular, certain of our manufacturing processes are projected to require particularly large quantities of deuterium for late-stage clinical trials and for commercialization. Consequently, any adverse impact on our ability to obtain deuterium oxide from our current suppliers, import deuterium oxide into the United States or export deuterium oxide to our contract manufacturers could have a particularly severe impact on our ability to develop or commercialize those product candidates.

Similarly, to develop and commercialize any of our licensed product candidates, our collaborators will need to obtain supplies of deuterium and will be subject to risks and requirements in connection with sourcing deuterium that are similar to the ones that we face. In addition, if any of our product candidates is approved by the FDA, then the FDA will also have regulatory jurisdiction over the manufacture and use of deuterium oxide and deuterated chemical intermediates or reagents in such products. Any adverse impact on our, or our collaborators', ability to obtain deuterium could delay or prevent the development or commercialization of our product candidates, which could have a material adverse effect on our business.



We contract with third parties for the manufacture and distribution of our product candidates for nonclinical and clinical testing and expect to continue to do so in connection with our future development and commercialization efforts. This reliance on third parties increases the risk that we will not have sufficient quantities of our product candidates or such quantities at an acceptable cost, or that the product candidates will not be of sufficient quality or reproducibility or produced on our desired schedule, which could delay, prevent or impair our development or commercialization efforts.

We currently rely, and expect to continue to rely, on third party contractors to manufacture nonclinical and clinical supplies of our product candidates and to package, label and ship these supplies. We expect to rely on third party contractors to manufacture, formulate, package, label and distribute commercial quantities of any product candidate that we commercialize following approval for marketing by applicable regulatory authorities. Reliance on such third party contractors entails risks, including:

- manufacturing delays, including if our third party contractors give greater priority to the supply of other products over our product candidates or if they otherwise do not satisfactorily perform according to the terms of the agreements between us and them;
- potential industrial accidents such as fires or explosions that compromise our product candidates or the ability of the contractors to timely deliver them;
- the possible termination or nonrenewal of agreements by our third party contractors at a time that is costly or inconvenient for us;
- potentially limited numbers of available contractors due to the need for uncommon equipment or expertise, or pre-existing conflicts of interest;
- the possible breach by the third party contractors of our agreements with them;
- possible theft of intellectual property or trade secrets;
- possible theft of our materials, including starting materials, intermediates, active pharmaceutical ingredients, or drug products;
- the failure of third party contractors to comply with applicable regulatory requirements;
- the possible mislabeling of clinical supplies, potentially resulting in the wrong dose amounts being supplied or active drug or placebo not being properly identified;
- possible contamination, or nonconformance with product or packaging specifications, of our product during or after its manufacture;
- possible interruptions in our contractors' operations, including departure of key personnel, disruption due to merger and acquisitions activities or supply chain disruptions;
- the possibility of clinical supplies not being delivered to clinical sites on time, leading to clinical trial interruptions, or of drug supplies not being distributed to commercial vendors in a timely manner, resulting in lost sales; and
- the possible misappropriation of our proprietary information, including our trade secrets and know-how.

If any of our product candidates are approved by any regulatory agency, we plan to enter into agreements with third party contract manufacturers for the commercial production and distribution of those products. It may be difficult for us to reach agreement with a contract manufacturer on satisfactory terms or in a timely manner, especially if the manufacturer believes it is uniquely suited to use our deuterium chemistry manufacturing processes or otherwise has unusual market power, or that our deuterium chemistry manufacturing processes bear greater production risks than manufacture of non-deuterated compounds. In addition, we may face competition for access to manufacturing facilities as there are a limited number of contract manufacturers operating under current good manufacturing practices, or cGMPs, that are capable of manufacturing our product candidates. Consequently, we may not be able to reach agreement with third party manufacturers on satisfactory terms, which could delay our commercialization efforts.

Third party manufacturers are required to comply with cGMPs and similar regulatory requirements outside the United States. Facilities used by our third party manufacturers must be approved by the FDA after we submit an NDA and before potential approval of the product candidate. Similar regulations apply to manufacturers of our product

candidates for use or sale in foreign countries. We do not directly control the manufacturing process and are completely dependent on our third party manufacturers for compliance with the applicable regulatory requirements for the manufacture of our product candidates. If our manufacturers fail to consistently manufacture material that conforms to the strict regulatory requirements of the FDA and any applicable foreign regulatory authority, or if they unacceptably deviate from standard operating procedures in the production of our product candidates, they will not be able to secure the applicable approval for their manufacturing facilities. If these facilities are not approved for commercial manufacture, we may need to find alternative manufacturing facilities, which could result in delays in obtaining approval for the applicable product candidate.

In addition, our manufacturers are subject to ongoing periodic inspections by the FDA and corresponding state and foreign agencies for compliance with cGMPs and similar regulatory requirements both prior to and following the receipt of marketing

approval for any of our product candidates. Some of these inspections may be unannounced. Failure by any of our manufacturers to comply with applicable cGMPs or other regulatory requirements could result in sanctions being imposed on us, including fines, injunctions, civil penalties, delays, suspensions or withdrawals of approvals, operating restrictions, interruptions in supply and criminal prosecutions, any of which could significantly and adversely affect supplies of our product candidates and have a material adverse impact on our business, financial condition and results of operations.

Our current and anticipated future dependence upon others for the manufacture of our product candidates may adversely affect our future profit margins and our ability to commercialize any products that receive marketing approval on a timely and competitive basis.

#### RISKS RELATED TO OUR INTELLECTUAL PROPERTY

If we are unable to obtain and maintain sufficient patent protection for our product candidates, or if the scope of the patent protection is not sufficiently broad, our competitors could develop and commercialize products similar or identical to ours, and our ability to successfully commercialize our product candidates may be adversely affected. Our success depends in large part on our ability to obtain and maintain patent protection in the United States and other countries with respect to our proprietary product candidates. If we do not adequately protect our intellectual property, competitors may be able to erode or negate any competitive advantage we may have, which could harm our business and ability to achieve profitability. To protect our proprietary position, we file patent applications in the United States and abroad related to our novel product candidates that are important to our business. The patent application and approval process is expensive, uncertain and time-consuming. We may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. Neither deuterium itself, nor the general concept of selective substitution of deuterium for hydrogen in existing pharmaceutical compounds, is patentable; therefore we usually seek patents on a compound-by-compound basis or on a relatively narrow genus of compounds. We are not guaranteed that patents will issue protecting any particular deuterated compound for which we seek patent protection. We also cannot guarantee that another company will not be able to find a different pattern of deuterium substitution that is equally or more effective in improving the characteristics of a non-deuterated compound, then patenting that deuterated compound and competing with us.

Our ability to obtain and maintain patent protection for our product candidates may be limited if disclosures of non-deuterated compounds are held to anticipate or make obvious claims of deuterated analogs of the same or similar compounds in any given territory. In addition, several large pharmaceutical and biotechnology companies have begun to pursue patent protection for deuterated analogs of their products and product candidates, and may in the future obtain patent protection that covers deuterated analogs of those product candidates. If patents directed primarily to non-deuterated compounds are deemed to protect deuterated analogs of those compounds or patent claims on deuterated analogs of compounds become common in the biotechnology and pharmaceutical industries, these factors may substantially limit our ability to seek and obtain patent protection for new product candidates based on deuterium modification of compounds. It may also limit our ability to develop new product candidates based on deuterium modification of such compounds without obtaining a license from those patent holders.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain. No consistent policy regarding the breadth of claims allowed in biotechnology and pharmaceutical patents has emerged to date in the United States or in many foreign jurisdictions. In addition, the determination of patent rights with respect to pharmaceutical compounds commonly involves complex legal and factual questions, which has in recent years been the subject of much litigation. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are highly uncertain.

Assuming the other requirements for patentability are met, currently, the first to file a patent application is generally entitled to the patent. However, prior to March 16, 2013, in the United States, the first to invent was entitled to the patent. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing, or in some cases not at all. Therefore we cannot be certain that we were the first to make the inventions claimed in our patents or pending patent applications, or that we were the first to file for patent protection of such inventions.

We may also become involved in opposition, derivation, reexamination, post grant review, inter partes review or interference proceedings, in the United States or elsewhere, challenging our patent rights or the patent rights of others. For example, in April 2017, Incyte Corporation filed an inter parties review, or IPR, petition with the PTAB, of the U.S. PTO, challenging the validity of U.S. Patent No. 9,249,149, which claims deuterium-modified versions of ruxolitinib, including CTP-543. In October 2017, the PTAB declined to institute the IPR and in November 2017, Incyte filed a request for rehearing of the PTAB's decision. In April 2018, the PTAB granted the request and instituted the IPR and a decision is expected by April 9, 2019. We intend to take

necessary actions to vigorously defend the patent. An adverse determination in any such submission, proceeding or litigation could reduce the scope of, or invalidate, our patent rights, allow third parties to commercialize our technology or product candidates and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize products without infringing third party patent rights.

Our pending and future patent applications may not result in patents being issued which protect our product candidates, in whole or in part, or which effectively prevent others from commercializing competitive products. Changes in either the patent laws or interpretation of the patent laws in the United States and other countries may diminish the value of our patents or narrow the scope of our patent protection. In addition, the laws of foreign countries may not protect our rights to the same extent or in the same manner as the laws of the United States. For example, European patent law restricts the patentability of methods of treatment of the human body more than United States law does.

Even if our patent applications issue as patents, they may not issue in a form that will provide us with any meaningful protection, prevent competitors from competing with us or otherwise provide us with any competitive advantage. Our competitors may be able to circumvent our patents by developing similar or alternative technologies or products in a non-infringing manner. Our competitors may also seek approval to market their own products similar to or otherwise competitive with our products. Alternatively, our competitors may seek to market generic versions of any approved products by submitting ANDAs to the FDA in which they claim that patents owned or licensed by us are invalid, unenforceable or not infringed. In these circumstances, we may need to defend or assert our patents, or both, including by filing lawsuits alleging patent infringement. In any of these types of proceedings, a court or other agency with jurisdiction may find our patents invalid or unenforceable, or that our competitors are competing in a non-infringing manner. In certain territories, losses to an infringing product may not be sufficiently great to justify the costs of challenging the infringer and asserting our rights. In some situations, governments have allowed or enabled the sale of competing products that infringe a company's intellectual property. Thus, even if we have valid and nominally enforceable patents, these patents still may not provide protection against competing products or processes sufficient to achieve our business objectives.

The issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, and our owned and licensed patents may be challenged in the courts or patent offices in the United States and abroad, including challenges through the U.S. Patent and Trademark Office post-grant review procedures. Such challenges may result in loss of exclusivity or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and products. In addition, given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized.

If we are unable to protect the confidentiality of our trade secrets, the value of our technology could be materially adversely affected and our business would be harmed.

While we have obtained composition of matter patents with respect to our most advanced product candidates, our DCE Platform is not patented. In seeking to develop and maintain a competitive position through our DCE Platform and as to other aspects of our business, we rely on trade secrets, including unpatented know-how, technology and other proprietary information. We seek to protect these trade secrets, in part, by entering into non-disclosure and confidentiality agreements with parties who have access to them, such as our consultants, independent contractors, advisors, corporate collaborators, outside scientific collaborators, contract manufacturers, suppliers and other third parties. We also enter into confidentiality and invention or patent assignment agreements with employees and certain consultants. Any party with whom we have executed such an agreement may breach that agreement and disclose our proprietary information, including our trade secrets, and we may not be able to obtain adequate remedies for such breaches. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive and time-consuming, and the outcome is unpredictable. In addition, if any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent such third party, or those to whom they communicate such technology or information, from using that technology or information to compete with us. If any of our trade secrets were to be disclosed to or independently developed by a competitor, our business and

competitive position could be harmed.

Third parties may sue us alleging that we are infringing their intellectual property rights, and such litigation could be costly and time consuming and could prevent or delay us from developing or commercializing our product candidates. Our commercial success depends, in part, on our ability to develop, manufacture, market and sell our product candidates without infringing the intellectual property and other proprietary rights of third parties. Our CTP-543 compound is based, and potential future product candidates may be based, on products that are covered by issued patents or patent applications, the holders of which may attempt to assert claims against us. To date, we are not aware of any judicial decision holding that a

patent that covers a non-deuterated compound should be construed to also cover deuterated analogs thereof, absent specific claims with respect to the deuterated analogs. However, any such judicial decision, or legal proceedings asserting such claims, could increase the likelihood of potential infringement claims being asserted against us. If any third party patents or patent applications are found to cover our product candidates or their methods of use, we may not be free to manufacture or market our product candidates as planned without obtaining a license, which may not be available on commercially reasonable terms, or at all.

For example, CTP-543 is a deuterium-modified version of ruxolitinib. Ruxolitinib is marketed in the U.S. by Incyte Corporation under the name Jakafi. Incyte has patents covering ruxolitinib that may be unexpired if and when we seek marketing approval for CTP-543. Incyte also has a US patent that broadly claims deuterated analogs of ruxolitinib. On June 27, 2017, we filed a Post Grant Review with the Patent Trial and Appeal Board, or PTAB, seeking to invalidate all claims of Incyte's U.S. Patent, which includes claims relating to deuterated ruxolitinib analogs. In January 2018, the PTAB did not grant our petition to challenge the validity of the '335 patent. In May 2018, our request for reconsideration was denied. In October 2017, the PTAB denied a petition by Incyte to institute inter partes review, or IPR, of Concert's U.S. Patent No. 9,249,149. The '149 patent claims deuterated analogs of ruxolitinib including CTP-543. In November 2017, Incyte filed a request for rehearing of the PTAB's decision. In April 2018, the PTAB granted the request and instituted the IPR. A decision is expected by April 9, 2019.

In addition, Columbia University is the assignee of a patent claiming the use of ruxolitinib and other named JAK inhibitors for the treatment of hair loss disorders, including alopecia areata, which may be unexpired if and when we seek marketing approval for CTP-543. If we have to defend ourselves in a patent infringement suit, we may incur significant expenses in doing so. Such litigation could delay our ability to market, or prevent us from marketing, CTP-543.

There is a substantial amount of intellectual property litigation in the biotechnology and pharmaceutical industries, and we may become party to, or threatened with, litigation or other adversarial proceedings regarding intellectual property rights with respect to our products candidates, including interference proceedings before the U.S. Patent and Trademark Office. Third parties may assert infringement claims against us based on existing or future intellectual property rights. The outcome of intellectual property litigation is subject to uncertainties that cannot be adequately quantified in advance. The coverage of patents is subject to interpretation by the courts, and the interpretation is not always uniform. If we are sued for patent infringement, we would need to demonstrate that our product candidates, products or methods either do not infringe the relevant patent claims or that these patent claims are invalid or unenforceable, and we may not be able to do this. Proving invalidity is difficult. For example, in the United States, proving invalidity under most circumstances requires a showing of clear and convincing evidence to overcome the presumption of validity enjoyed by issued patents. We may also assert that a patent claim for a corresponding non-deuterated compound does not cover our product. Even if we are successful in these proceedings, we may incur substantial costs and the time and attention of our management and scientific personnel could be diverted in pursuing these proceedings, which could have a material adverse effect on us. In addition, we may not have sufficient resources to bring these actions to a successful conclusion.

If we are found to infringe a third party's intellectual property rights, we could be forced, including by court order, to cease developing, manufacturing or commercializing the infringing product candidate or product and could be required to pay potentially significant damages. Alternatively, we may be required to obtain a license from such third party in order to use the infringing technology and continue developing, manufacturing or marketing the infringing product candidate. However, we may not be able to obtain any required license on commercially reasonable terms, or at all. Even if we were able to obtain a license, it could be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. In addition, we could be found liable for monetary damages, including treble damages and attorneys' fees if we are found to have willfully infringed a patent. A finding of infringement could prevent us from commercializing our product candidates or force us to cease some of our business operations, which could materially harm our business. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar negative impact on our business.

We may become involved in lawsuits to protect or enforce our patents or other intellectual property, which could be expensive, time consuming and unsuccessful.

Competitors may infringe our patents, trademarks, copyrights or other intellectual property. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time consuming and divert the time and attention of our management and scientific personnel. In any patent infringement proceeding, there is a risk that a court will decide that a patent of ours is invalid or unenforceable, in whole or in part, and that we do not have the right to stop the other party from using the invention at issue. There is also a risk that, even if the validity and enforceability of such patents is upheld, the court will construe the patent's claims narrowly or decide that we do not have the right to stop the other party from using the invention at issue on the grounds that our patent claims do not cover the invention. An adverse outcome in a



litigation or proceeding involving our patents could limit our ability to assert our patents against those parties or other competitors, and may curtail or preclude our ability to exclude third parties from making and selling similar or competitive products. Any of these occurrences could adversely affect our competitive business position, business prospects and financial condition.

Even if we establish infringement, the court may decide not to grant an injunction against further infringing activity and instead award only monetary damages, which may not be an adequate remedy. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during litigation. Moreover, there can be no assurance that we will have sufficient financial or other resources to file and pursue such infringement claims, which typically last for years before they are concluded. Even if we ultimately prevail in such claims, the monetary cost of such litigation and the diversion of the attention of our management and scientific personnel could outweigh any benefit we receive as a result of the proceedings.

#### RISKS RELATED TO REGULATORY APPROVAL AND OTHER LEGAL COMPLIANCE MATTERS

Even if we complete the necessary nonclinical studies and clinical trials the marketing approval process is expensive, time consuming and uncertain and we may not obtain approvals for the commercialization of some or all of our product candidates. As a result, we cannot predict when or if, and in which territories, we, or our collaborators, will obtain marketing approval to commercialize a product candidate.

The research, testing, manufacturing, labeling, approval, selling, marketing, promotion and distribution of drug products are subject to extensive regulation by the FDA and comparable foreign regulatory authorities, which regulations differ from country to country. Failure to obtain marketing approval for a product candidate in a given territory will prevent us and our collaborators from commercializing the product candidate in that territory. Our product candidates are in various stages of development and are subject to the risks of failure inherent in drug development. We, and our collaborators, have not submitted an application for or received marketing approval for any of our product candidates in the United States or in any other jurisdiction. We have limited experience in filing and supporting the applications necessary to gain marketing approvals.

The process of obtaining marketing approvals, both in the United States and abroad, is lengthy, expensive and uncertain. It may take many years, if approval is obtained at all, and can vary substantially based upon a variety of factors, including the type, complexity and novelty of the product candidates involved. This is the case even though the deuterated compounds that we produce and seek to develop can have similar pharmacological properties as their corresponding non-deuterated compounds. Even if, as a result of any such similarities, we, or our collaborators, obtain clearance from the FDA and other regulatory authorities to follow expedited development programs for some deuterated compounds that reference and rely on previous findings for non-deuterated compounds, the review and approval of our product candidates may still take a substantial period of time. Conversely, in certain countries regulators may consider our deuterated compounds to be equivalent to non-deuterated compounds that possess regulatory exclusivity and therefore refuse to approve our compounds until the expiration of that exclusivity.

In addition, changes in marketing approval policies during the development period, changes in or the enactment or promulgation of additional statutes, regulations or guidance or changes in regulatory review for each submitted product application, may cause delays in the approval or rejection of an application. Regulatory authorities have substantial discretion in the approval process and may refuse to accept any application or may decide that our data are insufficient for approval and require additional nonclinical, clinical or other studies. In addition, varying interpretations of the data obtained from nonclinical and clinical testing could delay, limit or prevent marketing approval of a product candidate. Any marketing approval we, or our collaborators, ultimately obtain may be limited or subject to restrictions or post-approval commitments that render the approved product not commercially viable. Any delay in obtaining or failure to obtain required approvals could materially adversely affect our ability or that of our collaborators to generate revenue from the particular product candidate, which likely would result in significant harm to our financial position and adversely impact our stock price.

Failure to obtain marketing approval in international jurisdictions would prevent our product candidates from being marketed abroad.

In order to market and sell our products in the European Union and many other jurisdictions, we, or our collaborators, must obtain separate marketing approvals and comply with numerous and varying regulatory requirements. The approval procedure varies among countries and can involve additional testing. The time required to obtain approval may differ substantially from

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that required to obtain FDA approval. The marketing approval process outside the United States generally includes all of the risks associated with obtaining FDA approval. In addition, in many territories outside the United States, it is required that the product be approved for reimbursement before the product can be approved for sale in that territory. Our products may not receive commercially feasible prices in any given territory, or the price offered for our products in a territory may have an adverse effect on their prices in other territories if we were to accept. We, and our collaborators, may not obtain approvals from regulatory authorities outside the United States on a timely basis, if at all. Approval by the FDA does not ensure approval by regulatory authorities in other countries or jurisdictions, and approval by one regulatory authority outside the United States does not ensure approval by regulatory authorities in other countries or jurisdictions or by the FDA.

Even if we, or our collaborators, obtain marketing approvals for our product candidates, the terms of approvals and ongoing regulation of our products may limit how we, or they, manufacture and market our products, which could materially impair our ability to generate revenue.

Once marketing approval has been granted, an approved product and its manufacturer and marketer are subject to ongoing review and extensive regulation. We, and our collaborators, must therefore comply with requirements concerning advertising and promotion for any of our product candidates for which we or they obtain marketing approval. Promotional communications with respect to prescription drugs are subject to a variety of legal and regulatory restrictions and must be consistent with the information in the product's approved labeling. Thus, we and our collaborators will not be able to promote any products we develop for indications or uses for which they are not approved.

In addition, manufacturers of approved products and those manufacturers' facilities are required to comply with extensive FDA requirements, including ensuring that quality control and manufacturing procedures conform to cGMPs, which include requirements relating to quality control and quality assurance as well as the corresponding maintenance of records and documentation and reporting requirements. We, our contract manufacturers, our collaborators and their contract manufacturers could be subject to periodic unannounced inspections by the FDA to monitor and ensure compliance with cGMPs.

Accordingly, assuming we, or our collaborators, receive marketing approval for one or more of our product candidates, we, and our collaborators, and our and their contract manufacturers will continue to expend time, money and effort in all areas of regulatory compliance, including manufacturing, production, product surveillance and quality control.

If we, and our collaborators, are not able to comply with post-approval regulatory requirements, we, and our collaborators, could have the marketing approvals for our products withdrawn by regulatory authorities and our, or our collaborators', ability to market any future products could be limited, which could adversely affect our ability to achieve or sustain profitability. Further, the cost of compliance with post-approval regulations may have a negative effect on our operating results and financial condition.

Any of our product candidates for which we, or our collaborators, obtain marketing approval in the future could be subject to post-marketing restrictions or withdrawal from the market and we, or our 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.

Any of our product candidates for which we, or our collaborators, obtain marketing approval in the future, as well as the manufacturing processes, post-approval studies and measures, labeling, advertising and promotional activities for such product, among other things, will be subject to continual requirements of and review by the FDA and other regulatory authorities. These requirements include submissions of safety and other post-marketing information and reports, registration and listing requirements, requirements relating to manufacturing, quality control, quality assurance and corresponding maintenance of records and documents, requirements regarding the distribution of samples to physicians and recordkeeping. Even if marketing approval of a product candidate is granted, the approval may be subject to limitations on the indicated uses for which the product may be marketed or to the conditions of approval, including the requirement to implement a Risk Evaluation and Mitigation Strategy, or REMS.

The FDA may also impose requirements for costly post-marketing studies or clinical trials and surveillance to monitor the safety or efficacy of a product. The FDA and other agencies, including the Department of Justice, closely regulate

and monitor the post-approval marketing and promotion of products to ensure that they are manufactured, marketed and distributed only for the approved indications and in accordance with the provisions of the approved labeling. The FDA imposes stringent restrictions on manufacturers' communications regarding off-label use and if we, or our collaborators, do not market any of our product candidates for which we, or they, receive marketing approval for only their approved indications, we, or they, may be subject to warnings or enforcement action for off-label marketing. Violation of the FDCA and other statutes, including the False

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Claims Act, relating to the promotion and advertising of prescription drugs may lead to investigations or allegations of violations of federal and state health care fraud and abuse laws and state consumer protection laws.

In addition, later discovery of previously unknown adverse events or other problems with our products or their manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may yield various results, including:

- restrictions on such products, manufacturers or manufacturing processes;
- restrictions on the indication, patient population, or other parameters for which the drug is approved;
- restrictions on the labeling or marketing of a product;
- restrictions on product distribution or use;
- requirements to conduct post-marketing studies or clinical trials;
- warning letters or untitled letters;
- withdrawal of the products from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- recall of products;
- fines, restitution or disgorgement of profits or revenues;
- reputational damage;
- suspension or withdrawal of marketing approvals;
- refusal to permit the import or export of products;
- product seizure; or
- injunctions or the imposition of civil or criminal penalties.

Recently enacted and future legislation may increase the difficulty and cost for us and our collaborators to obtain marketing approval of and commercialize our product candidates and affect the prices we, or they, may obtain.

In the United States and some foreign jurisdictions, there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could prevent or delay marketing approval of our product candidates, restrict or regulate post-approval activities and affect our ability, or the ability of our collaborators, to profitably sell any products for which we, or they, obtain marketing approval.

In the United States, the Medicare Prescription Drug, Improvement, and Modernization Act of 2003, or the MMA, changed the way Medicare covers and pays for pharmaceutical products. The legislation expanded Medicare coverage for drug purchases by the elderly and introduced a new reimbursement methodology based on average sales prices for physician administered drugs. In addition, this legislation provided authority for limiting the number of drugs that will be covered in any therapeutic class. Cost reduction initiatives and other provisions of this legislation could decrease the coverage and price that we receive for any approved products. While the MMA only addresses drug benefits for Medicare beneficiaries, private payors often follow Medicare coverage policy and payment limitations in setting their own reimbursement rates. Therefore, any reduction in reimbursement that results from the MMA may result in a similar reduction in payments from private payors.

In March 2010, President Obama signed into law the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Affordability Reconciliation Act, or collectively the PPACA.

Among the provisions of the PPACA of potential importance to our product candidates are the following:

- an annual, non-deductible fee on any entity that manufactures or imports specified branded prescription drugs and biologic agents;
- 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 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.

In addition, other legislative changes have been proposed and adopted since the PPACA 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, 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 new laws may result in additional reductions in Medicare and other healthcare funding.

Legislative and regulatory proposals have been made to expand post-approval requirements and restrict sales and promotional activities for pharmaceutical products. We cannot be sure whether additional legislative changes will be enacted, or whether the FDA regulations, guidance or interpretations will be changed, or what the impact of such changes on the marketing approvals of our product candidates, if any, may be. In addition, increased scrutiny by the United States Congress of the FDA's approval process may significantly delay or prevent marketing approval, as well as subject us and our collaborators to more stringent product labeling and post-marketing testing and other requirements.

Our future relationships with customers and third party payors will be subject to applicable anti-kickback, fraud and abuse and other healthcare laws and regulations, which could expose us to criminal sanctions, civil penalties, contractual damages, reputational harm and diminished profits and future earnings.

Healthcare providers, physicians and third party payors will play a primary role in the recommendation and prescription of any products for which we obtain marketing approval. Our future arrangements with third party payors and customers, if any, will subject us to broadly applicable fraud and abuse and other healthcare laws and regulations. The laws and regulations may constrain the business or financial arrangements and relationships through which we market, sell and distribute any products for which we obtain marketing approval. Restrictions under applicable federal and state healthcare laws and regulations in the U.S. include the following:

**Anti-Kickback Statute.** The federal healthcare anti-kickback statute prohibits, among other things, persons from knowingly and willfully soliciting, offering, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward, or in return for, either the referral of an individual for, or the purchase, order or recommendation or arranging of, any good or service, for which payment may be made under a federal healthcare program such as Medicare and Medicaid;

**False Claims Act.** The federal False Claims Act imposes criminal and civil penalties, including through civil whistleblower or qui tam actions, against individuals or entities for, among other things, knowingly presenting, or causing to be presented false or fraudulent claims for payment by a federal healthcare program or making a false statement or record material to payment of a false claim or avoiding, decreasing or concealing an obligation to pay money to the federal government, with potential liability including mandatory treble damages and significant per-claim penalties, currently set at \$5,500 to \$11,000 per false claim;

**HIPAA.** The federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, imposes criminal and civil liability for executing a scheme to defraud any healthcare benefit program or 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, and, as amended by the Health Information Technology for Economic and Clinical Health Act and its implementing regulations, also imposes obligations, including mandatory contractual terms and technical safeguards, with respect to maintaining the privacy, security and transmission of individually identifiable health information;

**Transparency Requirements.** Federal laws require applicable manufacturers of covered drugs to report payments and other transfers of value to physicians, other healthcare providers and teaching hospitals, as well as ownership and investment interests held by physicians and other healthcare providers and their immediate family members; and

**Analogous State and Foreign Laws.** Analogous state and foreign fraud and abuse laws and regulations, such as state anti-kickback and false claims laws can apply to sales or marketing arrangements and claims involving healthcare items or services. In addition, some state laws require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government in addition to requiring drug manufacturers to report information related to payments to physicians and

other health care providers or marketing expenditures and govern the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

Some state laws require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government and require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing



expenditures. State and foreign laws also govern the privacy and security of health information in some circumstances, many of which differ from each other in significant ways and often are not pre-empted by HIPAA, thus complicating compliance efforts.

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 our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, damages, fines, imprisonment, exclusion of products from government funded healthcare programs, such as Medicare and Medicaid, and the curtailment or restructuring of our operations. If any of the physicians or other healthcare providers or entities with whom we expect to do business is 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.

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

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

We maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials, but this insurance may not provide adequate coverage against potential liabilities. We do not maintain insurance for environmental liability or toxic tort claims that may be asserted against us.

In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. Current or future environmental laws and regulations may impair our research, development or production efforts, which could adversely affect our business, financial condition, results of operations or prospects. In addition, failure to comply with these laws and regulations may result in substantial fines, penalties or other sanctions. Governments outside the United States tend to impose strict price controls, which may adversely affect our revenues, if any.

In some countries, such as the countries of the European Union, the pricing of prescription pharmaceuticals is subject to governmental control. In these countries, pricing negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a product. To obtain reimbursement or pricing approval in some countries, we, or our collaborators, may be required to conduct a clinical trial that compares the cost-effectiveness of our product to other available therapies. If reimbursement of our products is unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, our business could be materially harmed.

#### **RISKS RELATED TO EMPLOYEE MATTERS AND MANAGING GROWTH**

Our future success depends on our ability to retain our Chief Executive Officer and other key executives and to attract, retain and motivate qualified personnel.

Our industry has experienced a high rate of turnover of management personnel in recent years. Our ability to compete in the highly competitive biotechnology and pharmaceuticals industries depends upon our ability to attract and retain highly qualified managerial, scientific and medical personnel. We are highly dependent on the pharmaceutical research and development and business development expertise of Roger D. Tung, our President and Chief Executive Officer, as well as the other principal members of our management, scientific and development team. Although we have formal employment agreements with our executive officers, these agreements do not prevent them from

terminating their employment with us at any time. In addition, although we maintain a key-man insurance policy with respect to Dr. Tung, we do not carry key-man insurance on any of our other executive officers or employees and may not carry any key-man insurance in the future.

If we lose one or more of our executive officers, our ability to implement our business strategy successfully could be seriously harmed. Furthermore, replacing executive officers may be difficult and may take an extended period of time because of the limited number of individuals in our industry with the breadth of skills and experience required to develop, gain marketing approval of and commercialize products successfully. Competition to hire from this limited pool is intense, and we may be unable to hire, train, retain or motivate these additional key personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar personnel. We also experience competition for the hiring of scientific and clinical personnel from universities and research institutions. In addition, we rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development and commercialization strategy. Our consultants and advisors may be employed by employers other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us. If we are unable to continue to attract and retain high quality personnel, our ability to develop and commercialize product candidates will be limited.

We expect to grow our organization and, as a result, we may encounter difficulties in managing our growth, which could disrupt our operations.

As our pipeline grows and matures, we expect to experience significant growth in the number of our employees and the scope of our operations, including in the areas of drug manufacturing, regulatory affairs and sales, clinical development, marketing and distribution. Our management may need to divert a disproportionate amount of its attention away from our day-to-day activities to devote time to managing these growth activities. To manage these growth activities, we must continue to implement and improve our managerial, operational and financial systems, expand our facilities and continue to recruit and train additional qualified personnel. Due to our limited financial resources and the limited experience of our management team in managing a company with such anticipated growth, we may not be able to effectively manage the expansion of our operations or recruit and train additional qualified personnel. Moreover, the expected expansion of our operations may lead to significant costs and may divert our business development resources. Any inability to manage growth could delay the execution of our business plans or disrupt our operations.

#### RISKS RELATED TO OUR COMMON STOCK

The price of our common stock may be volatile and fluctuate substantially, which could result in substantial losses for purchasers of our common stock.

The trading price of our common stock has been, and may continue to be, volatile and could be subject to wide fluctuations in response to various factors, some of which are beyond our control. The stock market in general and the market for smaller pharmaceutical and biotechnology companies in particular have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. The market price for our common stock may be influenced by many factors, including:

- the success or failure of existing or new competitive products or technologies;
- the timing, advancement of and results of nonclinical studies and clinical trials of any of our product candidates;
- commencement or termination of collaborations for our development programs;
- failure, delays, changes to or discontinuation of any of our development programs;
- regulatory or legal developments in the United States and other countries;
- regulatory actions relating to our product candidates;
- developments or disputes concerning patent applications, issued patents or other proprietary rights;
- the recruitment or departure of key personnel;
- disclosures by our collaborators relating to our product candidates or competitive programs;
- merger or acquisition activity of our collaborators;
- the level of expenses related to any of our product candidates or clinical development programs;
- the results of our efforts to develop additional product candidates or products;
- actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;
- announcement or expectation of additional financing efforts;

- receipt or expectation of receipt of revenues such as milestones, royalties, grants and license fees;
- sales of our common stock by us, our insiders or other stockholders;
- programmed trading based on technical stock chart or other inputs;
- portfolio restructuring by large shareholders;
- addition or removal of our stock from stock indices;
- variations in our financial results or those of companies that are perceived to be similar to us;
- changes in estimates or recommendations by securities analysts that cover our stock;

actions by short-sellers or supporters of our stock, including social media postings or reports;  
changes in the structure of healthcare payment systems;  
market conditions in the pharmaceutical and biotechnology sectors;  
legalization or the anticipation of possible legalization of drug reimportation from other countries;  
actual or anticipated changes in FDA practices;  
general economic, industry and market conditions; and  
the other factors described in this “Risk Factors” section.

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

Although we have listed our common stock on The NASDAQ Global Market, an active trading market for our common stock may not be sustained. In the absence of an active trading market for our common stock, investors may not be able to sell their common stock at or above the price at which they acquired their shares or at the times that they would like to sell. An inactive trading market may also impair our ability to raise capital to continue to fund operations by selling shares and may impair our ability to acquire other companies or technologies by using our shares as consideration.

We have broad discretion in the use of our cash reserves and may not use them effectively.

Our management has broad discretion to use our cash reserves and could use our cash reserves in ways that do not improve our results of operations or enhance the value of our common stock. The failure by our management to apply these funds effectively could result in financial losses that could have a material adverse effect on our business, cause the price of our common stock to decline and delay the development of our product candidates. Pending their use, we may invest our cash reserves in a manner that does not produce income or that loses value.

We are an “emerging growth company,” and the reduced disclosure requirements applicable to emerging growth companies may make our common stock less attractive to investors.

We are an “emerging growth company,” as defined in the JOBS Act, and may remain an emerging growth company for up to five years from the date of our initial public offering. For so long as we remain an emerging growth company, we are permitted and plan to rely on exemptions from certain disclosure requirements that are applicable to other public companies that are not emerging growth companies. These exemptions include not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002, or SOX Section 404, not being required to comply with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor’s report providing additional information about the audit and the financial statements, reduced disclosure obligations regarding executive compensation and exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and shareholder approval of any golden parachute payments not previously approved. Even after we no longer qualify as an emerging growth company, we may still continue to qualify as a “smaller reporting company,” which would allow us to continue to take advantage of many of the same exemptions from disclosure requirements. We cannot predict whether investors will find our common stock less attractive if we rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

In addition, the JOBS Act provides that an emerging growth company can take advantage of an extended transition period for complying with new or revised accounting standards. This allows an emerging growth company to delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. We have irrevocably elected not to avail ourselves of this exemption from new or revised accounting standards and, therefore, we are subject to the same new or revised accounting standards as other public companies that are not emerging growth companies.

We will continue to incur increased costs as a result of operating as a public company, and our management will be required to devote substantial time to new compliance initiatives and corporate governance practices.

As a public company, we are incurring and expect to incur additional significant legal, accounting and other expenses that we did not incur as a private company. We expect that these expenses will further increase after we are no longer an “emerging growth company.” The Sarbanes-Oxley Act of 2002, or SOX, the Dodd-Frank Wall Street Reform and Consumer Protection Act, the listing requirements of The NASDAQ Global Market and other applicable securities

rules and regulations impose various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. We expect that we will need to hire additional personnel to comply with the requirements of being a public company, and our management and other personnel will need to devote a substantial amount of time towards maintaining compliance with these requirements. These rules and regulations are often subject to varying

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interpretations, in many cases due to their lack of specificity, and, as a result, their application in practice may evolve over time as new guidance is provided by regulatory and governing bodies. This could result in continuing uncertainty regarding compliance matters and higher costs necessitated by ongoing revisions to disclosure and governance practices.

Pursuant to SOX Section 404 we are required to evaluate the effectiveness of our internal control over financial reporting as of the end of each fiscal year and to report on this evaluation in our Annual Report on Form 10-K for the year. However, while we remain an emerging growth company, we will not be required to include an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. We will need to continue to dedicate internal resources, potentially engage outside consultants and adopt a detailed work plan to assess and document the adequacy of internal control over financial reporting, continue steps to improve control processes as appropriate, validate through testing that controls are functioning as documented and implement a continuous reporting and improvement process for internal control over financial reporting. Despite our efforts, there is a risk that when we are required to deliver the attestation report we will not be able to conclude that our internal control over financial reporting is effective as required by SOX Section 404. If we identify one or more material weaknesses, it could result in an adverse reaction in the financial markets due to a loss of confidence in the reliability of our financial statements.

A significant portion of our total outstanding shares may be sold into the market in the near future, which could cause the market price of our common stock to decline significantly, even if our business is doing well.

Sales of a substantial number of shares of our common stock in the public market could occur at any time. These sales, or the perception in the market that the holders of a large number of shares of common stock intend to sell shares, could reduce the market price of our common stock.

In addition, as of February 22, 2019, there were 4,541,732 shares subject to outstanding options and restricted stock units under our equity compensation plans, all of which shares we have registered under the Securities Act on a registration statement on Form S-8. These shares will be able to be freely sold in the public market upon exercise, as permitted by any applicable vesting requirements, except to the extent they are held by our affiliates, in which case such shares will become eligible for sale in the public market as permitted by Rule 144 under the Securities Act. Furthermore, as of February 26, 2019, there were 61,273 shares subject to an outstanding warrant to purchase common stock. These shares will become eligible for sale in the public market, to the extent such warrant is exercised, as permitted by Rule 144 under the Securities Act.

We do not anticipate paying any cash dividends on our capital stock in the foreseeable future, accordingly, stockholders must rely on capital appreciation, if any, for any return on their investment.

We have never declared or paid cash dividends on our capital stock. We currently plan to retain all of our future earnings, if any, to finance the operation, development and growth of our business. Furthermore, any future debt agreements may preclude us from paying dividends. As a result, capital appreciation, if any, of our common stock will be the sole source of gain for our stockholders for the foreseeable future.

Our executive officers, directors and principal stockholders, if they choose to act together, have the ability to substantially influence all matters submitted to stockholders for approval.

As of December 31, 2018, our executive officers and directors, combined with our stockholders who owned more than 5% of our outstanding common stock, and all affiliates, in the aggregate, beneficially owned shares representing approximately 42.6% of our capital stock. As a result, if these stockholders were to choose to act together, they would be able to substantially influence all matters submitted to our stockholders for approval, as well as our management and affairs. For example, these persons, if they choose to act together, would substantially influence the election of directors and approval of any merger, consolidation or sale of all or substantially all of our assets. This concentration of ownership control may:

• delay, defer or prevent a change in control;

• entrench our management or the board of directors; or

• impede a merger, consolidation, takeover or other business combination involving us that other stockholders may desire.

Future sales of a substantial number of our common shares by our principal stockholders could depress the trading price of our common stock.

If our principal stockholders sell substantial amounts of shares of our common stock in the public market or if the market anticipates that these sales could occur, the market price of shares of our common stock could decline. These sales may make it

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more difficult for us to sell equity or equity-related securities in the future at a time and price that we deem appropriate, or to use equity as consideration for future acquisitions.

Provisions in our corporate charter documents and under Delaware law could make an acquisition of us, which may be beneficial to our stockholders, more difficult and may prevent attempts by our stockholders to replace or remove our current management.

Provisions in our corporate charter and our bylaws may discourage, delay or prevent a merger, acquisition or other change in control of us that stockholders may consider favorable, including transactions in which our stockholders might otherwise receive a premium for their shares. These provisions could also limit the price that investors might be willing to pay in the future for shares of our common stock, thereby depressing the market price of our common stock. In addition, because our board of directors is responsible for appointing the members of our management team, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our board of directors. Among other things, these provisions:

- establish a classified board of directors such that all members of the board are not elected at one time;
- allow the authorized number of our directors to be changed only by resolution of our board of directors;
- limit the manner in which stockholders can remove directors from the board;
- establish advance notice requirements for nominations for election to the board of directors or for proposing matters that can be acted on at stockholder meetings;
- require that stockholder actions must be effected at a duly called stockholder meeting and prohibit actions by our stockholders by written consent;
- limit who may call a special meeting of stockholders;
- authorize our board of directors to issue preferred stock without stockholder approval, which could be used to institute a “poison pill” that would work to dilute the stock ownership of a potential hostile acquirer, effectively preventing acquisitions that have not been approved by our board of directors; and
- require the approval of the holders of at least 75% of the votes that all our stockholders would be entitled to cast to amend or repeal certain provisions of our charter or bylaws.

Moreover, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, which prohibits a person who owns in excess of 15% of our outstanding voting stock from merging or combining with us for a period of three years after the date of the transaction in which the person acquired in excess of 15% of our outstanding voting stock, unless the merger or combination is approved in a prescribed manner. This could discourage, delay or prevent someone from acquiring us or merging with us, whether or not it is desired by, or beneficial to, our stockholders.

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

The trading market for our common stock depends on the research and reports that securities or industry analysts publish about us or our business. We do not have any control over these analysts. There can be no assurance that analysts will cover us, or provide favorable coverage. If one or more analysts downgrade our stock or change their opinion of our stock, our share price may decline. In addition, if one or more analysts cease coverage of our Company or fail to regularly publish reports on us, we could lose visibility in the financial markets, which could cause our share price or trading volume to decline.

#### ITEM 1B. Unresolved Staff Comments

None

ITEM 2. Properties

We lease our principal facilities, which consist of approximately 55,500 square feet of office, research and laboratory space located at 65 Hayden Avenue, Lexington, Massachusetts. We relocated to this location in the third quarter of 2018. The lease covering this space expires on January 1, 2029. The lease for our prior principal facility located at 99 Hayden Avenue, Lexington, Massachusetts expired on September 30, 2018. We believe that the new facilities are sufficient for our current needs for the foreseeable future.

ITEM 3. Legal Proceedings

The PTAB instituted an IPR brought against our U.S. Patent No. 9,249,149 by Incyte Corporation. The '149 patent claims deuterated analogs of ruxolitinib including CTP-543. In October 2017, the PTAB initially denied the petition to institute the IPR. In November 2017, Incyte filed a request for rehearing of the PTAB's decision. In April 2018, the PTAB granted the request and instituted the IPR. A written decision by the PTAB is expected by April 9, 2019.

ITEM 4. Mine Safety Disclosures

Not applicable.

Part II

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

MARKET INFORMATION

Our common stock has been publicly traded on the NASDAQ Global Market under the symbol "CNCE" since February 13, 2014.

HOLDERS

As of January 31, 2019, there were 14 holders of record of our common stock. This number does not include beneficial owners whose shares are held by nominees in street name.

DIVIDENDS

We have never declared or paid any cash dividends on our capital stock. We currently intend to retain all of our future earnings, if any, to finance the growth and development of our business. We do not intend to pay any cash dividends to the holders of our common stock in the foreseeable future.

PERFORMANCE GRAPH

The following performance graph and related information shall not be deemed to be "soliciting material" or to be "filed" with the SEC for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, or the Exchange Act, or otherwise subject to the liabilities under that Section, nor shall such information be incorporated by reference into any future filing under the Exchange Act or the Securities Act of 1933, as amended, or the Securities Act, except to the extent that we specifically incorporate it by reference into such filing.

The following graph compares the performance of our common stock to The NASDAQ Composite Index and to The NASDAQ Biotechnology Index from February 13, 2014 (the first date that shares of our common stock were publicly traded) through December 31, 2018. The comparison assumes \$100 was invested after the market closed on February 13, 2014 in our common stock and in each of the foregoing indices, and it assumes reinvestment of dividends, if any. The stock price performance included in this graph is not necessarily indicative of future stock price performance.

**PURCHASE OF EQUITY SECURITIES**

We did not purchase any of our registered equity securities during the period covered by this Annual Report on Form 10-K.

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## ITEM 6. Selected Financial Data

The following tables set forth our selected consolidated financial data and have been derived from our audited consolidated financial statements. You should read the following selected consolidated financial data together with our consolidated financial statements and accompanying notes appearing elsewhere in this Annual Report on Form 10-K and the "Management's Discussion and Analysis of Financial Condition and Results of Operations" section of this Annual Report on Form 10-K. Our historical results for any prior period are not necessarily indicative of the results that may be expected in any future period.

| (in thousands, except per share data)  | Years ended December 31, |            |            |           |            |
|--|--------------------------|------------|------------|-----------|------------|
|  | 2018                     | 2017       | 2016       | 2015      | 2014       |
| <b>Results of Operations</b>   |                          |            |            |           |            |
| Total revenue (1)  | \$ 10,505                | \$ 143,891 | \$ 174     | \$ 66,729 | \$ 8,576   |
| Operating expenses:  |                          |            |            |           |            |
| Research and development   | \$ 43,149                | \$ 30,223  | \$ 36,983  | \$ 28,885 | \$ 27,474  |
| General and administrative   | 22,940                   | 21,019     | 14,358     | 13,056    | 11,700     |
| Total operating expenses   | 66,089                   | 51,242     | 51,341     | 41,941    | 39,174     |
| (Loss) Income from operations  | (55,584 )                | 92,649     | (51,167 )  | 24,788    | (30,598 )  |
| Interest and other (expense) income, net   | (127 )                   | 2,690      | 447        | (185 )    | (1,101 )   |
| Provision (Benefit) for income taxes   | 313                      | (300 )     | —          | 429       | —          |
| Net (loss) income  | \$(56,024)               | \$95,639   | \$(50,720) | \$24,174  | \$(31,699) |
| Net (loss) income applicable to common stockholders - basic  | \$(56,024)               | \$95,195   | \$(50,720) | \$24,174  | \$(31,754) |
| Net (loss) income applicable to common stockholders - diluted  | \$(56,024)               | \$95,210   | \$(50,720) | \$24,174  | \$(31,754) |
| <b>Earnings Per Share</b>  |                          |            |            |           |            |
| Net (loss) income per share applicable to common stockholders - basic  | \$(2.40 )                | \$4.20     | \$(2.28 )  | \$1.14    | \$(2.00 )  |
| Net (loss) income per share applicable to common stockholders - diluted  | \$(2.40 )                | \$4.06     | \$(2.28 )  | \$1.09    | \$(2.00 )  |
| Weighted-average number of common shares used in net (loss) income per share applicable to common stockholders - basic   | 23,370                   | 22,641     | 22,233     | 21,152    | 15,842     |
| Weighted-average number of common shares used in net (loss) income per share applicable to common stockholders - diluted | 23,370                   | 23,442     | 22,233     | 22,267    | 15,842     |
| <b>Financial Condition</b>   |                          |            |            |           |            |
| Cash and cash equivalents  | \$ 17,770                | \$ 27,665  | \$ 40,555  | \$ 92,510 | \$ 13,396  |
| Investments, available for sale  | 135,544                  | 175,500    | 55,630     | 49,680    | 65,836     |
| Working capital  | 171,400                  | 199,289    | 92,159     | 137,481   | 63,102     |
| Total assets   | 192,547                  | 211,736    | 100,395    | 146,932   | 84,454     |
| Deferred revenue   | 10,533                   | 10,301     | 10,050     | 10,170    | 15,821     |
| Loan payable, net of discount  | —                        | —          | —          | —         | 7,101      |
| Total stockholders' equity   | 167,740                  | 196,432    | 85,594     | 130,635   | 54,825     |

(1) On January 1, 2018, we adopted Financial Accounting Standards Board (FASB) Accounting Standards Update (ASU) 2014-19, Revenue from Contracts with Customers ("ASC 606"). For detailed information regarding the adoption of ASC 606 and the impact on our consolidated financial statements, see Note 2 to the accompanying consolidated financial statements appearing elsewhere in this Annual Report on Form 10-K.



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

You should read the following discussion and analysis of our financial condition and results of operations together with our consolidated financial statements and the related notes appearing elsewhere in this Annual Report on Form 10-K. Some of the information contained in this discussion and analysis or set forth elsewhere in this report, including information with respect to our plans and strategy for our business and related financing, includes forward-looking statements that involve risks and uncertainties. You should read the "Risk Factors" section in Part 1—Item 1A. of this report for a discussion of important factors that could cause actual results to differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis.

### OVERVIEW

We are a clinical stage biopharmaceutical company applying our extensive knowledge of deuterium chemistry to discover and develop novel small molecule drugs. Selective incorporation of deuterium into known molecules has the potential, on a case-by-case basis, to provide better pharmacokinetic or metabolic properties, thereby enhancing their clinical safety, tolerability or efficacy. Our approach typically starts with previously studied compounds, including approved drugs, which we believe may be improved with deuterium substitution. Our technology provides the opportunity to develop products that may compete with the non-deuterated drug in existing markets or to leverage its known activity to expand into new indications. Our deuterated chemical entity platform, or DCE Platform®, has broad potential across numerous therapeutic areas. As discussed in detail in Item 1 above, we have a robust pipeline of wholly owned and collaboration programs.

Since our inception in 2006, we have devoted substantially all of our resources to our research and development efforts, including activities to develop our deuterated chemical entity platform, or DCE Platform, and our core capabilities in deuterium chemistry, identify potential product candidates, undertake nonclinical studies and clinical trials, manufacture clinical trial material in compliance with current good manufacturing practices, provide general and administrative support for these operations and establish our intellectual property. We have generated an accumulated deficit of \$116.5 million since inception through December 31, 2018 and will require substantial additional capital to fund our research and development. We do not have any products approved for sale and have not generated any revenue from product sales. We have funded our operations primarily through the public offering and private placement of our equity, debt financing and funding from collaborations, patent assignments, and other arrangements. In March 2015, we sold 3,300,000 shares of common stock at a price to the public of \$15.15 per share, resulting in net proceeds to us of \$46.7 million, after deducting the underwriting discounts, commissions and offering-related transaction costs.

On March 3, 2017, we entered into an Asset Purchase Agreement (the "Asset Purchase Agreement") with Vertex Pharmaceuticals, Inc., through Vertex Pharmaceuticals (Europe) Limited ("Vertex"), pursuant to which we agreed to sell and assign CTP-656, now known as VX-561, and other cystic fibrosis assets of the Company, for up to \$250 million subject to the satisfaction of certain closing conditions. On July 25, 2017, the Asset Purchase Agreement closed and Vertex paid us \$160 million in cash consideration, with \$16 million initially held in escrow, which was released to us in February 2019. Additional information concerning the sale of CTP-656 is discussed further in Note 12 to the consolidated financial statements and Item 1, each appearing elsewhere in this Annual Report on Form 10-K.

The Company's operating results may fluctuate significantly from year to year, depending on the timing and magnitude of cash payments received pursuant to collaboration and licensing arrangements and other agreements and the timing and magnitude of clinical trial and other development activities under our current development programs. We generated a net loss of \$56.0 million for the year ended December 31, 2018, net income of \$95.6 million for the year ended December 31, 2017, and a net loss of \$50.7 million for the year ended December 31, 2016. The net income generated during the year ended December 31, 2017 was primarily the result of the Asset Purchase Agreement with Vertex, discussed above and in further detail in Note 12 to the consolidated financial statements appearing elsewhere in this Annual Report on Form 10-K.

We expect to continue to incur significant expenses and operating losses for at least the next several years. We expect our expenses will increase substantially in connection with our ongoing activities as we continue research and development efforts and develop and conduct additional nonclinical studies and clinical trials with respect to our product candidates.

We do not expect to generate revenue from product sales unless and until we, or our collaborators, obtain marketing approval for one or more of our product candidates, which we expect will take a number of years and is subject to significant uncertainty. If we obtain, or believe that we are likely to obtain, marketing approval for any product candidates for which we retain commercialization rights, and intend to commercialize a product, we expect to incur significant commercialization expenses related to product sales, marketing, manufacturing and distribution. We expect to seek to fund our operations through a



combination of equity offerings, debt financings, additional collaborations and licensing arrangements, and other sources for at least the next several years. However, we may be unable to raise additional funds or enter into such other arrangements when needed on favorable terms or at all. Our failure to raise capital or enter into such other arrangements as and when needed would force us to delay, limit, reduce or terminate our research and development programs and could have a material adverse effect on our financial condition and our ability to develop our products. We will need to generate significant revenues to achieve sustained profitability and we may never do so.

## COLLABORATIONS

We have entered into a number of collaborations for the research, development and commercialization of deuterated compounds. To date, our collaborations have provided us with significant funding for both our specific development programs and our DCE Platform. Our collaborators also have applied their considerable scientific, development, regulatory and commercial capabilities to the development of our compounds. In addition, in some instances, where we develop and seek to collaborate with respect to deuterated analogs of marketed drugs or of drug candidates that are more advanced in clinical trials, our collaborators may be eligible for an expedited development or regulatory pathway by relying on previous clinical data regarding their corresponding non-deuterated compound. We believe that our collaborations have contributed to our ability to progress our product candidates and build our DCE Platform. We have established the following key collaborations, which are discussed further in Note 12 to the consolidated financial statements appearing elsewhere in this Annual Report on Form 10-K.

AVP-786 is a combination of deudextromethorphan and an ultra-low dose of quinidine being investigated for the treatment of neuro-psychiatric disorders that is being developed under a development and license agreement with Avanir. In November 2015, Avanir announced the initiation of the Phase 3 clinical program to evaluate the safety and efficacy of AVP-786 for the treatment of agitation associated dementia of the Alzheimer's type. It expects to enroll approximately 850 patients in two U.S. Phase 3 trials. The two North American Phase 3 trials are expected to be completed in 2019 and are expected to be part of the NDA package. Additionally, in October 2017, Avanir initiated a global Phase 3 trial which is expected to enroll approximately 400 patients to evaluate the safety and efficacy of AVP-786 for the treatment of agitation associated with dementia of the Alzheimer's type.

CTP-730 is a deuterated analog of apremilast that is being developed under a collaboration with Celgene. In January 2019, Celgene announced their intent to merge with Bristol-Myers Squibb. Apremilast is a selective phosphodiesterase 4 (PDE4) inhibitor approved in various countries for the treatment of moderate to severe psoriasis and psoriatic arthritis. We have completed the Phase 1 clinical evaluation of CTP-730. Once daily dosing of 50 mg of CTP-730 administered for seven days in the Phase 1 clinical trial demonstrated similar steady state exposure to historical data for 30 mg of apremilast twice daily. Treatment with CTP-730 was generally well-tolerated and no serious adverse events were observed. Celgene is responsible for any development of CTP-730 beyond the completed Phase 1 clinical trials. Celgene is assessing the path forward for CTP-730. However, CTP-730 has not advanced into new trials at this time.

Jazz Pharmaceuticals is evaluating several formulation and technology options as part of its once nightly oxybate program. Jazz Pharmaceuticals initially evaluated JZP-386, a deuterium containing high sodium analog of Xyrem, which demonstrated favorable deuterium-related effects. However its current once-nightly development efforts are focused on lower sodium compounds. The collaboration with Jazz Pharmaceuticals provides for the evaluation of deuterium as an option for a once nightly sodium oxybate product (D-SXB).

## ASSET PURCHASE AGREEMENT

On March 3, 2017, we entered into an Asset Purchase Agreement with Vertex pursuant to which we sold and assigned CTP-656 and other cystic fibrosis assets of the Company to Vertex. On July 25, 2017, the transaction contemplated by the Asset Purchase Agreement closed, and Vertex paid us \$160 million in cash consideration with \$16 million initially held in escrow, which was released in February 2019. Additional information concerning the sale of CTP-656 is discussed further in Note 12 to the consolidated financial statements appearing elsewhere in this Annual Report on

Form 10-K.

Additionally, upon the achievement of certain milestone events, Vertex has agreed to pay us an aggregate of up to \$90 million. Of this amount, \$50 million will become payable to us upon receipt of FDA marketing approval for a combination treatment regimen containing CTP-656, now known as VX-561, for patients with cystic fibrosis, and \$40 million will become payable to us upon completion of a pricing and reimbursement agreement in the first of the United Kingdom, Germany or France with respect to a combination treatment regimen containing CTP-656 for patients with cystic fibrosis.

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## FINANCIAL OPERATIONS OVERVIEW

### Revenue

We have not generated any revenue from the sales of products. All of our revenue to date has been generated through collaboration, license and research arrangements with collaborators and nonprofit organizations for the development and commercialization of product candidates, a patent assignment agreement, and an asset sale. On January 1, 2018, we adopted Financial Accounting Standards Board (FASB) Accounting Standards Update (ASU) 2014-19, Revenue from Contracts with Customers ("ASC 606" or "the new revenue standard"). We adopted ASC 606 using the modified retrospective approach. For detailed information regarding the adoption of ASC 606 and the impact on our consolidated financial statements, see Note 2 to the accompanying consolidated financial statements appearing elsewhere in this Annual Report on Form 10-K.

The terms of these agreements may include one or more of the following types of payments: non-refundable license fees, payments for research and development activities, payments based upon the achievement of specified milestones, payment of license exercise or option fees relating to product candidates and royalties on any net product sales. To date, we have received non-refundable upfront payments, several milestone payments, payments for research and development services provided to our collaborators, a change in control payment pursuant to a patent assignment agreement, and a payment for the sale of an asset. However, we have not yet earned any license exercise or option fees, sales-based milestone payments or royalty revenue as a result of product sales.

In the future, we will seek to generate revenue from a combination of product sales and milestone payments and royalties on product sales in connection with our current collaborations, our asset sale with Vertex, or other collaborations we may enter into.

### Research and development expenses

Research and development expenses consist primarily of costs incurred for the development of our product candidates, which include:

- employee-related expenses, including salary, benefits, travel and stock-based compensation expense;
- expenses incurred under agreements with contract research organizations and investigative sites that conduct our clinical trials;
- the cost of acquiring, developing and manufacturing clinical trial materials;
- facilities, depreciation and other expenses, which include direct and allocated expenses for rent and maintenance of facilities, insurance and other supplies;
- platform-related lab expenses, which includes costs related to synthesis, analysis and in vitro and in vivo characterization of deuterated compounds to support the selection and progression of potential product candidates;
- expenses related to consultants and advisors; and
- costs associated with nonclinical activities and regulatory operations.

Research and development costs are expensed as incurred. Costs for certain development activities are recognized based on an evaluation of the progress to completion of specific tasks using information and data provided to us by our vendors and our clinical sites.

A significant portion of our research and development costs have been external costs, which we track on a program-by-program basis. These external costs include fees paid to investigators, consultants, central laboratories and contract research organizations in connection with our clinical trials, and costs related to acquiring and manufacturing clinical trial materials. Our internal research and development costs are primarily personnel-related costs, depreciation and other indirect costs. We do not track our internal research and development expenses on a program-by-program basis as they are deployed across multiple projects under development.

The successful development of any of our product candidates is highly uncertain. As such, at this time, we cannot reasonably predict with certainty the duration and completion costs of the current or future clinical trials of any of our product candidates or if, when, or to what extent we will generate revenues from the commercialization and sale of any of our product candidates that obtain marketing approval. We may never succeed in achieving regulatory approval for any of our product candidates. The duration, costs, and timing of clinical trials and development of our product

candidates will depend on a variety of factors, including:

• the scope and rate of progress of our ongoing as well as any additional clinical trials and other research and development activities;

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• conduct of and results from ongoing as well as any additional clinical trials and research and development activities;  
• significant and changing government regulation;  
• the terms and timing and receipt of any regulatory approvals;  
• the performance of our collaborators;  
• our ability to manufacture any of our product candidates that we are developing or may develop in the future; and  
• the expense and success of filing, prosecuting, defending and enforcing any patent claims and other intellectual property rights, including potential claims that we infringe other parties' intellectual property.

A change in the outcome of any of these variables with respect to the development of a product candidate could mean a significant change in the costs and timing associated with the development of that product candidate. For example, if the FDA or another regulatory authority were to require us to conduct clinical trials or other research and development activities beyond those that we currently anticipate will be required for the completion of clinical development of a product candidate, or if we experience significant delays in enrollment in any of our clinical trials, we could be required to expend significant additional financial resources and time on the completion of clinical development. 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, due to the increased size and duration of later-stage clinical trials and the manufacturing that is typically required for those later-stage clinical trials. We expect research and development costs to increase significantly for the foreseeable future as our product candidate development programs progress but 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 related costs for personnel, including stock-based compensation and travel expenses for our employees in executive, operational, finance, legal, business development and human resource functions. Other general and administrative expenses include facility-related costs, depreciation and other expenses not allocated to research and development expense and professional fees for directors, accounting and legal services and expenses associated with obtaining and maintaining patents. In 2017, we also incurred expenses responding to the Federal Trade Commission's requests for information and documentation in connection with their review of the transaction contemplated by the Vertex Asset Purchase Agreement. In both 2018 and 2017, we incurred expenses for intellectual property matters related to CTP-543.

We anticipate that our general and administrative expenses will increase in the future as our pipeline grows and matures. Additionally, if and when we believe a regulatory approval of the first product candidate that we intend to commercialize on our own appears likely, we anticipate an increase in payroll and related expenses as a result of our preparation for commercial operations, especially as it relates to the sales, marketing and distribution of our product candidates.

#### Investment income

Investment income consists of interest income earned on cash equivalents and investments. The amount of investment income earned in any particular period may vary primarily as a result of the amount of cash equivalents and investments held during the period and the types of securities included in our portfolio during the period. Our current investment policy is to maintain a diversified investment portfolio of U.S. government-backed securities and money market mutual funds consisting of U.S. government-backed securities.

#### Unrealized Gains (Losses) on Marketable Equity Securities

As discussed in Note 2 to the consolidated financial statements appearing elsewhere in this Annual Report on Form 10-K, effective January 1, 2018 we prospectively adopted ASU 2016-01, Recognition and Measurement of Financial Assets and Financial Liabilities, resulting in the recognition of the effects of changes in fair value of marketable equity

securities within net income. Unrealized gains (losses) on marketable equity securities consists of changes in the fair value of common shares of Processa held by us, discussed further in Note 12 to the consolidated financial statements appearing elsewhere in this Annual Report on Form 10-K.

#### Interest and other expense

Interest and other expense consists primarily of interest expense on amounts outstanding under our prior debt facilities with Hercules Technology Growth Capital, Inc., or Hercules, and amortization of debt discount. On June 8, 2017, we entered into a loan agreement with Hercules in the amount of \$30.0 million which we then paid off on September 7, 2017 in the amount of \$30.8 million pursuant to a payoff letter. All our outstanding indebtedness and obligations owed to Hercules were paid in full, and the loan agreement was terminated. Additional information regarding the debt facility is available in Note 13 of the consolidated financial statements appearing elsewhere in this Annual Report on Form 10-K.

#### Income Taxes

We record a provision or benefit for income taxes on pre-tax income or loss based on our estimated effective tax rate for the year. We recorded \$0.3 million in income tax expense and \$0.3 million in income tax benefit during the years ended December 31, 2018 and 2017, respectively. No tax provision was recorded during the year ended December 31, 2016 due to the net loss generated. The tax expense in 2018 is primarily the result of taxes assessed on the \$16 million initially held in escrow under the Asset Purchase Agreement with Vertex. The tax benefit realized in fiscal year 2017 is the result of the enactment of the Tax Cuts and Jobs Act (TCJA) that changed corporate alternative minimum tax ("AMT"), resulting in an expected refund for AMT paid in fiscal year 2015. As of December 31, 2015, the U.S. federal tax code limited the use of net operating loss carryforwards to ninety percent of AMT income resulting in an effective tax rate of approximately two percent.

#### Loss on extinguishment of debt

In connection with the loan agreement entered into with Hercules on June 8, 2017 and subsequently repaid on September 7, 2017, we recognized a loss on the extinguishment of debt for \$1.4 million. Additional information regarding the debt facility is available in Note 13 to the consolidated financial statements appearing elsewhere in this Annual Report on Form 10-K.

#### 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 have been prepared in accordance with U.S. generally accepted accounting principles. The preparation of these financial statements requires us to make judgments and estimates that affect the reported amounts of assets, liabilities, revenues, and expenses and the disclosure of contingent assets and liabilities in our financial statements. We base our estimates on historical experience, known trends and events, and various other factors that are believed to be reasonable under the circumstances. Actual results may differ from these estimates. On an ongoing basis, we evaluate our judgments and estimates in light of changes in circumstances, facts and experience. The effects of material revisions in estimates, if any, will be reflected in the consolidated financial statements prospectively from the date of change in estimates.

While our significant accounting policies are described in more detail in the notes to our consolidated financial statements appearing elsewhere in this Annual Report on Form 10-K, we believe the following accounting policies used in the preparation of our financial statements require the most significant judgments and estimates:

- revenue recognition;
- accrued research and development expense;
- stock-based compensation; and
- income taxes.

#### Revenue recognition

We have primarily generated revenue through arrangements with collaborators for the development and commercialization of product candidates. In fiscal year 2017, we generated revenue through an Asset Purchase Agreement with Vertex.

We adopted ASC 606 effective January 1, 2018. ASC 606 is a single comprehensive model for entities to use in accounting for revenue arising from contracts with customers and supersedes most current revenue recognition

guidance, including industry-specific guidance. The new revenue standard is based on the principle 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. The new revenue standard also requires additional disclosure about the nature, amount, timing and uncertainty of revenue and cash flows arising from customer contracts, and costs to obtain or fulfill contracts. We applied ASC 606 on January 1, 2018 to all contracts using the modified retrospective approach. For additional details regarding



our adoption of ASC 606 and our associated accounting policies, refer to Notes 2 and 12 to the consolidated financial statements appearing elsewhere in this Annual Report on Form 10-K.

#### Accrued research and development expenses

As part of the process of preparing our financial statements, we are required to estimate our accrued expenses as of each balance sheet date. This process involves reviewing open contracts and purchase orders, communicating with our personnel to identify 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. We periodically confirm the accuracy of our estimates with the service providers and make adjustments if necessary. Examples of estimated accrued research and development expenses include fees paid to:

• contract research organizations in connection with clinical trials;

- investigative sites in connection with clinical trials;

• vendors in connection with nonclinical development activities; and

• vendors related to product manufacturing, development and distribution of clinical supplies.

We generally accrue expenses related to research and development activities based on the services received and efforts expended pursuant to contracts with multiple contract research organizations that conduct and manage clinical trials on our behalf as well as other vendors that provide research and development services. The financial terms of these agreements are subject to negotiation, vary from contract to contract and may result in uneven payment flows. There may be instances in which payments made to our vendors will exceed the level of services provided and result in a prepayment of the clinical expense. Payments under some of these contracts depend on factors such as the successful enrollment of subjects and the completion of clinical trial milestones. In accruing service fees, we estimate the time period over which services will be performed and the level of effort to be expended in each period. If the actual timing of the performance of services or the level of effort varies from our estimate, we adjust the accrual or prepaid accordingly. Non-refundable advance payments for goods and services that will be used in future research and development activities are expensed when the activity has been performed or when the goods have been received rather than when the payment is made.

Although we do not expect our estimates to be materially different from amounts actually incurred, if our estimates of the status and timing of services performed differ from the actual status and timing of services performed, we may report amounts that are too high or too low in any particular period. To date, there have been no material differences from our estimates to the amounts actually incurred.

#### Stock-Based Compensation

Since our inception in May 2006, we have applied the fair value recognition provisions of FASB Accounting Standards Codification (ASC) Topic 718, Compensation-Stock Compensation, which we refer to as ASC 718, to account for stock-based compensation arrangements with our employees. Stock-based compensation arrangements with non-employees has not been significant. We use the Black-Scholes-Merton option pricing model for determining the estimated fair value for stock-based awards on the date of grant, which requires the use of subjective assumptions to determine the fair value of stock-based awards, including the award's expected term and the price volatility of the underlying stock. We recognize the value of the portion of the awards that is ultimately expected to vest as expense over the requisite vesting periods on a ratable basis for the entire award. Our awards granted to employees generally have a ten year term and typically vest over a four year period.

Expected volatility was estimated using a weighted-average of our historical volatility of our common stock and the historical volatility of the common stock of a representative group of publicly traded companies from the biopharmaceutical industry with similar characteristics as us, including stage of product development and therapeutic

focus. We will continue to apply this process until a sufficient amount of historical information regarding the volatility of our own stock price becomes available.

The expected term of awards represents the period of time that the awards are expected to be outstanding. We use the simplified method as prescribed by the Securities and Exchange Commission Staff Accounting Bulletin No. 107, Share-Based Payment as we do not have sufficient historical exercise data to provide a reasonable basis upon which to estimate the expected term of stock options granted to employees.

We utilize a dividend yield of zero based on the fact that we have never paid cash dividends and have no current intention of paying cash dividends. The risk-free interest rate was estimated using an average of treasury bill interest rates over a period

commensurate with the expected term of the option at the time of grant. Forfeitures are estimated at the time of grant and revised, if necessary, in subsequent periods if actual forfeitures differ from those estimates.

We have computed the fair value of employee stock options at the date of grant using the following weighted-average assumptions:

|                         | Year ended December 31, |   |           |   |           |   |
|-------------------------|-------------------------|---|-----------|---|-----------|---|
|                         | 2018                    |   | 2017      |   | 2016      |   |
| Expected volatility     | 77.22                   | % | 78.15     | % | 78.29     | % |
| Expected term           | 6.0 years               |   | 6.0 years |   | 6.0 years |   |
| Risk-free interest rate | 2.64                    | % | 2.07      | % | 1.36      | % |
| Expected dividend yield | —                       | % | —         | % | —         | % |

We granted restricted stock units and performance stock units to our employees and members of our senior management team. We recognize compensation expense for restricted stock units ratably over the required service period. For awards with performance conditions in which the award does not vest unless the performance condition is met, we recognize expense only if we estimate that achievement of the performance condition is probable. If we conclude that vesting is probable, we recognize expense from the date that we reach this conclusion through the estimated vesting date using an accelerated attribution method.

#### Income Taxes

We record income taxes under the liability method. Deferred tax assets and liabilities reflect our estimation of the future tax consequences of temporary differences between the carrying amounts of assets and liabilities for book and tax purposes. We determine deferred income taxes based on the differences in accounting methods and timing between financial statement and income tax reporting. Accordingly, we determine the deferred tax asset or liability for each temporary difference based on the enacted tax rates expected to be in effect when we realize the underlying items of income and expense. We consider many factors when assessing the likelihood of future realization of our deferred tax assets, including our recent earnings experience, expectations of future taxable income, and the carryforward periods available to us for tax reporting purposes, as well as other relevant factors. We establish a valuation allowance to reduce deferred tax assets to the amount we believe is more likely than not to be realized. Due to inherent complexities arising from the nature of our business, future changes in income tax law, or variances between our actual and anticipated operating results, we make certain judgments and estimates, including our ability to realize our deferred tax assets and our ability to use our operating loss carryforwards and tax credits to offset taxable income. Therefore, actual income taxes could materially vary from these estimates.

Our ability to use our operating loss carryforwards and tax credits to offset taxable income is subject to restrictions under Sections 382 and 383 of the United States Internal Revenue Code (the “Internal Revenue Code”). Net operating loss and tax credit carryforwards may become subject to an annual limitation in the event of certain cumulative changes in the ownership interest of significant shareholders over a three-year period in excess of 50 percent, as defined under Sections 382 and 383 of the Internal Revenue Code. Such changes would limit our use of operating loss carryforwards and tax credits. In such a situation, we may be required to pay income taxes, even though significant operating loss carryforwards and tax credits exist. In determining the tax provisions for fiscal years 2017 and 2015, we assessed our ability to use our net operating loss carryforwards in accordance with Sections 382 and 383 of the Internal Revenue Code, discussed further in Note 10 to the notes to the consolidated financial statements appearing elsewhere in this Annual Report on Form 10-K.

On December 22, 2017, the President of the United States signed into law the Tax Cuts and Jobs Act (“TCJA”). This legislation makes broad and complex changes to the U.S. tax code, including, but not limited to, (i) reducing the U.S. federal statutory tax rate from 35% to 21%; (ii) eliminating the corporate alternative minimum tax (AMT) and

changing how existing AMT credits can be realized; (iii) modifying the officer's compensation limitation, and (iv) changing rules related to uses and limitations of net operating loss carryforwards created in tax years beginning after December 31, 2017.

As a result of the enacted law, we were required to revalue deferred tax assets and liabilities existing as of December 31, 2017 from the 35% federal rate in effect through the end of 2017, to the new 21% rate. Furthermore, we recorded a reduction to our deferred tax assets and a corresponding reduction to our valuation allowance. Accordingly, there was no impact to our income statement due to the reduction in the U.S. corporate tax rate. Due to the changes to corporate AMT, we recorded an AMT benefit in fiscal year 2017 due to the expected refund for AMT paid in fiscal year 2015.

Our preliminary estimate of the TCJA and the remeasurement of our deferred tax assets and liabilities was subject to the finalization of management's analysis related to certain matters, such as developing interpretations of the provisions of the TCJA, changes to certain estimates and the filing of our tax returns. The final determination of the TCJA and the remeasurement of our deferred assets and liabilities was completed during 2018 as permitted in accordance with Staff Accounting Bulletin No. 118, and there was no change from the initial estimate.

For additional details regarding our accounting for income taxes, see Note 10 to the consolidated financial statements appearing elsewhere in this Annual Report on Form 10-K.

#### PENDING AND RECENTLY ADOPTED ACCOUNTING PRONOUNCEMENTS

For detailed information regarding recently issued accounting pronouncements and the expected impact on our consolidated financial statements, see Note 2 to the consolidated financial statements appearing elsewhere in this Annual Report on Form 10-K.

#### RESULTS OF OPERATIONS

Comparison of the years ended December 31, 2018 and 2017

The following table summarizes our results of operations for the years ended December 31, 2018 and 2017, together with the changes in those items in dollars.

| (in thousands)                                  | Year ended December 31, |           |             |
|---|-------------------------|-----------|-------------|
|   | 2018                    | 2017      | Change      |
| Revenue:  |                         |           |             |
| License and research and development revenue    | \$ 10,505               | \$ 62     | \$ 10,443   |
| Other revenue                                   | —                       | 143,829   | (143,829 )  |
| Total revenue                                   | 10,505                  | 143,891   | (133,386 )  |
| Operating expenses:                             |                         |           |             |
| Research and development                        | 43,149                  | 30,223    | 12,926      |
| General and administrative                      | 22,940                  | 21,019    | 1,921       |
| Total operating expenses                        | 66,089                  | 51,242    | 14,847      |
| (Loss) Income from operations                   | (55,584 )               | 92,649    | (148,233 )  |
| Investment income                               | 2,787                   | 1,336     | 1,451       |
| Other income                                    | 12                      | 3,601     | (3,589 )    |
| Interest and other expense                      | —                       | (815 )    | 815         |
| Loss on extinguishment of debt                  | —                       | (1,432 )  | 1,432       |
| Unrealized loss on marketable equity securities | (2,926 )                | —         | (2,926 )    |
| (Loss) Income before income taxes               | (55,711 )               | 95,339    | (151,050 )  |
| Provision (Benefit) for income taxes            | 313                     | (300 )    | 613         |
| Net (loss) income                               | \$ (56,024 )            | \$ 95,639 | \$(151,663) |

#### License and Research and Development Revenue

License and research and development revenue was \$10.5 million for the year ended December 31, 2018 as compared to \$62 thousand for the prior year period, an increase of \$10.4 million. In 2018, we granted Processa an exclusive, worldwide, royalty-bearing license to develop, manufacture and commercialize CTP-499 in exchange for upfront consideration of 2,090,301 shares of common stock of Processa with a fair value of \$10.5 million on the date of the transaction, which was recorded as license and research and development revenue during fiscal year 2018. For further details related to our transaction with Processa, refer to Note 12 to the consolidated financial statements appearing elsewhere in this Annual Report on Form 10-K. License and research and development revenue in the 2017 period was for services performed under our Celgene and Jazz Pharmaceuticals collaboration agreements.



## Other Revenue

Other revenue recognized during the year ended December 31, 2017 of \$143.8 million was attributable to the closing of the transaction contemplated by the Asset Purchase Agreement with Vertex, discussed in detail in Note 12 to the consolidated financial statements appearing elsewhere in this Annual Report on Form 10-K.

Effective January 1, 2018, we adopted ASC 606 using the modified retrospective approach. As a result of the adoption, the cumulative impact to retained earnings at January 1, 2018 was \$15.8 million. Of the \$15.8 million, \$16.0 million was attributable to the recognition of a contract asset related to the escrow payment that became due in January 2019 under our Vertex Asset Purchase Agreement. Because the amount was recognized in the ASC 606 transition adjustment, the escrow payment did not result in additional revenue when the cash was received in February 2019. For additional details related to our adoption of ASC 606, see Notes 2 and 12 to the consolidated financial statements appearing elsewhere in this Annual Report on Form 10-K.

As of December 31, 2018, we had deferred revenue of:

\$7.8 million related to our collaboration with Celgene, consisting of \$1.3 million related to the R&D Services Performance Obligation, \$0.1 million related to the Supply Performance Obligation and \$6.4 million related to the First and Second Discount Performance Obligations, as discussed further in Note 12 to the consolidated financial statements appearing elsewhere in this Annual Report on Form 10-K; and \$2.8 million related to a payment received from GSK.

## Research and development expenses

The following table summarizes our external research and development expenses, by program, for the years ended December 31, 2018 and 2017, with our internal research expenses separately classified by category. Because Avanir is conducting the clinical development of AVP-786 at its expense, we made no investment in the program during these periods.

| (in thousands)                           | Year ended December 31, |           |
|--|-------------------------|-----------|
|  | 2018                    | 2017      |
| CTP-543 external costs                   | \$ 9,254                | \$ 6,299  |
| CTP-692 external costs                   | 7,417                   | —         |
| CTP-656 external costs                   | —                       | 3,076     |
| CTP-730 external costs                   | —                       | 19        |
| External costs for other programs        | 3,147                   | 1,481     |
| Employee and contractor-related expenses | 17,876                  | 15,685    |
| Facility and other expenses              | 5,455                   | 3,663     |
| Total research and development expenses  | \$ 43,149               | \$ 30,223 |

Research and development expenses were \$43.1 million for the year ended December 31, 2018, compared to \$30.2 million for the prior year period, an increase of \$12.9 million. This increase was primarily due to an increase of \$7.4 million and \$3.0 million in direct external expenses associated with CTP-692 and CTP-543, respectively. The increase in CTP-692 external expenses in 2018 was attributed to costs incurred related to Phase 1 clinical and manufacturing activities to support Phase 1 and Phase 2 activities. The increase in CTP-543 external expenses was driven by the ongoing Phase 2 clinical study and manufacturing costs to support continued advancement of the CTP-543 program.

The cessation of CTP-656 external expenses in 2018 was attributable to the sale of CTP-656 to Vertex under the Asset Purchase Agreement in 2017.

The \$1.7 million increase in external costs for other programs is due to additional spending to identify additional product candidates.

The increase in employee and contractor-related expenses was primarily attributable to increased non-cash stock-based compensation expenses due to RSUs granted in the third quarter of 2017.



The increase in facility and other-related expense was primarily attributable to increased rent expense and depreciation for our new office and laboratory facility.

#### General and administrative expenses

General and administrative expenses were \$22.9 million for the year ended December 31, 2018, compared to \$21.0 million for the prior year. The increase of \$1.9 million was primarily attributable to a \$1.5 million increase in non-cash stock-based compensation expenses due to RSUs granted in the third quarter of 2017. Facility and other-related expenses increased by \$0.8 million during the 2018 period, due to increased rent expense and depreciation for our new office and laboratory facility, but were offset by a \$0.8 million net decrease in consulting and professional fees during the 2018 period following the closing of the CTP-656 Asset Purchase Agreement in 2017.

#### Investment income

Investment income was \$2.8 million for the year ended December 31, 2018, compared to \$1.3 million for the prior year period. The increase in investment income was attributable to an increase in investments held during the 2018 period as well as higher yields on our portfolio held during the 2018 period.

#### Other income

Other income was \$12 thousand during the year ended December 31, 2018, compared to \$3.6 million in the prior year. Other income in the year 2017 was due to the disgorgement of short-swing profits arising from sales of the Company's stock by a 10% stockholder pursuant to Section 16(b) of the Securities and Exchange Act of 1934.

#### Interest and other expense

Interest expense of \$0.8 million recorded during the year ended December 31, 2017 was attributable to the interest that was due under our loan facility with Hercules and amortization of the loan discount. All our outstanding indebtedness and obligations owed to Hercules were paid in full, and the loan agreement was terminated in September 2017. Additional information regarding the debt facility is available in Note 13 to the consolidated financial statements appearing elsewhere in this Annual Report on Form 10-K.

#### Loss on extinguishment of debt

As a result of the prepayment of the debt facility with Hercules, we recognized a loss on the extinguishment of debt of \$1.4 million. All our outstanding indebtedness and obligations owed to Hercules were paid in full on September 7, 2017, and the loan agreement was terminated.

#### Unrealized loss on marketable equity securities

The unrealized loss on marketable equity securities of \$2.9 million consists of changes in the fair value of common shares of Processa held by us, discussed further in Note 12 to the consolidated financial statements appearing elsewhere in this Annual Report on Form 10-K.

#### Provision for income taxes

We recorded \$0.3 million in income tax expense during the year ended December 31, 2018. The 2018 provision primarily consists of interest owed to Federal and State tax authorities due to the deferral of \$16 million in income from Vertex for cash initially held in escrow but received in February 2019.

We recorded a tax benefit of \$0.3 million during the year ended December 31, 2017. The tax benefit recorded in fiscal year 2017 is the result of the alternative minimum tax ("AMT") paid in fiscal year 2015, which is refundable under the Tax Cuts and Jobs Act of 2017. Income taxes that would otherwise have been due on the 2017 taxable income were offset with the tax benefit of net operating loss carryforwards which had previously had a full valuation allowance, except for \$1.9 million of AMT incurred due to the limitation on use of net operating loss carryforwards when determining AMT. However, the 2017 AMT is also refundable under the Tax Cuts and Jobs Act of 2017 and thus we have not recorded a tax provision for this amount. The



total amount of refundable AMT credits of \$2.3 million is reflected as income tax receivable in the consolidated balance sheet as of December 31, 2018.

We provide a full valuation allowance for any tax benefit related to net operating losses due to the uncertainty of the ability to realize such benefits.

#### Comparison of the years ended December 31, 2017 and 2016

The following table summarizes our results of operations for the years ended December 31, 2017 and 2016, together with the changes in those items in dollars.

| (in thousands)                               | Year ended December 31, |              |            |
|--|-------------------------|--------------|------------|
|  | 2017                    | 2016         | Change     |
| Revenue:                                     |                         |              |            |
| License and research and development revenue | \$ 62                   | \$ 174       | \$(112 )   |
| Other revenue                                | 143,829                 | —            | 143,829    |
| Total revenue                                | 143,891                 | 174          | 143,717    |
| Operating expenses:                          |                         |              |            |
| Research and development                     | 30,223                  | 36,983       | (6,760 )   |
| General and administrative                   | 21,019                  | 14,358       | 6,661      |
| Total operating expenses                     | 51,242                  | 51,341       | (99 )      |
| Income (Loss) from operations                | 92,649                  | (51,167 )    | 143,816    |
| Investment income                            | 1,336                   | 447          | 889        |
| Other income                                 | 3,601                   | —            | 3,601      |
| Interest and other expense                   | (815 )                  | —            | (815 )     |
| Loss on extinguishment of debt               | (1,432 )                | —            | (1,432 )   |
| Income (Loss) before income taxes            | 95,339                  | (50,720 )    | 146,059    |
| (Benefit) Provision for income taxes         | (300 )                  | —            | (300 )     |
| Net income (loss)                            | \$ 95,639               | \$ (50,720 ) | \$ 146,359 |

#### License and Research and Development Revenue

License and research and development revenue was \$62 thousand for the year ended December 31, 2017 as compared to \$174 thousand for the prior year period, a decrease of \$112 thousand. The decrease in license and research and development revenue in the 2017 period was primarily due to a decrease in revenue recognized for services performed under our Celgene and Jazz Pharmaceuticals collaboration agreements of \$58 thousand and \$54 thousand, respectively. These changes were attributable to the completion of clinical conduct under these programs in 2015.

#### Other Revenue

Other revenue recognized during the year ended December 31, 2017 of \$143.8 million was attributable to the closing of the transaction contemplated by the Asset Purchase Agreement with Vertex, discussed in detail in Note 12 to the consolidated financial statements appearing elsewhere in this Annual Report on Form 10-K.

#### Research and development expenses

The following table summarizes our external research and development expenses, by program, for the years ended December 31, 2017 and 2016, with our internal research expenses separately classified by category. Because Avanir is conducting the clinical development of AVP-786 at its expense, we made no investment in the program during these periods.



| (in thousands)                           | Year ended December 31, |           |
|--|-------------------------|-----------|
|  | 2017                    | 2016      |
| CTP-543 external costs                   | \$ 6,299                | \$ 7,603  |
| CTP-656 external costs                   | 3,076                   | 9,592     |
| CTP-730 external costs                   | 19                      | 31        |
| JZP-386 external costs                   | —                       | 19        |
| External costs for other programs        | 1,481                   | 1,732     |
| Employee and contractor-related expenses | 15,685                  | 14,523    |
| Facility and other expenses              | 3,663                   | 3,483     |
| Total research and development expenses  | \$ 30,223               | \$ 36,983 |

Research and development expenses were \$30.2 million for the year ended December 31, 2017, compared to \$37.0 million for the prior year period, a decrease of \$6.8 million. This decrease was primarily due to a decrease of \$6.5 million and \$1.3 million in direct external expenses associated with CTP-656 and CTP-543, respectively. The decrease in CTP-656 expenses in 2017 was attributable to costs incurred related to the Phase 1 clinical testing and Phase 2 manufacturing activities during the year ended December 31, 2016, compared to costs incurred related to the Phase 2 clinical testing through July 2017 when the sale of CTP-656 to Vertex under the Asset Purchase Agreement closed.

The decrease in CTP-543 external expenses was driven by the timing to initiate the Phase 2a clinical trial. The decrease in external costs for other programs of \$0.2 million was due to decreased consulting expenses for outsourced research development. The increase in employee and contractor-related expenses was primarily attributable to increased non-cash stock-based compensation expenses.

#### General and administrative expenses

General and administrative expenses were \$21.0 million for the year ended December 31, 2017, compared to \$14.4 million for the prior year. The increase of \$6.6 million was attributable to a \$4.1 million increase in consulting and professional fees associated with the CTP-656 Asset Purchase Agreement and intellectual property matters related to CTP-543, and a \$2.5 million increase in staffing costs, primarily due to an increase in non-cash stock-based compensation expenses.

#### Investment income

Investment income was \$1.3 million for the year ended December 31, 2017, compared to \$0.4 million for the prior year period. The increase is attributable to an increase in investments which is due to the upfront payment from Vertex as a result of the closing of the transaction contemplated by the Asset Purchase Agreement, discussed in Note 12 to the consolidated financial statements appearing elsewhere in this Annual Report on Form 10-K.

#### Other income

Other income was \$3.6 million during the year ended December 31, 2017 due to the disgorgement of short-swing profits arising from sales of the Company's stock by a 10% stockholder pursuant to Section 16(b) of the Securities and Exchange Act of 1934.

#### Interest and other expense

Interest expense recorded during the year ended December 31, 2017 is attributable to the interest that was due under our loan facility with Hercules and amortization of the loan discount. All our outstanding indebtedness and obligations owed to Hercules were paid in full, and the loan agreement was terminated in September 2017. Additional information regarding the debt facility is available in Note 13 to the consolidated financial statements appearing elsewhere in this Annual Report on Form 10-K.

Loss on extinguishment of debt

As a result of the prepayment of the debt facility with Hercules, we recognized a loss on the extinguishment of debt of \$1.4 million. All our outstanding indebtedness and obligations owed to Hercules were paid in full on September 7, 2017, and the loan agreement was terminated.

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### Provision for income taxes

We recorded a tax benefit of \$0.3 million during the year ended December 31, 2017. The tax benefit recorded in fiscal year 2017 is the result of the alternative minimum tax ("AMT") paid in fiscal year 2015, which is refundable under the Tax Cuts and Jobs Act of 2017. No tax benefit or provision was recorded during the year ended December 31, 2016 due to the net loss generated.

### LIQUIDITY AND CAPITAL RESOURCES

We have incurred cumulative losses and negative cash flows from operations since our inception in April 2006, and as of December 31, 2018, we had an accumulated deficit of \$116.5 million. Although we generated net income in fiscal year 2017 due to the payment from Vertex, we anticipate that we will continue to incur losses for at least the next several years. We expect that our research and development and general and administrative expenses will continue to increase and, as a result, we will need additional capital to fund our operations, which we may raise through a combination of equity offerings, debt financings, additional collaborations and licensing arrangements, and other sources.

We have financed our operations to date primarily through the public offering and private placement of our equity, debt financing and funding from collaborations and patent assignments. During February 2014, we completed our initial public offering, or IPO, whereby we sold 6,649,690 shares of common stock at a price to the public of \$14.00 per share, raising aggregate net proceeds of \$83.1 million. During March 2015, we sold 3,300,000 shares of common stock through an underwritten public offering at a price to the public of \$15.15 per share, raising aggregate net proceeds of \$46.7 million.

In June 2015, we received proceeds of \$50.2 million in connection with the change in control payment from Auspex, relating to Teva Pharmaceutical Industries Ltd.'s acquisition of Auspex, discussed further in Note 12 to the consolidated financial statements.

On July 25, 2017, the Vertex Asset Purchase Agreement, discussed further in Note 12 to the consolidated financial statements appearing elsewhere in this Annual report on Form 10-K, was completed and Vertex paid us \$160 million in cash consideration, with \$16 million initially held in escrow, which was released to us in February 2019.

As of December 31, 2018 we had cash and cash equivalents and investments of \$153.3 million. Cash in excess of immediate requirements is invested in accordance with our investment policy, primarily with a view to liquidity and capital preservation. Currently, our funds are held in U.S. government-backed securities and money market mutual funds consisting of U.S. government-backed securities.

### Cash flows

The following table sets forth the primary sources and uses of cash for each of the periods set forth below:

| (in thousands)   | Year ended December 31, |            |            |
|--|-------------------------|------------|------------|
|  | 2018                    | 2017       | 2016       |
| Net cash provided by (used in):                                |                         |            |            |
| Operating activities   | \$(48,029)              | \$104,084  | \$(45,343) |
| Investing activities   | 37,903                  | (121,307)  | (7,213)    |
| Financing activities   | (169)                   | 5,490      | 601        |
| Net decrease in cash, cash equivalents and restricted stock    | \$(10,295)              | \$(11,733) | \$(51,955) |
| Comparison of the years ended December 31, 2018, 2017 and 2016 |                         |            |            |

Operating activities. The cash provided by or used for operating activities generally approximates our net income (loss) adjusted for non-cash items and changes in operating assets and liabilities. The cash used during the year ended December 31, 2018 was largely driven by Phase 2 clinical studies and other development activities associated with CTP-543 as well as Phase 1 clinical studies and associated costs of CTP-692. The cash provided by operating activities during the year ended December 31, 2017 was primarily the result of the receipt of \$144 million upon the closing of the CTP-656 asset sale to Vertex in July 2017, partially offset by development activities associated with CTP-656, CTP-543, and research. The cash used during the





year ended December 31, 2016 was largely driven by Phase 1 clinical studies and other development activities associated with CTP-656 and CTP-543.

**Investing activities.** Net cash used in investing activities consisted of purchases of investments, purchases of fixed assets, and proceeds from the maturity of investments. Net cash used to purchase investments for the years ended December 31, 2018, 2017 and 2016 was \$99.4 million, \$206.2 million and \$132.3 million, respectively. Net cash provided by maturities of investments for the years ended December 31, 2018, 2017 and 2016 was \$140.2 million, \$85.8 million and \$125.9 million, respectively. Purchases of fixed assets for the years ended December 31, 2018, 2017 and 2016 was \$2.9 million, \$0.9 million and \$0.8 million, respectively. The increase in cash used to purchase fixed assets in fiscal year 2018 was due to the relocation of our headquarters to 65 Hayden Avenue, discussed further in Note 11 to the consolidated financial statements appearing elsewhere in this Annual Report on Form 10-K.

**Financing activities.** During the years ended December 31, 2018, 2017, and 2016, our financing activities used cash of \$0.2 million, and provided cash of \$5.5 million and \$0.6 million, respectively. Cash used during 2018 consisted of \$1.2 million used to pay withholding taxes on behalf of employees in connection with the vesting and settlement of restricted stock units, in exchange for the surrender of shares of common stock by employees, offset by \$1.0 million of proceeds from the exercise of stock options. During the 2017 period, cash provided by financing activities was largely driven by the net proceeds of \$29.7 million under our Loan Agreement with Hercules in June 2017 and proceeds from the exercise of stock options of \$6.6 million, partially offset by the prepayment of our Loan Agreement with Hercules in September 2017. The cash provided by financing activities during the year ended December 31, 2016 was attributable to proceeds from the exercise of stock options of \$0.6 million.

#### Credit Facilities

On June 8, 2017, we entered into a Loan Agreement with Hercules, which provided for up to \$30.0 million in funding, through a single advance. We incurred \$0.3 million in loan issuance costs paid directly to the lenders, which was offset against the loan proceeds as a loan discount. The advance under the Loan and Security Agreement bore interest at a variable rate of the greater of 8.55% and an amount equal to 8.55% plus the prime rate of interest minus 4.50%. Pursuant to the Loan Agreement, we had the option to prepay the principal of the Loan Agreement at any time subject to a prepayment charge; however the prepayment charge was waived upon the completion of the sale of CTP-656 to Vertex, discussed further in Note 12 to the consolidated financial statements appearing elsewhere in this Annual Report on Form 10-K, and the prepayment of the Term Loan Facility after the 90th day following the closing date of the Loan Agreement but prior to the six month anniversary of the closing date of the Loan Agreement.

On September 7, 2017, we paid a total of \$30.8 million to Hercules, representing the principal, accrued and unpaid interest, fees, costs and expenses outstanding under the Loan Agreement. Upon the payment of the \$30.8 million pursuant to a payoff letter between the Company and Hercules, all outstanding indebtedness and obligations of the Company owed to Hercules under the Loan Agreement were paid in full, and the Loan Agreement was terminated. In connection with the entry into the Loan Agreement, we issued warrants (the "Warrants") to certain entities affiliated with Hercules, exercisable for an aggregate of 61,273 shares of the Company's common stock at an exercise price of \$12.24 per share. The Warrants have a five year term, expiring June 8, 2022, and may be exercised on a cashless basis. The Hercules Warrants had a total relative fair value of \$0.5 million upon issuance and were recorded as a debt discount.

#### Operating capital requirements

We do not anticipate commercializing any of our product candidates for several years. Although we generated net income in 2017 and 2015 due to one-time payments from Vertex and Auspex, respectively, we anticipate that we will continue to generate losses for the foreseeable future, and we expect the losses to increase as we continue the development of, and seek regulatory approvals for, our product candidates, and begin to commercialize any approved products for which we retain commercialization rights. We are subject to all of the risks incident in the development of new drug products, and we may encounter unforeseen expenses, difficulties, complications, delays and other unknown factors that may adversely affect our business, as well as additional risks stemming from the unproven

nature of deuterated drugs.

Based on our current expectations, including with respect to our development plans, we believe our existing cash and cash equivalents and investments as of December 31, 2018 will enable us to fund our operating expenses and capital expenditure requirements into the second half of 2020. However, we will require additional capital for the further development of our

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existing product candidates and may also need to raise additional funds sooner to pursue other development activities related to additional product candidates.

To date, we have not generated any revenue from product sales. We do not expect to generate significant revenue from product sales unless and until we, or our collaborators, obtain marketing approval of and commercialize one of our current or future product candidates. Because our product candidates are in various stages of development and the outcome of these efforts is uncertain, we cannot estimate the actual amounts necessary to successfully complete development and commercialization of our product candidates or whether or when we will achieve profitability. We anticipate that we will continue to generate losses for the foreseeable future, and we expect the losses to increase as we continue the development of, and seek marketing approvals for, our product candidates, and begin to commercialize any approved products for which we retain commercialization rights.

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 and additional collaborations, strategic alliances and licensing arrangements, and other arrangements. Except for any obligations of our collaborators to reimburse us for research and development expenses or to make milestone payments under our agreements with them, we do not have any additional committed external sources of funds. Additional capital may not be available on reasonable terms, if at all. If we are unable to raise additional funds 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. If we raise additional funds through the issuance of additional debt or equity securities, it could result in dilution to our existing stockholders, increased fixed payment obligations and the issuance of securities with rights senior to those of our common stock. We may become subject to covenants under any future indebtedness that could limit our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends, which could adversely impact our ability to conduct our business.

Our expectation with respect to the period of time through which our financial resources will be adequate to support our operations is a forward-looking statement and involves risks and uncertainties, and actual results could vary as a result of a number of factors, including those discussed in the “Risk Factors” section of this Annual Report on Form 10-K. We have based this estimate on assumptions that may prove to be wrong, and we could utilize our available capital resources sooner than we currently expect. If we cannot expand our operations or otherwise capitalize on our business opportunities because we lack sufficient capital, our business, financial condition and results of operations could be materially adversely affected.

#### Contractual obligations

The following table summarizes our contractual obligations at December 31, 2018:

| (in thousands)                 | Total    | Less than<br>1 year | 1 to 3<br>years | 3 to 5<br>years | More than<br>5 years |
|--------------------------------|----------|---------------------|-----------------|-----------------|----------------------|
| Operating lease obligations(1) | \$31,348 | \$ 2,776            | \$5,328         | \$6,159         | \$ 17,085            |
| Total contractual obligations  | \$31,348 | \$ 2,776            | \$5,328         | \$6,159         | \$ 17,085            |

Consists of future lease payments under the operating lease for our office and laboratory at 65 Hayden Avenue, (1)Lexington, Massachusetts. The operating lease expires on January 1, 2029, with two optional extension terms of five years each.

We have an obligation to make a payment to GSK of up to \$2.8 million if we receive cash proceeds from the sale of common stock of Processa received in the 2018 licensing transaction, commercialize CTP-499 or if we receive cash proceeds from re-licensing or transferring the rights to our CTP-499 program.

We enter into contracts in the normal course of business with contract research organizations for clinical and nonclinical research studies, manufacturing, research supplies and other services and products for operating purposes.

These contracts generally provide for termination on notice, and therefore are cancelable contracts and not included in the table of contractual obligations and commitments.

#### OFF-BALANCE SHEET ARRANGEMENTS

We did not have during the periods presented, and we do not currently have, any off-balance sheet arrangements, as defined in the rules and regulations of the SEC.

#### ITEM 7A. Quantitative and Qualitative Disclosures About Market Risk

We are exposed to market risk related to changes in interest rates. Our current investment policy is to maintain a diversified investment portfolio in U.S. government-backed securities and money market mutual funds consisting of U.S. government-backed securities. Our cash is deposited in and invested through highly rated financial institutions in North America. As of December 31, 2018 and 2017, we had \$153.3 million and \$203.2 million of cash, cash equivalents and investments, respectively. The fair value of cash equivalents and investments is subject to change as a result of potential changes in market interest rates. Due to the short-term maturities of our cash equivalents and the low risk profile of these investments, an immediate 100 basis point change in interest rates at levels as of December 31, 2018 would not have a material effect on the fair market value of our cash equivalents and short term investments.

We contract with suppliers of raw materials and contract manufacturers internationally. Transactions with these providers are predominantly settled in U.S. dollars and, therefore, we believe that we have only minimal exposure to foreign currency exchange risks. We do not hedge against foreign currency risks.

Inflation generally affects us by increasing our cost of labor and clinical trial costs. We do not believe that inflation had a material effect on our business, financial condition or results of operations during the years ended December 31, 2018, 2017 or 2016.

#### ITEM 8. Financial Statements and Supplementary Data

##### INDEX TO FINANCIAL STATEMENTS

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Report of Independent Registered Public Accounting Firm  
To the Stockholders and the Board of Directors of Concert Pharmaceuticals, Inc.

### Opinion on the Financial Statements

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

### Adoption of New Accounting Standard

As discussed in Note 2 to the consolidated financial statements, the Company changed its method of accounting for revenue in 2018 due to the adoption of Accounting Standards Update (ASU) No. 2014-09, Revenue from Contracts with Customers (Topic 606), and related amendments.

### Basis for Opinion

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

We conducted our audits 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. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. As part of our audits we are required to obtain an understanding of internal control over financial reporting but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no opinion.

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 include 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 2007.  
Boston, Massachusetts  
February 28, 2019



CONCERT PHARMACEUTICALS, INC.  
CONSOLIDATED BALANCE SHEETS

|  | December 31,   |            |
|--|--|------------|
|  | 2018   | 2017       |
|  | (Amounts in thousands, except<br>share and per share data) |            |
| Assets   |  |            |
| Current assets:  |  |            |
| Cash and cash equivalents  | \$ 17,770  | \$ 27,665  |
| Investments, available for sale  | 135,544  | 175,500    |
| Marketable equity securities   | 7,525  | —          |
| Interest receivable  | 556  | 628        |
| Accounts receivable  | 15   | 155        |
| Contract asset (Note 12)   | 16,000   | —          |
| Prepaid expenses and other current assets  | 2,739  | 1,786      |
| Total current assets   | 180,149  | 205,734    |
| Property and equipment, net  | 8,919  | 2,165      |
| Restricted cash  | 1,157  | 1,557      |
| Other assets   | —  | 34         |
| Income taxes receivable  | 2,322  | 2,246      |
| Total assets   | \$ 192,547   | \$ 211,736 |
| Liabilities and stockholders' equity   |  |            |
| Current liabilities:   |  |            |
| Accounts payable   | \$ 1,277   | \$ 658     |
| Accrued expenses and other liabilities   | 5,669  | 4,299      |
| Income taxes payable   | 390  | 46         |
| Deferred revenue, current portion  | 1,413  | 1,442      |
| Total current liabilities  | 8,749  | 6,445      |
| Deferred revenue, net of current portion   | 9,120  | 8,859      |
| Deferred lease incentive, net of current portion   | 4,088  | —          |
| Deferred rent, net of current portion  | 2,850  | —          |
| Total liabilities  | 24,807   | 15,304     |
| Commitments (Note 11)  |  |            |
| Stockholders' equity:  |  |            |
| Preferred stock, \$0.001 par value per share; 5,000,000 shares authorized; no shares issued and outstanding in 2018 and 2017, respectively   | —  | —          |
| Common stock, \$0.001 par value per share; 100,000,000 shares authorized; 23,518,690 and 23,147,779 shares issued and 23,437,587 and 23,140,378 outstanding in 2018 and 2017, respectively | 23   | 23         |
| Additional paid-in capital   | 284,369  | 273,059    |
| Accumulated other comprehensive loss   | (137   | ) (407     |
| Accumulated deficit  | (116,515   | ) (76,243  |
| Total stockholders' equity   | 167,740  | 196,432    |
| Total liabilities and stockholders' equity   | \$ 192,547   | \$ 211,736 |

See accompanying notes.





## CONCERT PHARMACEUTICALS, INC.

## CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE INCOME (LOSS)

|   | Year ended                                    |          |            |
|---|---|----------|------------|
|   | December 31,                                  |          |            |
|   | 2018  | 2017     | 2016       |
|   | (Amounts in thousands, except per share data) |          |            |
| Revenue:  |   |          |            |
| License and research and development revenue  | \$10,505                                      | \$62     | \$174      |
| Other revenue (Note 12)   | —   | 143,829  | —          |
| Total revenue   | 10,505  | 143,891  | 174        |
| Operating expenses:   |   |          |            |
| Research and development  | 43,149  | 30,223   | 36,983     |
| General and administrative  | 22,940  | 21,019   | 14,358     |
| Total operating expenses  | 66,089  | 51,242   | 51,341     |
| (Loss) Income from operations   | (55,584 )                                     | 92,649   | (51,167 )  |
| Investment income   | 2,787   | 1,336    | 447        |
| Other income (Note 14)  | 12  | 3,601    | —          |
| Interest and other expense  | —   | (815 )   | —          |
| Loss on extinguishment of debt (Note 13)  | —   | (1,432 ) | —          |
| Unrealized loss on marketable equity securities (Note 12)   | (2,926 )                                      | —        | —          |
| (Loss) Income before income taxes   | (55,711 )                                     | 95,339   | (50,720 )  |
| Provision (Benefit) for income taxes  | 313   | (300 )   | —          |
| Net (loss) income   | \$(56,024)                                    | \$95,639 | \$(50,720) |
| Other comprehensive income (loss):  |   |          |            |
| Unrealized gain (loss) on investments, net of tax of \$76 in 2018   | 270   | (400 )   | 11         |
| Comprehensive (loss) income   | \$(55,754)                                    | \$95,239 | \$(50,709) |
| Net (loss) income attributable to common stockholders:  |   |          |            |
| Basic   | \$(56,024)                                    | \$95,195 | \$(50,720) |
| Diluted   | \$(56,024)                                    | \$95,210 | \$(50,720) |
| Net (loss) income per share attributable to common stockholders:  |   |          |            |
| Basic   | \$(2.40 )                                     | \$4.20   | \$(2.28 )  |
| Diluted   | \$(2.40 )                                     | \$4.06   | \$(2.28 )  |
| Weighted-average number of common shares used in net (loss) income per share attributable to common stockholders: |   |          |            |
| Basic   | 23,370  | 22,641   | 22,233     |
| Diluted   | 23,370  | 23,442   | 22,233     |

See accompanying notes.

CONCERT PHARMACEUTICALS, INC.  
CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY

|   | Common Stock   |             |        | Additional      | Accumulated                | Accumulated  | Total                |
|---|----------------|-------------|--------|-----------------|----------------------------|--------------|----------------------|
|   | Issued         | In Treasury | Amount | paid-in capital | other comprehensive income | deficit      | stockholders' equity |
|   | (in thousands) |             |        |                 |                            |              |                      |
| Balance at December 31, 2015                                  | 22,167         | 2           | \$ 22  | \$251,793       | \$ (18 )                   | \$(121,162 ) | \$ 130,635           |
| Exercise of stock options                                     | 153            | 1           | —      | 601             | —                          | —            | 601                  |
| Unrealized gain on short-term investments                     | —              | —           | —      | —               | 11                         | —            | 11                   |
| Stock-based compensation expense                              | —              | —           | —      | 5,067           | —                          | —            | 5,067                |
| Net loss  | —              | —           | —      | —               | —                          | (50,720 )    | (50,720 )            |
| Balance at December 31, 2016                                  | 22,320         | 3           | \$ 22  | \$257,461       | \$ (7 )                    | \$(171,882 ) | \$ 85,594            |
| Exercise of stock options                                     | 828            | 5           | 1      | 6,586           | —                          | —            | 6,587                |
| Unrealized loss on short-term investments                     | —              | —           | —      | —               | (400 )                     | —            | (400 )               |
| Stock-based compensation expense                              | —              | —           | —      | 8,500           | —                          | —            | 8,500                |
| Issuance of stock warrants                                    | —              | —           | —      | 512             | —                          | —            | 512                  |
| Net income  | —              | —           | —      | —               | —                          | 95,639       | 95,639               |
| Balance at December 31, 2017                                  | 23,148         | 8           | \$ 23  | \$273,059       | \$ (407 )                  | \$(76,243 )  | \$ 196,432           |
| Exercise of stock options                                     | 197            | 21          | —      | 1,016           | —                          | —            | 1,016                |
| Release of restricted stock units                             | 174            | 52          | —      | (1,206 )        | —                          | —            | (1,206 )             |
| Unrealized gain on short-term investments, net of tax of \$76 | —              | —           | —      | —               | 270                        | —            | 270                  |
| Stock-based compensation expense                              | —              | —           | —      | 11,500          | —                          | —            | 11,500               |
| Adoption of ASC 606 as of January 1, 2018                     | —              | —           | —      | —               | —                          | 15,752       | 15,752               |
| Net loss  | —              | —           | —      | —               | —                          | (56,024 )    | (56,024 )            |
| Balance at December 31, 2018                                  | 23,519         | 81          | \$ 23  | \$284,369       | \$ (137 )                  | \$(116,515 ) | \$ 167,740           |

See accompanying notes.

CONCERT PHARMACEUTICALS, INC.  
CONSOLIDATED STATEMENTS OF CASH FLOWS

|  | Year ended     |             |            |
|--|----------------|-------------|------------|
|  | December 31,   |             |            |
|  | 2018           | 2017        | 2016       |
|  | (in thousands) |             |            |
| Operating activities   |                |             |            |
| Net (loss) income  | \$(56,024)     | \$95,639    | \$(50,720) |
| Adjustments to reconcile net (loss) income to net cash (used in) provided by operating activities: |                |             |            |
| Depreciation and amortization  | 1,247          | 1,008       | 893        |
| Stock-based compensation expense   | 11,500         | 8,500       | 5,067      |
| Accretion of premiums and discounts on investments   | (469)          | ) 90        | 504        |
| Amortization of discount on loan payable   | —              | 166         | —          |
| Amortization of deferred lease incentive   | (703)          | ) (324)     | ) (315)    |
| Noncash license consideration (Note 12)  | (10,451)       | ) —         | —          |
| Other noncash items  | (77)           | ) —         | —          |
| Unrealized loss on marketable equity securities (Note 12)  | 2,926          | —           | —          |
| Loss on disposal of asset  | 52             | 46          | 2          |
| Loss on extinguishment of debt   | —              | 1,432       | —          |
| Changes in operating assets and liabilities:   |                |             |            |
| Accounts receivable  | 119            | (107)       | ) 43       |
| Interest receivable  | 72             | (464)       | ) 17       |
| Prepaid expenses and other current assets  | (953)          | ) (433)     | ) 314      |
| Other assets   | 34             | 33          | 11         |
| Accounts payable   | 435            | 113         | 44         |
| Accrued expenses and other liabilities   | 1,265          | 436         | (957)      |
| Income taxes receivable  | (76)           | ) (2,246)   | ) —        |
| Income taxes payable   | 344            | 46          | (75)       |
| Deferred rent  | 2,746          | (102)       | ) (51)     |
| Deferred revenue   | (16)           | ) 251       | (120)      |
| Net cash (used in) provided by operating activities  | (48,029)       | ) 104,084   | (45,343)   |
| Investing activities   |                |             |            |
| Purchases of property and equipment  | (2,869)        | ) (947)     | ) (770)    |
| Purchases of investments   | (99,435)       | ) (206,207) | (132,344)  |
| Maturities of investments  | 140,207        | 85,847      | 125,901    |
| Net cash provided by (used in) investing activities  | 37,903         | (121,307)   | (7,213)    |
| Financing activities   |                |             |            |
| Proceeds from loan, net  | —              | 29,659      | —          |
| Repayment of loan  | —              | (30,745)    | —          |
| Repurchase of common stock pursuant to share surrender   | (1,206)        | ) —         | —          |
| Proceeds from exercise of stock options  | 1,037          | 6,576       | 601        |
| Net cash (used in) provided by financing activities  | (169)          | ) 5,490     | 601        |
| Net decrease in cash, cash equivalents and restricted stock  | (10,295)       | ) (11,733)  | ) (51,955) |
| Cash, cash equivalents and restricted stock at beginning of period                                 | 29,222         | 40,955      | 92,910     |
| Cash, cash equivalents and restricted stock at end of period                                       | \$18,927       | \$29,222    | \$40,955   |
| Supplemental cash flow information:  |                |             |            |
| Cash paid for income taxes   | \$123          | \$1,900     | \$75       |
| Cash paid for interest   | \$—            | \$648       | \$—        |

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|  |         |       |      |
|--|---------|-------|------|
| Purchases of property and equipment unpaid at period end | \$188   | \$65  | \$20 |
| Tenant Improvements paid by landlord (Note 11)           | \$4,996 | \$—   | \$—  |
| Issuance of stock warrants                               | \$—     | \$512 | \$—  |

See accompanying notes.

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CONCERT PHARMACEUTICALS, INC.  
NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

1. Nature of Business

Concert Pharmaceuticals, Inc., or Concert or the Company, was incorporated on April 12, 2006 as a Delaware corporation with operations based in Lexington, Massachusetts. The Company is a clinical stage biopharmaceutical company that applies its extensive knowledge of deuterium chemistry to discover and develop novel small molecule drugs. The Company's approach starts with previously studied compounds, including approved drugs, that the Company believes can be improved with deuterium substitution to provide better pharmacokinetic or metabolic properties, enhancing clinical safety, tolerability or efficacy. The Company believes this approach may enable drug discovery and clinical development that is more efficient and less expensive than conventional small molecule drug research and development. The Company's pipeline includes multiple clinical-stage candidates and a number of preclinical compounds that it is currently assessing.

In March 2015, the Company sold 3,300,000 shares of common stock in a public offering at a price to the public of \$15.15 per share, resulting in net proceeds to the Company of approximately \$46.7 million after deducting underwriting discounts and commissions and offering expenses. In June 2015, the Company received a one-time payment of \$50.2 million from Auspex Pharmaceuticals, Inc., or Auspex, pursuant to a patent assignment agreement between Concert and Auspex. Concert became eligible to receive the payment due to a change of control of Auspex, which was acquired by Teva Pharmaceutical Industries Ltd. in May 2015 (see Note 12).

On March 3, 2017, the Company entered into an Asset Purchase Agreement (the "Asset Purchase Agreement") with Vertex Pharmaceuticals, Inc., through Vertex Pharmaceuticals (Europe) Limited ("Vertex"), pursuant to which the Company agreed to sell and assign CTP-656, now known as VX-561, and other cystic fibrosis assets of the Company, for up to \$250 million subject to the satisfaction of certain closing conditions. On July 25, 2017, the transaction contemplated by the Asset Purchase Agreement closed and Vertex paid the Company \$160 million in cash consideration, with \$16 million to be held in escrow. Additional information concerning the sale of CTP-656 is discussed further in Note 12.

The Company had cash and cash equivalents and investments of \$153.3 million at December 31, 2018. The Company believes that its cash and cash equivalents and investments at December 31, 2018 will be sufficient to allow the Company to fund its current operating plan for at least the next twelve months from the date of issuance of the financial statements. The Company may pursue additional cash resources through public or private financings and by establishing collaborations with or licensing its technology to other companies and through other arrangements. Since its inception, the Company has generated an accumulated deficit of \$116.5 million through December 31, 2018. The Company's operating results may fluctuate significantly from year to year, depending on the timing and magnitude of clinical trial and other development activities under its current development programs. Substantially all the Company's net losses have resulted from costs incurred in connection with its research and development programs and from general and administrative costs associated with its operations. The Company expects to continue to incur significant expenses and increasing operating losses for at least the next several years.

The Company is subject to risks common to companies in the biotechnology industry, including, but not limited to, risks of failure or unsatisfactory results of nonclinical studies and clinical trials, the need to obtain additional financing to fund the future development of its pipeline, the need to obtain marketing approval for its product candidates, the need to successfully commercialize and gain market acceptance of its product candidates, dependence on key personnel, protection of proprietary technology, compliance with government regulations, development by competitors of technological innovations and ability to transition from pilot-scale manufacturing to large-scale production of products.

Unless otherwise indicated, all amounts are in thousands except share and per share amounts.

2. Basis of Presentation and Significant Accounting Policies

Basis of Presentation

The consolidated financial statements have been prepared in accordance with accounting principles generally accepted in the United States of America, or GAAP. Management has determined that the Company operates in one segment: the development of pharmaceutical products on its own behalf or in collaboration with others. All long-lived assets of the Company reside in the United States.

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CONCERT PHARMACEUTICALS, INC.  
NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

The accompanying consolidated financial statements include the accounts of Concert Pharmaceuticals, Inc. and its wholly owned subsidiaries. All intercompany transactions and balances have been eliminated.

The Company considers events or transactions that occur after the balance sheet date but before the financial statements are issued to provide additional evidence relative to certain estimates or to identify matters that require additional disclosure.

#### Estimates and Uncertainties

The preparation of the consolidated financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets, liabilities, equity, revenue, expenses and the disclosure of contingent assets and liabilities and the Company's ability to continue as a going concern. In preparing the consolidated financial statements, management used estimates in the following areas, among others: revenue recognition; income tax expense; stock-based compensation expense; accrued expenses; and the evaluation of the existence of conditions and events that raise substantial doubt regarding the Company's ability to continue as a going concern. Actual results could differ from those estimates.

#### Cash, Cash Equivalents and Investments

Cash equivalents include all highly liquid investments maturing within 90 days from the date of purchase. Investments consist of securities with original maturities greater than 90 days when purchased. The Company classifies these investments as available-for-sale and records them at fair value in the accompanying consolidated balance sheets. Unrealized gains or losses are included in accumulated other comprehensive income (loss). Premiums or discounts from par value are amortized to investment income over the life of the underlying investment.

Although available to be sold to meet operating needs or otherwise, securities are generally held through maturity. The Company classifies all investments as current assets as these assets are readily available for use in current operations. The cost of securities sold is determined based on the specific identification method for purposes of recording realized gains and losses. During 2018 and 2017, there were no realized gains or losses on sales of investments, and no investments were adjusted for other than temporary declines in fair value.

The Company reviews available-for-sale securities for other-than-temporary impairment whenever the fair value of an available-for-sale security is less than the amortized cost and evidence indicates that an available-for-sale security's carrying amount is not recoverable within a reasonable period of time. Other-than-temporary impairments of investments are recognized in the consolidated statements of operations if the Company has experienced a credit loss, has the intent to sell the security, or if it is more likely than not that the Company will be required to sell the security before recovery of the amortized cost basis. Evidence considered in this assessment includes reasons for the impairment, compliance with the Company's investment policy, the severity and the duration of the impairment and changes in value subsequent to the end of the period.

#### Marketable Equity Securities

Marketable equity securities consist of the fair value of common shares of Processa held by the Company, as discussed further in Note 12. As discussed below in this Note 2, effective January 1, 2018 the Company prospectively adopted ASU 2016-01, resulting in the recognition of the effects of changes in fair value of equity securities within net income.

The Company reviews investments in marketable securities for other-than-temporary impairment whenever the fair value of the investment is less than the cost and evidence indicates that an investment's carrying amount is not recoverable within a reasonable period of time. To determine whether an impairment is other-than-temporary, the



Company considers whether it has an intent to sell, or whether it is more likely than not that the Company will be required to sell, the investment before recovery of the investment's cost basis. Evidence considered in this assessment includes reasons for the impairment, the severity and the duration of the impairment and changes in value subsequent to year-end.

#### Fair Value of Financial Measurements

The Company has certain financial assets and liabilities that are recorded at fair value which have been classified as Level 1, 2 or 3 within the fair value hierarchy as described in the accounting standards for fair value measurements:

Level 1—quoted prices for identical instruments in active markets;

CONCERT PHARMACEUTICALS, INC.  
NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

Level 2—quoted prices for similar instruments in active markets, quoted prices for identical or similar instruments in markets that are not active, and model-derived valuations in which all significant inputs and significant value drivers are observable in active markets; and

Level 3—valuations derived from valuation techniques in which one or more significant value drivers are unobservable.

For additional information related to fair value measurements, please read Note 3 to the consolidated financial statements.

#### Concentrations of Credit Risk

Financial instruments that potentially subject the Company to concentration of credit risk consist principally of money market funds, investments (including interest receivable), and accounts receivable. Our current investment policy is to maintain a diversified investment portfolio in U.S. government-backed securities and money market mutual funds consisting of U.S. government-backed securities. Our cash is deposited in and invested through highly rated financial institutions in North America.

The Company has not experienced any credit losses in these accounts and does not believe it is exposed to any significant credit risk on these funds. The Company has no foreign exchange contracts, option contracts or other foreign exchange hedging arrangements.

At December 31, 2018 and 2017, substantially all of the Company's cash was deposited in accounts at two financial institutions, thus limiting the amount of credit exposure to any one financial institution. These amounts at times may exceed federally insured limits.

Accounts receivable generally represent amounts due from collaboration partners. The Company monitors economic conditions to identify facts or circumstances that may indicate that any of its accounts receivable are at risk of collection.

#### Property and Equipment

Property and equipment are recognized at cost and depreciated over their estimated useful lives using the straight-line method. Leasehold improvements are amortized over the shorter of their useful life or the related lease term. Repair and maintenance costs are expensed as incurred, whereas major improvements are capitalized as additions to property and equipment. Potential impairment is assessed when there is evidence that events or circumstances indicate that the carrying amount of an asset may not be recovered. No such impairment losses have been recorded through December 31, 2018.

#### Rent Expense

Rent expense for the year ended December 31, 2018 consists of the Company's previous facility at 99 Hayden Avenue, Lexington, Massachusetts, and the Company's new facility at 65 Hayden Avenue, Lexington, Massachusetts. The Company ceased recognizing rent expense for 99 Hayden Avenue when the lease expired on September 30, 2018. The Company began recognizing rent expense for 65 Hayden Avenue on January 1, 2018 when it obtained access to the premises.

The Company's operating lease for its new facility at 65 Hayden Avenue in Lexington, Massachusetts provides for scheduled annual rent increases throughout the lease term. The Company recognizes the effects of the scheduled rent increases on a straight-line basis over the full term of the lease, which expires on January 1, 2029. Additionally, the Company has received certain lease incentives in connection with its existing Lexington, Massachusetts facility lease, which are recognized as a reduction to rent expense over the remaining lease term. Refer to Note 11 for additional details regarding the Company's operating leases.

Rent expense for the years ended December 31, 2018, 2017, and 2016 was \$3.3 million, \$1.1 million, and \$1.2 million, respectively.

#### Contingencies

The Company records liabilities for legal and other contingencies when information available to the Company indicates that it is probable that a liability has been incurred and the amount of loss can be reasonably estimated. Legal costs in connection with legal and other contingencies are expensed as costs are incurred. No liabilities for legal and

other contingencies were accrued as of December 31, 2018 and 2017.

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CONCERT PHARMACEUTICALS, INC.  
NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

### Revenue Recognition

The Company has generated revenue through arrangements with collaborators and nonprofit organizations for the development and commercialization of product candidates, a patent assignment agreement and an asset sale. On January 1, 2018, the Company adopted Financial Accounting Standards Board (FASB) Accounting Standards Update (ASU) 2014-09, Revenue from Contracts with Customers and all related amendments ("ASC 606" or "the new revenue standard"). ASC 606 is a single comprehensive model for entities to use in accounting for revenue arising from contracts with customers and supersedes most legacy revenue recognition guidance, including industry-specific guidance. The new revenue standard is based on the principle 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. To achieve this core principle, ASC 606 provides that an entity should apply the following steps: (1) identify the contract(s) with a customer, (2) identify the performance obligations in the contract, (3) determine the transaction price, (4) allocate the transaction price to the performance obligations in the contract and (5) recognize revenue when (or as) the entity satisfies a performance obligation. The new revenue standard also requires additional disclosure about the nature, amount, timing and uncertainty of revenue and cash flows arising from customer contracts and costs to obtain or fulfill contracts. The Company applied ASC 606 on January 1, 2018 to all contracts using the modified retrospective approach. As a result of the adoption, the cumulative effect to retained earnings at January 1, 2018 was \$15.8 million. The comparative information has not been restated and continues to be reported under the accounting standards in effect for those periods.

The adoption of the new standard had an immaterial impact on the Company's reported revenues, operating income and changes in operating cash flows for the twelve months ended December 31, 2018 as compared to what reported amounts would have been under legacy guidance. The cumulative effect of the changes made to the consolidated January 1, 2018 balance sheet for the adoption of the new revenue standard was as follows:

| (Amounts in thousands)                   | Balance at<br>December<br>31, 2017 | ASC 606<br>Adjustments | Opening<br>Balance<br>at<br>January<br>1, 2018 |
|--|------------------------------------|------------------------|--|
| <b>Assets</b>                            |                                    |                        |  |
| Contract Asset                           | \$ —                               | \$ 16,000              | \$ 16,000                                      |
| <b>Liabilities and Equity</b>            |                                    |                        |  |
| Deferred revenue, current portion        | \$ 1,442                           | \$ (14 )               | \$ 1,428                                       |
| Deferred revenue, net of current portion | 8,859                              | 261                    | 9,120  |
| Retained earnings                        | 76,243                             | 15,753                 | 60,490   |

The impact of adopting the new revenue standard primarily relates to the treatment of the consideration held in escrow under the Vertex Asset Purchase Agreement. Under previous authoritative guidance, the Company concluded that it would not recognize the Vertex escrow consideration until it was received. However, under ASC 606, the Vertex escrow consideration represents variable consideration and was included in the transaction price at contract inception, discussed further in Note 12. There was no material effect on the accounting for income taxes resulting from the adoption of ASC 606.

### Research and Development Costs

Research and development costs are expensed as incurred.

Research and development expenses are comprised of costs incurred in providing research and development activities, including salaries and benefits, facilities costs, overhead costs, contract research and development services, and other outside costs. Nonrefundable advance payments for goods and services that will be used in future research and

development activities are expensed when the activity has been performed or when the goods have been received rather than when the payment is made.

External research and development expenses associated with the Company's programs include clinical trial site costs, research compounds and clinical manufacturing costs, costs incurred for consultants and other outside services, such as data management and statistical analysis support, and materials and supplies used in support of the clinical and nonclinical programs. Internal costs of the Company's clinical program include salaries, benefits, stock based compensation, and an allocation of the Company's facility costs. When third-party service providers' billing terms do not coincide with the

CONCERT PHARMACEUTICALS, INC.  
NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

Company's period-end, the Company is required to make estimates of its obligations to those third parties, including clinical trial and pharmaceutical development costs, contractual services costs and costs for supply of its drug candidates, incurred in a given accounting period and record accruals at the end of the period. The Company bases its estimates on its knowledge of the research and development programs, services performed for the period, past history for related activities and the expected duration of the third-party service contract, where applicable.

#### Accounting for Stock-Based Compensation

The Company issues stock options and restricted-stock-units, or RSUs, to certain employees, officers and directors. The Company accounts for stock compensation using the fair value method, which results in the recognition of compensation expense over the vesting period of the awards. See Note 8 for additional information.

#### Income Taxes

The Company provides deferred tax assets and liabilities for the expected future tax consequences of temporary differences between the Company's financial statement carrying amounts and the tax basis of assets and liabilities using enacted tax rates expected to be in effect in the years in which the differences are expected to reverse. A valuation allowance is provided to reduce the deferred tax assets to the amount that will more likely than not be realized.

The Company evaluates tax positions taken, or expected to be taken, in the course of preparing its tax returns to determine whether the tax positions are "more likely than not" of being sustained by the applicable tax authority. Tax positions not deemed to meet the more-likely-than-not threshold would be recognized as a tax expense.

On December 22, 2017, the President of the United States signed into law the Tax Cuts and Jobs Act ("TCJA"). This legislation made broad and complex changes to the U.S. tax code, including, but not limited to, (i) reducing the U.S. federal statutory tax rate from 35% to 21%; (ii) eliminating the corporate alternative minimum tax (AMT) and changing how existing AMT credits can be realized; (iii) changing rules related to uses and limitations of net operating loss carryforwards created in tax years beginning after December 31, 2017, and (iv) modifying the officer's compensation limitation. The Company recognized the effects of changes in tax law, including the TCJA, in the period the law is enacted. Accordingly, the effects of the TCJA were recognized in the financial statements for the year ended December 31, 2017.

For additional details regarding the accounting for income taxes, see Note 10.

#### Guarantees

As permitted under Delaware law, the Company indemnifies its officers and directors for certain events or occurrences while the officer or director is, or was, serving at the Company's request in such capacity. The term of the indemnification is for the officer's or director's lifetime. The maximum potential amount of future payments the Company could be required to make is unlimited; however, the Company has directors' and officers' insurance coverage that limits its exposure and enables it to recover a portion of any future amounts paid.

The Company leases office space under non-cancelable operating leases which are further described in Note 11. The Company has standard indemnification arrangements under the leases that requires it to indemnify the landlords against all costs, expenses, fines, suits, claims, demands, liabilities, and actions directly resulting from any breach, violation, or non-performance of any covenant or condition of the Company's leases.

Pursuant to the Asset Purchase Agreement, discussed further in Note 12, the Company agreed to indemnify Vertex for certain matters, including breaches of specified representations and warranties, covenants included in the Asset Purchase Agreement and specified tax claims. Representations and warranties, other than certain fundamental representations and warranties, survived for a period of eighteen months following the Closing and the maximum liability of the Company for claims by Vertex related to the breaches of such representations and warranties, with limited exceptions, was limited to the escrow amount, or \$16 million. In January 2019, the escrow period expired and the escrow of \$16.0 million was released to the Company in February 2019.

As of December 31, 2018 and 2017, the Company had not experienced any material losses related to these indemnification obligations, and no material claims with respect thereto were outstanding. The Company does not expect significant claims related to these indemnification obligations and, consequently, concluded that the fair value of these obligations is negligible, and no related reserves were established.

CONCERT PHARMACEUTICALS, INC.  
NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

Other Income

In the fiscal year ended December 31, 2017, the Company received \$3.6 million due to a disgorgement of short-swing profits arising from sales of the Company's stock by a 10% stockholder pursuant to Section 16(b) of the Securities and Exchange Act of 1934. The Company has classified the proceeds from the disgorgement as other income in the accompanying consolidated financial statements in fiscal year 2017.

Comprehensive (Loss) Income

Comprehensive (loss) income is comprised of net (loss) income and other comprehensive income or loss. Other comprehensive income or loss consists of unrealized gains and losses on investments.

Recently Adopted Accounting Pronouncements

On January 1, 2018, the Company adopted ASU 2014-09, Revenue from Contracts with Customers and all related amendments. For further discussion, see the Revenue Recognition discussion appearing elsewhere in this Note 2.

In November 2016, the FASB issued ASU 2016-18, Statement of Cash Flows - Restricted Cash (Topic 230). This standard requires companies to include amounts generally described as restricted cash and restricted cash equivalents in cash and cash equivalents when reconciling beginning-of-period and end-of-period total amounts shown on the statement of cash flows. The Company adopted this standard on January 1, 2018. The adoption of ASU 2016-18 resulted in the Company's cash, cash equivalents and restricted cash being included in the beginning and ending amounts for the periods shown on the statement of cash flows and was applied retroactively and reflected in the balances presented for any prior periods. The Company believes that the adoption of this guidance did not have a significant impact on its consolidated financial statements and related disclosures.

Restricted cash as of December 31, 2018 and 2017 is held as collateral for stand-by letters of credit issued by the Company to its landlords in connection with the leases of both the Company's current facility at 65 Hayden Avenue, Lexington, Massachusetts, as well as its previous facility at 99 Hayden Avenue, Lexington, Massachusetts.

Cash, cash equivalents and restricted cash consisted of the following:

|                           | December<br>31,<br>2018 | December<br>31,<br>2017 |
|---------------------------|-------------------------|-------------------------|
| Cash and cash equivalents | \$ 17,770               | \$ 27,665               |
| Restricted cash           | 1,157                   | 1,557                   |
|                           | \$ 18,927               | \$ 29,222               |

In January 2016, the FASB issued ASU 2016-01, Recognition and Measurement of Financial Assets and Financial Liabilities, which revises the classification and measurement of investments in equity securities. ASU 2016-01 generally requires that equity investments, except for those accounted for under the equity method of accounting, be measured at fair value and changes in fair value are recognized in net income. Unrealized gains or losses from other investments, including debt securities, continue to be included in accumulated other comprehensive income (loss). Effective January 1, 2018, the Company prospectively adopted this new standard resulting in the recognition of the effects of changes in fair value of equity securities within net income (loss) in the consolidated statement of operations and comprehensive loss. Refer to Note 12 for discussion of the Company's marketable equity securities holdings.

Pending Accounting Pronouncements

In February 2016, the FASB issued ASU 2016-02, Leases (Topic 842). ASU 2016-02 requires lessees to recognize assets and liabilities on the balance sheet for the rights and obligations created by all leases with terms of more than 12 months. ASU 2016-02 will also require certain qualitative and quantitative disclosures designed to give financial



statement users information on the amount, timing, and uncertainty of cash flows arising from leases. ASU 2016-02 will be effective for the Company on January 1, 2019. The Company plans to adopt the new standard with a cumulative-effect adjustment to the opening balance of retained earnings at that date and no restatement of comparative periods' financial information, as recently allowed by the FASB. The Company is currently evaluating the effect of adopting the requirements of ASU 2016-02 as it relates to the

CONCERT PHARMACEUTICALS, INC.  
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accounting for its facility lease at 65 Hayden Avenue, Lexington, Massachusetts, which was executed in December 2017 and is discussed further in Note 11. The Company has performed a preliminary evaluation of other material contracts to determine if any contain embedded leases, and has not identified any to date. The Company's lease for its 99 Hayden Avenue, Lexington, Massachusetts facility expired in September 2018 and as a result will not be evaluated under the scope of ASU 2016-02. The Company expects to elect certain relief options offered in ASU 2016-02 including the package of practical expedients, the option not to separate lease and non-lease components and instead to account for them as a single lease component, and the option not to recognize right-of-use assets and lease liabilities that arise from short-term leases (i.e., leases with terms of twelve months or less).

In June 2016, the FASB issued ASU No. 2016-13, Financial Instruments-Credit Losses (Topic 326)-Measurement of Credit Losses on Financial Instruments, or ASU 2016-13. The new standard requires entities to measure all expected credit losses for financial assets held at the reporting date based on historical experience, current conditions and reasonable and supportable forecasts. ASU 2016-13 will become effective for the Company for fiscal years beginning after December 15, 2019, with early adoption permitted. The Company is currently evaluating the impact ASU 2016-13 will have on its financial statements and related disclosures.

In June 2018, the FASB issued ASU 2018-07 (Topic 718) - Improvements to Nonemployee Share-Based Payment Accounting that expands the scope of Topic 718 to include share-based payment transactions for acquiring goods and services from nonemployees. ASU 2018-07 provides that an entity should apply the requirements of Topic 718 to nonemployee awards except for certain exemptions specified in the amendment. The guidance is effective for fiscal years beginning after December 15, 2018, including interim reporting periods within that fiscal year. Early adoption is permitted, but no earlier than an entity's adoption date of ASC 606. The Company believes that this guidance will not have a significant impact on its consolidated financial statements and related disclosures.

### 3. Fair Value Measurements

The tables below present information about the Company's financial assets and liabilities that are measured and carried at fair value as of December 31, 2018 and 2017 (in thousands) and indicate the level within the fair value hierarchy where each measurement is classified.

|                                       | Level 1   | Level 2  | Level 3 | Total      |
|---------------------------------------|-----------|----------|---------|------------|
| December 31, 2018                     |           |          |         |            |
| Cash equivalents:                     |           |          |         |            |
| Money market funds                    | \$7,643   | \$—      | \$      | —\$7,643   |
| U.S. Treasury obligations             | 1,748     | —        | —       | 1,748      |
| Investments, available for sale:      |           |          |         |            |
| U.S. Treasury obligations             | 34,103    | 746      | —       | 34,849     |
| Government agency securities          | 64,733    | 35,962   | —       | 100,695    |
| Marketable equity securities:         |           |          |         |            |
| Corporate equity securities (Note 12) | 7,525     | —        | —       | 7,525      |
| Total                                 | \$115,752 | \$36,708 | \$      | —\$152,460 |

|                                  | Level 1 | Level 2 | Level 3 | Total    |
|----------------------------------|---------|---------|---------|----------|
| December 31, 2017                |         |         |         |          |
| Cash equivalents:                |         |         |         |          |
| Money market funds               | \$8,108 | \$—     | \$      | —\$8,108 |
| Investments, available for sale: |         |         |         |          |
| U.S. Treasury obligations        | 53,910  | —       | —       | 53,910   |

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|                              |            |           |    |             |
|------------------------------|------------|-----------|----|-------------|
| Government agency securities | 88,651     | 32,939    | —  | 121,590     |
| Total                        | \$ 150,669 | \$ 32,939 | \$ | —\$ 183,608 |

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NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

## 4. Cash, Cash Equivalents, Investments and Marketable Equity Securities

Cash, cash equivalents, available for sale investments, and marketable equity securities included the following at December 31, 2018 and 2017:

|                                 | Average maturity | Amortized cost | Unrealized gains | Unrealized losses | Fair value |
|---------------------------------|------------------|----------------|------------------|-------------------|------------|
| December 31, 2018               |                  |                |                  |                   |            |
| Cash                            |                  | \$ 8,379       | \$ —             | \$ —              | \$ 8,379   |
| Money market funds              |                  | 7,643          | —                | —                 | 7,643      |
| U.S. Treasury obligations       | 31 days          | 1,748          | —                | —                 | 1,748      |
| Cash and cash equivalents       |                  | \$ 17,770      | \$ —             | \$ —              | \$ 17,770  |
| U.S. Treasury obligations       | 151 days         | \$ 34,856      | \$ 2             | \$ (9 )           | \$ 34,849  |
| Government agency securities    | 153 days         | 100,748        | 7                | (60 )             | 100,695    |
| Investments, available for sale |                  | \$ 135,604     | \$ 9             | \$ (69 )          | \$ 135,544 |

|  | Acquisition value | Unrealized gains | Unrealized losses | Fair value |
|--|-------------------|------------------|-------------------|------------|
| December 31, 2018                      |                   |                  |                   |            |
| Marketable equity securities (Note 12) | \$ 10,451         | \$ —             | \$ (2,926 )       | \$ 7,525   |

|                                 | Average maturity | Amortized cost | Unrealized gains | Unrealized losses | Fair value |
|---------------------------------|------------------|----------------|------------------|-------------------|------------|
| December 31, 2017               |                  |                |                  |                   |            |
| Cash                            |                  | \$ 19,557      | \$ —             | \$ —              | \$ 19,557  |
| Money market funds              |                  | 8,108          | —                | —                 | 8,108      |
| Cash and cash equivalents       |                  | \$ 27,665      | \$ —             | \$ —              | \$ 27,665  |
| U.S. Treasury obligations       | 184 days         | \$ 54,004      | \$ —             | \$ (94 )          | \$ 53,910  |
| Government agency securities    | 229 days         | 121,903        | —                | (313 )            | 121,590    |
| Investments, available for sale |                  | \$ 175,907     | \$ —             | \$ (407 )         | \$ 175,500 |

## 5. Restricted Cash

At December 31, 2018 and 2017, restricted cash was \$1.2 million and \$1.6 million, respectively. Restricted cash as of December 31, 2018 and 2017 is held as collateral for stand-by letters of credit issued by the Company to its landlords in connection with the leases of both the Company's current facility at 65 Hayden Avenue, Lexington, Massachusetts, as well as its previous facility at 99 Hayden Avenue, Lexington, Massachusetts. For additional information regarding the Company's leases, please reference Note 11.

|  | December 31, 2018 | December 31, 2017 |
|--|-------------------|-------------------|
| Letter of Credit: 99 Hayden Avenue, Lexington, Massachusetts | \$ —              | \$ 400            |
| Letter of Credit: 65 Hayden Avenue, Lexington, Massachusetts | 1,157             | 1,157             |
|  | \$ 1,157          | \$ 1,557          |



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### 6. Property and Equipment

Property and equipment consists of the following at December 31, 2018 and 2017 (in thousands):

|   | Estimated useful life<br>(in years)              | December 31, December 31, |          |
|---|--|---------------------------|----------|
|   |  | 2018                      | 2017     |
| Laboratory equipment                              | 5  | \$ 3,484                  | \$ 2,674 |
| Computer, telephone and office equipment          | 3  | 875                       | 147      |
| Software  | 3  | 206                       | 160      |
| Leasehold improvements                            | Lesser of useful life<br>or remaining lease term | 5,866                     | 6,551    |
|   |  | 10,431                    | 9,532    |
| Less accumulated depreciation and<br>amortization |  | (1,512 )                  | (7,367 ) |
|   |  | \$ 8,919                  | \$ 2,165 |

Depreciation and amortization expense was charged to operations in the amounts of \$1.2 million, \$1.0 million, and \$0.9 million for the years ended December 31, 2018, 2017, and 2016, respectively.

### 7. Accrued Expenses and Other Liabilities

Accrued expenses and other liabilities consist of the following (in thousands):

|   | December 31, December 31, |          |
|---|---------------------------|----------|
|   | 2018                      | 2017     |
| Accrued professional fees and other       | \$ 672                    | \$ 628   |
| Employee compensation and benefits        | 3,067                     | 2,797    |
| Research and development expenses         | 1,476                     | 521      |
| Deferred lease incentive, current portion | 454                       | 249      |
| Deferred rent, current portion            | —                         | 104      |
|   | \$ 5,669                  | \$ 4,299 |

### 8. Stock Compensation

#### Stock incentive plans

The Company previously sponsored an Amended and Restated 2006 Stock Option and Grant Plan, or the 2006 Plan, which provided for the issuance of shares of common stock in the form of incentive stock options, nonstatutory stock options, awards of stock and direct stock purchase opportunities to directors, officers, employees and consultants of the Company. The 2006 Plan was replaced by the Company's 2014 Stock Incentive Plan, or the 2014 Plan, which became effective in February 2014. The 2014 Plan provides for the grant of incentive stock options, nonstatutory stock options, restricted stock awards, restricted stock units, stock appreciation rights and other stock-based awards. In addition, the 2014 Plan includes an "evergreen provision" that allows for an annual increase in the number of shares of common stock available for issuance under the 2014 Plan. Effective January 1, 2019, 937,503 shares were added to the 2014 Plan for future issuance pursuant to this evergreen provision.

The 2006 Plan has no shares remaining available for grant, although existing stock options granted under the 2006 Plan remain outstanding. As of December 31, 2018, 1,488,976 shares were available for future grant under the 2014 Plan.

#### Stock options

Stock options are granted with an exercise price equal to the closing market price of the Company's common stock on the date of grant. Stock options generally vest ratably over three or four years and have contractual terms of ten years. Stock options are valued using the Black-Scholes-Merton option valuation model and compensation cost is recognized

based on such fair value over the period of vesting.

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The following table provides certain information related to the Company's outstanding stock options:

|   | Year ended<br>December 31,               |         |         |
|---|--|---------|---------|
|   | 2018                                     | 2017    | 2016    |
|   | (in thousands, except<br>per share data) |         |         |
| Weighted average fair value of options granted, per option        | \$17.74                                  | \$7.67  | \$10.42 |
| Aggregate grant date fair value of options vested during the year | \$7,755                                  | \$6,212 | \$4,614 |
| Total cash received from exercises of stock options               | \$1,037                                  | \$6,576 | \$601   |
| Total intrinsic value of stock options exercised                  | \$2,599                                  | \$8,692 | \$1,160 |

The weighted average fair value of options granted in the years ended December 31, 2018, 2017 and 2016, reflect the following weighted-average assumptions:

|                         | Year ended December 31, |   |           |   |           |   |
|-------------------------|-------------------------|---|-----------|---|-----------|---|
|                         | 2018                    |   | 2017      |   | 2016      |   |
| Expected volatility     | 77.22                   | % | 78.15     | % | 78.29     | % |
| Expected term           | 6.0 years               |   | 6.0 years |   | 6.0 years |   |
| Risk-free interest rate | 2.64                    | % | 2.07      | % | 1.36      | % |
| Expected dividend yield | —                       | % | —         | % | —         | % |

Expected volatility. For the years ended December 31, 2018 and 2017, expected volatility was estimated using a weighted-average of the Company's historical volatility of its common stock and the historical volatility of the common stock of a representative group of publicly traded companies from the biopharmaceutical industry with similar characteristics as the Company, including stage of product development and therapeutic focus. The Company will continue to apply this process until a sufficient amount of historical information regarding the volatility of its own stock price becomes available.

For year ended December 31, 2016, the Company estimated expected volatility using only the historical volatility from a representative group of publicly traded companies from the biopharmaceutical industry with similar characteristics including stage of product development and therapeutic focus.

Expected term. The expected term of awards represents the period of time that the awards are expected to be outstanding. The expected term was determined using the simplified method as prescribed by the Securities and Exchange Commission Staff Accounting Bulletin No. 107, Share-Based Payment as the Company does not have sufficient historical exercise data to provide a reasonable basis upon which to estimate the expected term of stock options granted to employees.

Risk-free interest rate. For the years ended December 31, 2018, 2017 and 2016, the risk-free interest rate was estimated using an average of treasury bill interest rates over a period commensurate with the expected term of the option at the time of grant.

Expected dividend yield. The expected dividend yield is zero as the Company has not paid any dividends to date and has no current intention of paying cash dividends.

Forfeiture rate. The Company elected to estimate potential forfeiture of stock grants and adjust compensation cost recorded accordingly. The estimate of forfeitures is adjusted over the requisite service period to the extent that actual forfeitures differ, or are expected to differ, from such estimates. Changes in estimated forfeitures are recognized through a cumulative catch-up in the period of change and impact the amount of stock compensation expense to be recognized in future periods. For the years ended December 31, 2018, 2017 and 2016, the Company assumed forfeiture rates of approximately 7%, 7%, and 6%, respectively.



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The following is a summary of option activity under the 2006 Plan and 2014 Plan:

|  | Number of<br>Option Shares | Weighted<br>Average<br>Exercise<br>Price per<br>Share | Weighted<br>Average<br>Remaining<br>Contractual<br>Term<br>(In years) | Aggregate<br>Intrinsic<br>Value<br>(In thousands) |
|--|----------------------------|---|---|---|
| Outstanding at December 31, 2017                     | 2,889,722                  | \$ 11.25  |   |   |
| Granted  | 968,043                    | \$ 26.01  |   |   |
| Exercised  | (196,861 )                 | \$ 7.27   |   |   |
| Forfeited or expired                                 | (103,498 )                 | \$ 19.20  |   |   |
| Outstanding at December 31, 2018                     | 3,557,406                  | \$ 15.26  | 6.97  | 5,834   |
| Exercisable at December 31, 2018                     | 2,134,434                  | \$ 12.27  | 6.05  | 5,052   |
| Vested and expected to vest at December 31, 2018 (1) | 3,444,612                  | \$ 15.06  | 6.92  | 5,780   |

This represents the number of vested stock option shares as of December 31, 2018, plus the number of unvested (1) stock option shares that the Company estimated as of December 31, 2018 would vest, based on the unvested stock option shares at December 31, 2018 and an estimated forfeiture rate of 7%.

As of December 31, 2018 there was \$16.9 million of total unrecognized compensation cost related to stock options that are expected to vest. Total unrecognized compensation cost will be adjusted for future changes in forfeitures. The stock option costs are expected to be recognized over a weighted-average remaining vesting period of 2.3 years.

#### Restricted Stock units

On July 6, 2017, the Company granted 0.5 million restricted stock units, or RSUs, to executives and employees. The awards granted to employees are service-based, whereas the awards granted to executives are a blend of service-based and performance-based. Assuming all service and performance conditions were achieved, fifty percent of the RSUs would vest on March 31, 2018, and the remaining fifty percent of the RSUs would vest on March 31, 2019. Certain executive awards were subject to the achievement of defined performance criteria prior to March 31, 2018, including the closing of the Asset Purchase Agreement with Vertex and the institution by the Patent Trial and Appeal Board ("PTAB") of the Post Grant Review ("PGR") petition filed by the Company against Incyte Corporation. In January 2018, the PTAB decided not to institute the PGR petition and, as a result, the corresponding performance-based awards will not vest.

The Company is using the accelerated attribution method to recognize expense over the required service period based on its estimate of the number of performance-based awards that will vest. If there is a change in the estimate of the number of performance-based awards that are probable of vesting, the Company will cumulatively adjust compensation expense in the period that the change in estimate is made.

RSUs are not included in issued and outstanding common stock until the shares are vested and released. As of December 31, 2018, 174,050 RSUs had vested. The fair value of an RSU is measured based on the market price of the underlying common stock as of the date of grant.

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The following is a summary of RSU activity, including both time-based and performance-based restricted stock units for the year ended December 31, 2018:

|                                  | Number of<br>RSU<br>Shares | Weighted<br>Average<br>Grant<br>Date Fair<br>Value |
|----------------------------------|----------------------------|--|
| Outstanding at December 31, 2017 | 517,300                    | \$ 13.87   |
| Granted                          | —                          | \$ —   |
| Released                         | (174,050 )                 | \$ 13.87   |
| Forfeited                        | (115,100 )                 | \$ 13.87   |
| Outstanding at December 31, 2018 | 228,150                    | \$ 13.87   |

As of December 31, 2018, there was \$0.6 million of unrecognized compensation cost related to restricted stock units that are expected to vest. This amount excludes compensation cost related to restricted stock units where the performance conditions are not considered probable of being satisfied. The costs from restricted stock units that are considered probable of vesting are expected to be recognized over a weighted average remaining vesting period of 0.3 year.

Stock-based compensation expense

Total compensation cost recognized for all stock-based compensation awards in the consolidated statements of operations and comprehensive income (loss) is as follows (in thousands):

|  | For the Year Ended<br>December 31, |          |          |
|--|------------------------------------|----------|----------|
|  | 2018                               | 2017     | 2016     |
| Research and development               | \$5,221                            | \$3,708  | \$2,147  |
| General and administrative             | 6,279                              | 4,792    | 2,920    |
| Total stock-based compensation expense | \$ 11,500                          | \$ 8,500 | \$ 5,067 |

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9. Earnings (Loss) Per Share

Basic net earnings (loss) per common share is calculated by dividing net income (loss) allocable to common stockholders by the weighted-average common shares outstanding during the period, without consideration of common stock equivalents.

For periods with net income, diluted net earnings per share is calculated by either (i) adjusting the weighted-average shares outstanding for the dilutive effect of common stock equivalents, including warrants, stock options and restricted stock units outstanding for the period as determined using the treasury stock method or (ii) the two-class method considering common stock equivalents, whichever is more dilutive.

For purposes of the diluted net loss per share calculation, common stock equivalents are excluded from the calculation if their effect would be anti-dilutive. As such, basic and diluted net loss per share applicable to common stockholders are the same for periods with a net loss.

The two-class method is an earnings allocation formula that treats a participating security as having rights to earnings that otherwise would have been available to common stockholders. The Company has outstanding warrants, including those issued in connection with the Loan and Security Agreement described in Note 13, that are deemed to be participating securities. The two-class method was not applied for the years ended December 31, 2018 and 2016 as the Company's participating securities do not have any obligation to absorb net losses. The Company applied the two-class method to calculate basic and diluted net earnings per share of common stock for the year ended December 31, 2017. The following table illustrates the determination of earnings (loss) per share for each period presented.

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|  | For the Year Ended December 31,          |          |            |
|--|--|----------|------------|
|  | 2018                                     | 2017     | 2016       |
|  | (in thousands, except per share amounts) |          |            |
| <b>Basic Earnings per Share</b>  |  |          |            |
| Numerator:   |  |          |            |
| Net (loss) income  | \$(56,024)                               | \$95,639 | \$(50,720) |
| Income attributable to participating securities - basic  | —  | 444      | —          |
| (Loss) income attributable to common stockholders - basic  | (56,024 )                                | 95,195   | (50,720 )  |
| Denominator:   |  |          |            |
| Weighted average shares outstanding  | 23,370                                   | 22,641   | 22,233     |
| Net (loss) income per share applicable to common stockholders - basic  | \$(2.40 )                                | \$4.20   | \$(2.28 )  |
| <b>Diluted Earnings per Share</b>  |  |          |            |
| Numerator:   |  |          |            |
| Net (loss) income  | (56,024 )                                | 95,639   | (50,720 )  |
| Income attributable to participating securities - diluted  | —  | 429      | —          |
| (Loss) income attributable to common stockholders - diluted  | (56,024 )                                | 95,210   | (50,720 )  |
| Denominator:   |  |          |            |
| Weighted average shares outstanding  | 23,370                                   | 22,641   | 22,233     |
| Dilutive impact from:  |  |          |            |
| Stock options  | —  | 688      | —          |
| Restricted stock units   | —  | 113      | —          |
| Weighted average shares outstanding - diluted  | 23,370                                   | 23,442   | 22,233     |
| Net (loss) income per share applicable to common stockholders - diluted  | \$(2.40 )                                | \$4.06   | \$(2.28 )  |
| Anti-dilutive potential common stock equivalents excluded from the calculation of net (loss) income per share: |  |          |            |
| Stock options  | 666                                      | 1,833    | 620        |
| Restricted stock units   | 185                                      | 408      | —          |
| Warrants   | 132                                      | —        | 71         |

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## 10. Income Taxes

## New Tax Legislation

On December 22, 2017, the President of the United States signed into law the Tax Cuts and Jobs Act (“TCJA”). This legislation made broad and complex changes to the U.S. tax code, including, but not limited to, (i) reducing the U.S. federal statutory tax rate from 35% to 21%; (ii) eliminating the corporate alternative minimum tax (AMT) and changing how existing AMT credits can be realized; (iii) modifying the officer’s compensation limitation, and (iv) changing rules related to uses and limitations of net operating loss carryforwards created in tax years beginning after December 31, 2017. Specifically, the TCJA limited the amount the Company is able to deduct for net operating loss carryforwards generated in taxable years beginning after December 31, 2017 to 80% of taxable income however these net operating loss carryforwards can be carried forward indefinitely.

The Company recognizes the effects of changes in tax law, including the TCJA, in the period the law is enacted. Accordingly, the effects of the TCJA were recognized in the financial statements for the year ended December 31, 2017. As a result of the change in law, the Company recorded a reduction to its deferred tax assets of \$8.6 million and a corresponding reduction to its valuation allowance due to the reduction in the U.S. federal statutory rate from 35% to 21%.

In addition, the new legislation also repealed the corporate Alternative Minimum Tax (“AMT”) for years after 2017. Corporations that were previously subject to the AMT and have AMT tax credit carryforwards as of December 31, 2017, are eligible for a refund of these credits for tax years beginning after 2017 and before 2022. The Company was subject to AMT in the amount of \$1.9 million in 2017. Since the AMT paid on its 2017 tax return generated an AMT credit that will be refundable between 2018 and 2022, the Company recorded a \$1.9 million income tax receivable rather than a tax expense for 2017. Further, the Company also had a deferred tax asset for its AMT credit carryforward related to its AMT liability paid in 2015 in the amount of \$0.3 million. This deferred tax asset was previously offset by a full valuation allowance. As a result of the change in law, the Company reclassified the 2015 AMT credit carryforward from deferred tax assets to income tax receivable during 2017. As of December 31, 2018, the Company has a \$2.3 million income tax receivable related to AMT taxes in prior years.

At December 31, 2018, the Company has completed its accounting for the tax effects of the enactment of the TCJA and no adjustments were recorded to the provisional amounts previously recorded.

## Income Taxes

During the year ended December 31 2018, the Company recorded net loss before taxes of \$55.7 million and, since it maintains a full valuation allowance on its deferred tax assets, the Company did not record an income tax benefit for the year ended December 31, 2018. On July 25, 2017, the transaction contemplated by the Asset Purchase Agreement with Vertex, as discussed in Note 12, closed and Vertex paid the Company \$160 million in cash consideration, with \$16 million to be held in escrow. For income tax purposes, the \$16 million held in escrow is recognized under the installment method and is therefore deferred until the cash is received by the Company. Under the provisions of Section 453A of the Internal Revenue Code, the Company is required to recognize interest on the deferred tax liability with respect to the portion of the installment sale outstanding as of the close of each taxable year that exceeds \$5 million. As a result, during 2018 the Company recorded a provision of \$0.4 million, which includes \$0.2 million of interest accrued for tax year 2017 and \$0.2 million of interest accrued for tax year 2018. Additionally, during 2018 the Company recorded unrealized gains on its investments in available-for-sale securities in other comprehensive income. Intraproduct tax allocation rules require the Company to allocate its provision for income taxes between continuing operations and other categories of earnings, such as other comprehensive income. In periods in which the Company has a year-to-date pre-tax loss from continuing operations and pre-tax income in other categories of earnings, such as other comprehensive income, the Company must allocate the tax provision to the other categories of earnings. The Company then records a related tax benefit in continuing operations. As a result of these rules, the Company recorded a benefit of \$0.1 million in continuing operations, with a corresponding reduction in other comprehensive income of \$0.1 million, related to its unrealized gains on its investments in available-for-sale securities during 2018.

During the year ended December 31 2017, the Company recorded net income before taxes of \$95.3 million. As a result of the enactment of the TCJA, which allows for AMT to be refundable, the Company recorded a tax receivable

of \$2.2 million as of December 31, 2017 and a \$0.3 million income tax benefit during the year ended December 31, 2017. The tax benefit is the result of the removal of its valuation allowance on its AMT credit carryforward as previously described. Income taxes that would otherwise have been due on the 2017 taxable income were offset with the tax benefit of net operating loss carryforwards which had previously had a full valuation allowance, except for \$1.9 million of AMT incurred due to the limitation on use of net operating loss carryforwards when determining AMT. However, the 2017 AMT is also refundable under the Tax Cuts and

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Jobs Act of 2017 and thus we have not recorded a tax provision for this amount. The total amount of refundable AMT credits of \$2.2 million is reflected as income tax receivable in the accompanying consolidated balance sheet as of December 31, 2017. We provide a full valuation allowance for any tax benefit related to net operating losses due to the uncertainty of the ability to realize such benefits.

During the year ended December 31, 2016, the Company recorded a net loss of \$50.7 million and, since it maintained a full valuation allowance on its deferred tax assets, the Company did not record an income tax benefit for the year ended December 31, 2016.

The Company's ability to use its operating loss carryforwards and tax credit carryforwards to offset taxable income is subject to restrictions under Sections 382 and 383 of the United States Internal Revenue Code (the "Internal Revenue Code"). Net operating loss and tax credit carryforwards are subject to an annual limitation in the event of certain cumulative changes in the ownership interest of significant shareholders over a three-year period in excess of 50 percent, as defined under Sections 382 and 383 of the Internal Revenue Code. Such changes would limit the Company's use of its operating loss and tax credit carryforwards. In such a situation, the Company may be required to pay income taxes, even though significant operating loss and tax credit carryforwards exist. Additionally, any future financing could result in a change in control, as defined by Sections 382 and 383, which could further limit the Company's use its operating loss and tax credit carryforwards. In determining the tax provisions for fiscal year 2018, we assessed our ability to use our net operating loss carryforwards in accordance with Sections 382 and 383 of the Internal Revenue Code.

A reconciliation of the federal statutory income tax rate and the Company's effective income tax rate is as follows:

|  | Year ended December 31, |         |         |
|--|-------------------------|---------|---------|
|  | 2018                    | 2017    | 2016    |
| Federal statutory income tax rate          | 21.0 %                  | (35.0)% | 34.0 %  |
| State income taxes                         | 6.2 %                   | (5.1 )% | 4.5 %   |
| Change in valuation allowance              | (30.9)%                 | 46.2 %  | (40.3)% |
| Research and development and other credits | 4.0 %                   | 2.5 %   | 3.1 %   |
| Permanent items                            | (0.3 )%                 | 0.8 %   | (1.0 )% |
| Alternative minimum tax                    | — %                     | — %     | — %     |
| Other                                      | (0.6 )%                 | — %     | (0.3 )% |
| Federal rate change                        | — %                     | (9.1 )% | — %     |
| Effective income tax rate                  | (0.6 )%                 | 0.3 %   | — %     |

The significant components of the Company's net deferred tax assets consist of the following (in thousands):

|   | December 31, |           |
|---|--------------|-----------|
|   | 2018         | 2017      |
| Deferred tax assets:                                    |              |           |
| Net operating loss carryforwards                        | \$23,841     | \$11,670  |
| Deferred revenue  | 2,878        | 2,733     |
| Research and development and other credit carryforwards | 15,597       | 13,399    |
| Fixed assets  | —            | 236       |
| Other   | 7,985        | 3,575     |
|   | 50,301       | 31,613    |
| Valuation allowance                                     | (44,453 )    | (31,613 ) |
| Total deferred tax assets, net of valuation allowance   | \$5,848      | \$—       |
| Deferred tax liabilities:                               |              |           |
| Fixed assets  | \$1,477      | \$—       |
| Gain deferred under installment method                  | 4,371        | —         |
| Total deferred tax liabilities                          | \$5,848      | \$—       |
| Net deferred tax assets                                 | \$—          | \$—       |





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Subject to the limitations described above and the impacts of the TCJA, at December 31, 2018, the Company had gross federal net operating loss carryforwards of \$99.5 million and state net operating loss carryforwards of \$46.7 million available to reduce future taxable income, of which \$54.8 million of the gross federal net operating loss carryforwards and \$46.7 million of the state net operating loss carryforwards will expire at various dates beginning in 2034. Approximately \$44.7 million of the federal net operating loss carryforwards will be carried forward indefinitely. The Company also had federal and state tax credit carryforwards of \$11.7 million and \$4.9 million, respectively, available to reduce future tax liabilities, which expire at various dates through 2038.

The Company adopted ASU 2016-09, Improvements to Employee Share-Based Payment Accounting on January 1, 2017. As a result of adoption, the deferred tax assets associated with net operating losses as of December 31, 2016 have increased by \$3.2 million. These amounts were offset by a corresponding increase in the valuation allowance.

The adoption of ASU 2016-09 had no impact on the Company's operations, financial position or cash flows.

The Company adopted ASC 606 using the modified retrospective transition method as permissible for all contracts not yet completed as of January 1, 2018. This created approximately \$4.3 million of deferred tax liabilities relating to federal and state deferred revenue that are fully offset by a corresponding decrease in the valuation allowance. As a result, there was no cumulative effect adjustment to accumulated deficit.

Realization of the future tax benefits is dependent on many factors, including the Company's ability to generate taxable income within the carryforward period. The Company currently has deferred tax assets in excess of its deferred tax liabilities, resulting in the Company having net deferred tax assets. The Company has evaluated the positive and negative evidence bearing upon the realizability of its net deferred tax assets and concluded that it is more likely than not that the Company will not realize the benefit of its net deferred tax assets. As a result, the net deferred tax assets have been fully reserved at December 31, 2018 and 2017.

At December 31, 2018, the Company had no unrecognized tax benefits. The Company has not conducted a study of its research and development credit carryforwards. A study may result in an adjustment to the Company's research and development credit carryforwards; however, until a study is completed and any adjustment is known, no amounts will be presented as an uncertain tax position. A full valuation allowance has been provided against the Company's research and development credit carryforwards and, if an adjustment is required, this adjustment would be offset by an adjustment to the valuation allowance. Thus, there would be no impact to the consolidated balance sheet or statement of operations if an adjustment were required.

Interest and penalty charges, if any, related to unrecognized tax benefits would be classified as income tax expense in the accompanying statement of operations. As of December 31, 2018, the Company had no accrued interest related to uncertain tax positions.

The Company is currently open to examination under the statute of limitations by the Internal Revenue Service and state jurisdictions for the tax years ended 2015 through 2017. Carryforward tax attributes generated in years prior to 2015 may still be adjusted upon future examination if they have or will be used in a future period. The Company is currently not under examination by the Internal Revenue Service or any other jurisdictions for any tax years. Since the Company is in a loss carryforward position, the Company is generally subject to examination by the U.S. federal, state and local income tax authorities for all tax years in which a loss carryforward is available.

## 11. Commitments

The Company currently leases 55,522 square feet of office and laboratory space (the "Lease") located at 65 Hayden Avenue, Lexington, Massachusetts (the "Premises"). The Company occupied the Premises in the third quarter of 2018, however the Company gained access to the space on January 1, 2018 in order to start making certain tenant improvements. Accordingly, for accounting purposes the lease commencement date is January 1, 2018. The Lease term extends ten years following January 1, 2019. The Company is entitled to two five-year options to extend the Lease. The Lease is accounted for as an operating lease.

The Lease provides for annual base rent of approximately \$2.8 million in the first year following the Base Rent Commencement Date of January 1, 2019, which increases on a yearly basis by 3.0% (subject to an abatement of base rent of approximately \$0.5 million at the beginning of the second year of the Lease term if the Company is not in default under the Lease). The Company is also obligated to pay the Landlord for certain costs, taxes and operating expenses related to the Premises, subject to certain exclusions. The Company is recognizing rental expense on a straight-line basis, beginning on the

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lease commencement date of January 1, 2018, over the term of the lease with corresponding rent differential accounted for as deferred rent.

The Company received an improvement allowance from the Landlord of approximately \$5.0 million for certain permitted costs related to the design of Company improvements to the Premises, consisting of normal tenant improvements. The Company is deemed to be the owner of these tenant improvements during the lease term. The Company is accounting for the tenant improvements funded by the Landlord as a lease incentive obligation to be amortized against operating lease expense on a straight-line basis over the term of the Lease. Leasehold improvements, including those funded by the Landlord and those funded by the Company, have been recognized as assets and are amortized on a straight-line basis over the term of the Lease.

The Company provided a security deposit, in the form of a stand-by letter of credit, in the amount of approximately \$1.2 million, which may be used by the Landlord to be applied for certain purposes upon the Company's breach of any provisions under the Lease.

The Lease contains customary provisions allowing the Landlord to terminate the Lease if the Company fails to remedy a breach of any of its obligations within specified time periods, or upon bankruptcy or insolvency of the Company.

The Company previously leased approximately 50,000 square feet of office and laboratory space at 99 Hayden Avenue, Lexington, Massachusetts under a non-cancelable operating lease agreement, or the 2008 Lease Agreement, as amended. The term of the 2008 Lease Agreement expired September 30, 2018.

The future minimum lease payments under the Lease is as follows (in thousands):

|                              | Base rent obligations |
|------------------------------|-----------------------|
| At December 31, 2018         |                       |
| 2019                         | \$ 2,776              |
| 2020                         | 2,383                 |
| 2021                         | 2,945                 |
| 2022                         | 3,034                 |
| 2023                         | 3,125                 |
| Greater than 5 years         | 17,085                |
| Total minimum lease payments | \$ 31,348             |

## 12. Revenue

The Company's revenue is currently generated through collaborative licensing agreements, patent assignments, and sales of intellectual property. The Company generates its revenue through one segment and the revenue recognized under each of the Company's arrangements during the current and prior periods is described below. The terms of these agreements may contain multiple promised goods or services or optional goods and services, including licenses, or options to obtain licenses, to product candidates, referred to as exclusive licenses, as well as research and development activities to be performed by the Company on behalf of the collaboration partner related to the licensed product candidates.

### Revenue recognition

Revenue is recognized when control of the promised goods or services are transferred to customers, in an amount that reflects the consideration the Company expects to be entitled to in exchange for transferring those goods or providing services. The Company accounts for a contract when it has approval and commitment from both parties, the rights of the parties are identified, payment terms are identified, the contract has commercial substance and collectability of consideration is probable.

When determining whether the customer has obtained control of the goods or services, the Company considers the point at which the customer may benefit from the goods or services. For licenses to product candidates, revenue is recognized upon grant or transfer of the exclusive license, as the Company's licenses are considered functional in nature. For research, development, and manufacturing activities, revenue is recognized as the work is performed using either the output or input method.

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Performance obligations

A performance obligation is a promise in a contract to transfer a distinct good or service to the customer, and is the unit of account in ASC 606. A contract's transaction price is allocated to each distinct performance obligation and recognized as revenue when, or as, the performance obligation is satisfied. The Company's contracts may contain multiple performance obligations if a promise to transfer goods or services is separately identifiable from other promises in a contract and, therefore, is considered distinct. For contracts with multiple performance obligations, the Company determines the standalone selling price of each performance obligation and allocates the total transaction price using the relative selling price basis. The Company recognizes performance obligations based on their nature.

Options to exclusive licenses

The collaborative arrangement with Celgene provides the customer the option to purchase additional licenses in addition to preclinical and clinical development services at a discount. These options are considered performance obligations as they provide the customer with material rights that the customer would not receive without entering into the contract. The portion of the transaction price attributed to a material right is recognized when the underlying option is exercised or when the option expires. To date, Celgene has not exercised any of its options that were determined to represent material rights.

Significant Payment Terms

The Company's revenue arrangements include payments to the Company of one or more of the following: a nonrefundable, upfront payment; milestone payments; payment of license exercise or option fees with respect to product candidates; fees for research and development services rendered; and royalties on commercial sales of licensed product candidates, if any. To date, the Company has received upfront payments, several milestone payments and certain research and development service payments but has not received any license exercise or option fees or earned royalty revenue as a result of product sales.

Under ASC 606, the Company estimates the amount of consideration to which it will be entitled in exchange for satisfying performance obligations. Based on the Company's current contracts, variable consideration primarily exists in the following forms: development and regulatory milestones, royalties and sales-based milestones, and consideration held in escrow for indemnification purposes. The Company utilizes the "most likely amount" variable consideration method for estimating development and regulatory milestone consideration to include in the transaction price and the "expected value" variable consideration method for the consideration held in escrow for indemnification purposes. The Company only includes an amount of variable consideration in the transaction price to the extent it is probable that a significant reversal in the cumulative revenue recognized will not occur when the uncertainty associated with the variable consideration is subsequently resolved. The Company refers to this as the variable consideration constraint.

Due to the uncertainty associated with the occurrence of the underlying events which would trigger development and regulatory milestone consideration under its revenue arrangements, with the exception of those development and regulatory milestones received to date, the Company has concluded the variable consideration associated with all development and regulatory milestones to be fully constrained as of the ASC 606 transition date and as of December 31, 2018 and therefore has not included such consideration in the transaction price for any of its revenue arrangements. The Company will re-assess this conclusion at each subsequent reporting period and will only include amounts associated with regulatory or development milestones in the transaction price when, or if, the variable consideration is determined to be released from the constraint.

To date, the Company has not recognized any royalties or sales-based milestones under its licensing and collaboration arrangements. Royalties and sales-based milestones qualify for the sales-and-usage exemption under ASC 606 as (i) royalties are based strictly on the sales-and-usage by the licensee and (ii) a license of IP is the sole or predominant item to which such royalties relate. Based on this exemption, these royalties are earned under the terms of a license agreement in the period the products are sold by the Company's collaborator and the Company has a present right to payment.

In accordance with ASC 606, the Company is required to adjust the transaction price for the effects of the time value of money if the timing of payments agreed to by the parties to the contract, explicitly or implicitly, provides the Company or its customer with a significant benefit of financing the transfer of goods or services. The Company concluded that its licensing and collaboration arrangements do not contain a significant financing component because the payment structure of its agreements arise from reasons other than providing a significant benefit of financing.

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Application of Practical Expedients

The collaborative arrangements with Glaxo Group Limited, or GSK, and Jazz Pharmaceuticals contained contract modifications. The Company elected to apply the transition practical expedient under ASC 606-10-65-1(f)(4) that allows an entity to reflect the aggregate effect of all contract modifications on contracts that were modified before the beginning of the earliest period presented under the new standard (that is, January 1, 2018) when (i) identifying the satisfied and unsatisfied performance obligations, (ii) determining the transaction price, and (iii) allocating the transaction price to the satisfied and unsatisfied performance obligations. The application of the practical expedient did not have a material effect on the Company's revenue recognition.

Contract Assets

As of January 1, 2018, the Company identified a contract asset of \$16.0 million associated to the Vertex indemnification payment held in escrow. As the receipt of the Vertex indemnification consideration involves more than the passage of time, the consideration was concluded to be conditional and therefore classified as a contract asset. As of December 31, 2018, there have been no changes to the balance of the Company's contract asset from the date of adoption of ASC 606. In February 2019, the escrow was released to the Company.

Contract Liabilities

As of January 1, 2018, the Company identified contract liabilities of \$10.5 million related to unsatisfied performance obligations as well as variable consideration paid in advance but currently constrained from recognition. Contract liabilities are presented as deferred revenue and classified as current or noncurrent based on the timing of when the Company expects to recognize revenue. During the twelve months ended December 31, 2018, \$16 thousand of deferred revenue was recognized into revenue. As of December 31, 2018, the Company recorded \$10.5 million of contract liabilities in the condensed consolidated statement of financial position.

Collaboration Arrangements

Celgene

In April 2013, the Company entered into a master development and license agreement with Celgene Corporation and Celgene International Sàrl, referred to together as Celgene, which agreement is primarily focused on the research, development and commercialization of specified deuterated compounds targeting inflammation or cancer.

The initial program in the collaboration is CTP-730, a deuterium-modified analog of apremilast. Celgene has an exclusive worldwide license to develop, manufacture and commercialize deuterated analogs of apremilast and certain close chemical derivatives thereof. The Company further granted Celgene licenses with respect to two additional programs and an option with respect to a third additional program.

With respect to the two additional license programs, the Company granted Celgene an upfront exclusive worldwide license to develop, manufacture and commercialize deuterated products that contain deuterated analogs of the agreed non-deuterated compounds. Celgene is restricted from utilizing their research, development and commercialization rights under each of these upfront licenses, unless, within seven years after the effective date of the agreement, Celgene pays the Company a license exercise fee. If Celgene does not elect to pay the license exercise fee during the seven year period, the license will expire. With respect to the option program, once a compound is selected, Celgene may exercise its option by paying the Company an option exercise fee within seven years of the effective date of the agreement, and upon Celgene's exercise of the option the Company will grant to Celgene an exclusive worldwide license to develop, manufacture and commercialize deuterated products that contain deuterated analogs of the selected non-deuterated compound.

As a result of the restrictions placed on the two additional license programs that preclude Celgene from exercising its rights under the respective licenses without the payment of a significant license exercise fee, for accounting purposes the Company concluded that it had effectively provided Celgene an option to obtain licenses to those programs. The Company was responsible for conducting and funding research and early development activities for the CTP-730 program at its own expense pursuant to mutually agreed upon development plans. This included the completion of single and multiple ascending dose Phase 1 clinical trials in 2015.





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The Company does not have any obligation to conduct any research or development activities for any of the additional programs unless and until Celgene exercises its rights with respect to such program and pays the applicable exercise fee. If Celgene exercises its rights with respect to any additional program and pays the Company the applicable exercise fee, the Company is responsible, at its own expense, for conducting research and development activities for such program pursuant to agreed-upon development plans until the completion of Phase 1 clinical trial, which will be defined in each development plan on a program-by-program basis. In addition, if Celgene exercises its rights with respect to the option program and pays the Company the applicable option exercise fee, the Company is responsible for seeking to generate a deuterated compound for clinical development in the selected option program. Oversight of the development program for each program under the Celgene Agreement is guided by separate JSCs.

Celgene is solely responsible for all research, development and commercialization costs with respect to the initial program beyond the Phase 1 clinical trials that the Company conducts. If Celgene exercises its rights with respect to any additional program, Celgene will be solely responsible for all research, development and commercialization costs for such program following the completion of the first Phase 1 clinical trial for such program.

Under the terms of the agreement, the Company received a non-refundable upfront payment of \$35.0 million. In October 2015, the Company received an \$8.0 million development milestone payment based on the completion of Phase 1 clinical evaluation of CTP-730. In addition, the Company is eligible to earn an additional \$15.0 million development milestone payment, up to \$247.5 million in regulatory milestone payments and up to \$50.0 million in sales-based milestone payments related to products within the CTP-730 program. The next milestone payment the Company may be entitled to achieve under the CTP-730 program is \$15.0 million related to the first actual dosing in a Phase 3 clinical trial or, if earlier, acceptance for filing of a new drug application, or NDA. If Celgene exercises its rights with respect to either of the two additional license programs, the Company will receive a license exercise fee for the applicable program of \$30.0 million and will also be eligible to earn up to \$23.0 million in development milestone payments and up to \$247.5 million in regulatory milestone payments for that program. Additionally, with respect to one of the additional license programs, the Company is eligible to receive up to \$100.0 million in milestone payments based on net sales of products, and with respect to the other additional license program, the Company is eligible to receive up to \$50.0 million in milestone payments based on net sales of products. If Celgene exercises its option with respect to the option program, in respect of a compound to be identified at a later time, the Company will receive an option exercise fee of \$10.0 million and will be eligible to earn up to \$23.0 million in development milestone payments and up to \$247.5 million in regulatory milestone payments.

In addition, with respect to each program, Celgene is required to pay the Company royalties on worldwide net sales of each licensed product at defined percentages ranging from the mid-single digits to low double digits below 20%. The royalty rate is reduced on a country-by-country basis during any period within the royalty term when there is no patent claim or regulatory exclusivity covering the licensed product in the particular country.

Under ASC 606, the Company's collaborative arrangement with Celgene contains the following performance obligations: (i) an exclusive worldwide license to develop, manufacture and commercialize deuterated analogs of apremilast related to the CTP-730 program, or the License Performance Obligation, (ii) obligations to perform research and development services associated with the CTP-730 program, or the R&D Services Performance Obligation, (iii) obligation to supply nonclinical and clinical trial material related to the CTP-730 program, or the Supply Performance Obligation, (iv) material right related to the first additional license program for which the non-deuterated compound has been selected, or the First Discount Performance Obligation and (v) material right related to the second additional license program for which the non-deuterated compound has been selected, or the Second Discount Performance Obligation.

The transaction price, exclusive of amounts subject to the variable consideration constraint, as of the transition date consisted of the \$35.0 million non-refundable upfront payment and the \$8.0 million milestone payment received upon successful completion of the Phase 1 clinical program totaling to \$43.0 million. The Company allocated the upfront arrangement consideration of \$35.0 million among the performance obligations using the relative selling price method based on the standalone selling prices of each performance obligation, which is generally the price at which it would sell such deliverable if it were to be sold regularly on a standalone basis. The standalone selling price of License

Performance Obligation was based on historical valuations for other licensing arrangements entered into by the Company. The standalone selling prices of the R&D Services Performance Obligation and the Supply Performance Obligation were based on the expected cost plus margin approach. The standalone selling prices of the First and Second Discount Performance Obligations were based the expected value of the options. The Company allocated the \$8.0 million milestone payment to only the License Performance Obligation, R&D Services Performance Obligation and the Supply Performance Obligation using the relative selling price method based on the standalone selling prices of each of these three performance obligations. The Company concluded that the achievement of the

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performance-based milestone for the CTP-730 program should only be allocated to the performance obligations associated to the CTP-730 program as the achievement of the milestone related specifically to the Company's efforts with respect to the satisfaction of the performance obligations related to that program. The transaction price was allocated as follows: (i) \$21.7 million to the License Performance Obligation, (ii) \$11.0 million to the R&D Services Performance Obligation, (iii) \$4.0 million to the Supply Performance Obligation, (iv) \$3.2 million to the First Discount Performance Obligation and (v) \$3.2 million to the Second Discount Performance Obligation. Revenue is recognized when the performance obligation is considered satisfied. The License Performance Obligation was considered satisfied at contract inception as the exclusive license transferred control to the customer at this point in time. The R&D Services Performance Obligation and the Supply Performance Obligation are satisfied over time using the input method based on costs incurred determined by estimates of associated effort and cost of services adjusted for a reasonable profit margin such that they represent estimated market rates for similar services on a standalone basis. The First Discount Performance Obligation and the Second Discount Performance Obligation shall be considered satisfied upon the option's exercise or expiration. Unsatisfied performance obligations represent contract liabilities that are presented as deferred revenue within the accompanying consolidated balance sheet. The collaborative arrangement with Celgene contains consideration that is variable based on the customer's achievement of certain development, regulatory, and sales-based milestones in addition to royalties upon the customer's commercial success with licensed programs. The next milestone payment of \$15.0 million upon first actual dosing in a Phase 3 clinical trial, or if earlier, acceptance for filing of a new drug application, or NDA, for the CTP-730 program is considered variable consideration that is fully constrained due to the uncertainty associated to the achievement of the milestone. As a result, the variable consideration that is considered fully constrained related to this and the remaining development and regulatory milestones will not be recognized until the time at which the constraint is released. The consideration related to royalty and sales-based milestones are recognized pursuant to the sales- and usage-based exemption under ASC 606. The variable consideration related to sales-based milestones and royalties will be recognized in the period the products are sold by Celgene and the Company has a present right to payment. During the year ended December 31, 2018, the Company recognized no revenue for the R&D Services and Supply Performance Obligation as no services were performed. During the years ended December 31, 2017 and 2016 the Company recognized revenue of \$20 thousand, and \$62 thousand for the R&D Services and Supply Performance Obligations, respectively. The revenue in the prior year period was recognized in accordance with legacy authoritative guidance. The revenue was classified as license and research and development revenue in the accompanying consolidated statements of operations and comprehensive income (loss).

As of December 31, 2018, there was \$7.8 million of deferred revenue related to the Company's collaboration with Celgene, consisting of \$1.3 million related to the R&D Services Performance Obligation, \$0.1 million related to the Supply Performance Obligation and \$6.4 million related to the First and Second Discount Performance Obligations. The Company classified the \$1.4 million related to the R&D Services Performance Obligation and the Supply Performance Obligation as a current liability and the \$6.4 million related to the First and Second Discount Performance Obligations as a noncurrent liability in the accompanying consolidated balance sheet.

#### Avanir

In February 2012, the Company entered into a development and license agreement with Avanir Pharmaceuticals, Inc., or Avanir, under which the Company granted Avanir an exclusive worldwide license to develop, manufacture and commercialize deudextromethorphan-containing products. Avanir is currently focused on developing AVP-786, which is a combination of deudextromethorphan and an ultra-low dose of quinidine. Subsequent to the Company's agreement, Avanir was acquired by Otsuka Pharmaceutical Co., Ltd. and it is now a wholly owned subsidiary of Otsuka America, Inc.

Since June 2012, Avanir has elected to conduct all research and development activities, including manufacturing activities; however, the Company has received intellectual property cost reimbursements.

Under the agreement, the Company received a non-refundable upfront payment of \$2.0 million and has received milestone payments of \$6.0 million. The Company is also eligible to earn, with respect to licensed products comprising a combination of deudextromethorphan and quinidine, up to \$37.0 million in regulatory and commercial

launch milestone payments, of which \$21.5 million in development and regulatory milestone payments are associated with the first indication, and up to \$125.0 million in sales-based milestone payments. The next milestone payments that the Company may be entitled to receive are \$5.0 million upon acceptance for filing of a NDA, \$3.0 million upon acceptance for filing of a Marketing Authorization Application, or MAA, and \$1.5 million upon acceptance for filing of a NDA by the Ministry of Health, Labour and Welfare, or MHLW,

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related to AVP-786. In addition, the Company is eligible for higher development milestones, up to an additional \$43.0 million, for licensed products that do not require quinidine. Avanir is currently developing deudextromethorphan only in combination with quinidine.

Avanir also is required to pay the Company royalties at defined percentages ranging from the mid-single digits to low double digits below 20% on net sales of licensed products on a country-by-country basis. The royalty rate is reduced, on a country-by-country basis, during any period within the royalty term when there is no patent claim covering the licensed product in the particular country.

The Company determined that all promised goods and services related to the arrangement with Avanir were considered satisfied as of the ASC 606 adoption date of January 1, 2018.

The transaction price, exclusive of amounts subject to the variable consideration constraint, consists of the \$2.0 million non-refundable upfront payment and \$6.0 million in development milestone payments received totaling to \$8.0 million. As all promised goods and services were considered satisfied as of the ASC 606 adoption date, the arrangement consideration need not be allocated among the performance obligations because the arrangement consideration was fully recognized as of January 1, 2018.

The arrangement with Avanir contains consideration that is variable based on the customer's achievement of certain development, regulatory, and sales-based milestones in addition to royalties upon the customer's commercial success with the licensed product. The \$6.0 million resulting from the achievement of development milestones to date represents variable consideration that has been earned and therefore is not subject to constraint. The next milestones that the Company may be entitled to are regulatory milestones that represent variable consideration that is fully constrained due to the uncertainty associated to the achievement of milestones of this nature. As a result, the variable consideration that is considered fully constrained will not be recognized until the time at which the constraint is released. The consideration related to royalty and sales-based milestones are recognized pursuant to the sales- and usage-based exemption under ASC 606. The variable consideration related to sales-based milestones and royalties will be recognized in the period the products are sold by Avanir and the Company has a present right to payment.

#### Jazz Pharmaceuticals

In February 2013, the Company entered into a development and license agreement with Jazz Pharmaceuticals, Inc., or Jazz Pharmaceuticals, to research, develop and commercialize products containing a deuterated sodium oxybate analog, or D-SXB. Under the agreement, Jazz Pharmaceuticals initially focused on one analog, designated as JZP-386, a once-nightly oxybate product. Under the terms of the agreement, the Company granted Jazz Pharmaceuticals an exclusive, worldwide, royalty-bearing license under intellectual property controlled by the Company to develop, manufacture and commercialize D-SXB products including, but not limited to, JZP-386.

Under the agreement, the Company received a non-refundable upfront payment of \$4.0 million and is eligible to earn an aggregate of up to \$8.0 million in development milestone payments, up to \$35.0 million in regulatory milestone payments and up to \$70.0 million in sales-based milestone payments based on net product sales of licensed products. The next milestone payment that the Company may be entitled to receive is \$4.0 million related to initiation of the first Phase 2 clinical trial of a D-SXB compound.

In addition, Jazz Pharmaceuticals is required to pay the Company royalties at defined percentages ranging from the mid-single digits to low double digits below 20% on worldwide net sales of licensed products. The royalty rate is lowered, on a country-by-country basis, under certain circumstances as specified in the agreement.

The Company determined that all promised goods and services related to the collaborative arrangement with Jazz Pharmaceuticals were considered satisfied as of the ASC 606 adoption date of January 1, 2018.

The transaction price, exclusive of amounts subject to the variable consideration constraint, consists of the \$4.0 million non-refundable upfront payment. As all promised goods and services were considered satisfied as of the ASC 606 adoption date, the arrangement consideration need not be allocated among the performance obligations.

Furthermore, as all promised goods and services are considered satisfied, the consideration was fully recognized as of January 1, 2018.

The collaborative arrangement with Jazz Pharmaceuticals contains consideration that is variable based on the customer's achievement of certain development, regulatory, and sales-based milestones in addition to royalties upon

the customer's commercial success with the licensed product. The next milestone payment the Company may be entitled to receive of \$4.0

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million related to initiation of the first Phase 2 clinical trial of JZP-386 or D-SXB is considered variable consideration that is fully constrained due to the uncertainty associated to the achievement of the development milestone. As a result, the variable consideration that is considered fully constrained will not be recognized until the time at which the constraint is released. The consideration related to royalty and sales-based milestones are recognized pursuant to the sales- and usage-based exemption under ASC 606. The variable consideration related to royalties will be recognized in the period the products are sold by Jazz and the Company has a present right to payment.

Other Revenue Arrangements

Vertex

On March 3, 2017, the Company and Vertex entered into an Asset Purchase Agreement pursuant to which, subject to the satisfaction or waiver of the conditions therein, the Company sold and assigned to Vertex, CTP-656, a deuterated analog of ivacaftor now known as VX-561, and other cystic fibrosis assets of the Company. On July 25, 2017, the Closing Date, the transaction contemplated by the Asset Purchase Agreement closed and Vertex paid the Company \$160 million in cash consideration, with \$16 million initially held in escrow for a period of eighteen months. In February 2019, the \$16.0 million held in escrow was released to the Company.

Additionally, upon the achievement of certain milestone events, Vertex has agreed to pay the Company an aggregate of up to \$90 million. Of this amount, \$50 million will become payable to the Company upon receipt of FDA marketing approval for a combination treatment regimen containing VX-561, for patients with cystic fibrosis, and \$40 million will become payable to the Company upon completion of a pricing and reimbursement agreement in the first of the United Kingdom, Germany or France with respect to a combination treatment regimen containing VX-561 for patients with cystic fibrosis.

The Asset Purchase Agreement with Vertex contains a single performance obligation: all rights to develop, manufacture, and commercialize deuterated analogs of ivacaftor related to the CTP-656 program, including all intellectual property, permits and registrations, and records, documentation, and regulatory filings, in addition to an obligation to perform research and testing consulting services to facilitate the transfer of materials, documents, and knowledge up to the close of the Asset Purchase Agreement, referred to as the Transfer of IP Performance Obligation. The Asset Purchase Agreement with Vertex contains consideration that is variable based on Vertex's achievement of certain regulatory milestones in addition to the \$16.0 million held in escrow to indemnify Vertex, which was released to us February 2019. The regulatory milestone payments are fully constrained due to the uncertainty associated to the achievement of the respective milestones. The Company concluded that an indemnification claim was remote and as a result was not subject to the variable consideration constraint at the ASC 606 transition date. Accordingly, the variable consideration of \$16.0 million was included in the transaction price.

The transaction price was \$160.0 million, as the variable consideration associated with the escrow amount was not subject to the constraint. As the arrangement contained a single performance obligation, the Company attributed the full transaction price (exclusive of amounts subject to the variable consideration constraint) of \$160.0 million to the Transfer of IP Performance Obligation.

The Transfer of IP Performance Obligation was satisfied as of the Closing Date as the control of CTP-656 transferred to Vertex, the customer. As a result, the full transaction price was recognized as revenue as of the ASC 606 adoption date.

As of December 31, 2018, the Vertex indemnification variable consideration represented a contract asset to be released from escrow 18 months following the Closing Date and was classified as a current asset in the accompanying consolidated balance sheet. In February 2019, the \$16.0 million initially held in escrow was released to the Company.

GSK

In May 2009, the Company entered into a research and development collaboration and license agreement with GSK to research, develop and commercialize multiple products containing deuterated compounds, including CTP-499. The agreement with GSK, as subsequently amended, expired in May 2012 after GSK opted out of further development under the agreement and made a \$2.8 million payment to the Company. The Company has an obligation to make a payment to GSK of up to \$2.8 million if the Company commercializes CTP-499 or if the Company receives cash

proceeds from re-licensing or transferring the rights to the CTP-499 program.

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Under the new revenue standard, the \$2.8 million payment represents variable consideration that is fully constrained as of the ASC 606 adoption date due to the likelihood the Company may be required to repay GSK as a result of the transaction contemplated by the License and Option Agreement with Promet Therapeutics, LLC, discussed further in this Note 12. The \$2.8 million payment is a contract liability that is classified as deferred revenue as of December 31, 2018 and will not be recognized as revenue until the repayment obligation lapses.

**Auspex**

In September 2011, the Company entered into a patent assignment agreement with Auspex Pharmaceuticals, Inc., or Auspex, pursuant to which the Company assigned to Auspex a U.S. patent application relating to deuterated pirfenidone analogs. Under the terms of the agreement, the Company is eligible to receive certain royalty payments, or the Royalty Payments, equal to a percentage in the low single digits of net sales in the United States invoiced by Auspex or any of its affiliates, with respect to certain pharmaceutical products containing a deuterated pirfenidone analog. The patent assignment agreement further provides that if Auspex sells to another party all of its U.S. rights to certain deuterated pirfenidone products, or if Auspex grants to another party a license to sell certain deuterated pirfenidone products in the United States, the Company will receive an amount, or the Sublicense/Sale Payments, equal to a percentage in the teens of any proceeds Auspex receives therefrom that are attributable to the rights to such deuterated pirfenidone products in the United States. In addition, the patent assignment agreement provides that if Auspex is acquired in a change in control transaction at any time while it, or any of its affiliates, own certain patents or patent applications related to deuterated pirfenidone, the Company will receive within a specified period following the closing of the transaction 1.44% of any proceeds payable as consideration for the change in control transaction, including any amounts paid to stockholders and certain equity holders of Auspex. Any such change in control payment to the Company is credited to Auspex as a deduction against any future Royalty Payments and Sublicense/Sale Payments that may become due under the agreement, such that Auspex will not be required to make further Royalty Payments and Sublicense/Sale Payments to the Company until the aggregate amount of such Royalty Payments and Sublicense/Sale Payments exceeds the amount of such change in control payment. The patent assignment agreement expires upon the earlier to occur of (1) receipt by the Company of the final Sublicense/Sale Payment arising from (a) the sale of Auspex's U.S. rights to certain deuterated pirfenidone products or (b) Auspex's grant of an exclusive license to sell certain deuterated pirfenidone products in the United States in all indications and fields, or (2) the expiration of the last claim owned by Auspex or any of its affiliates in certain patents or patent applications related to deuterated pirfenidone analogs.

Under the agreement, Concert became eligible to receive a one-time payment of \$50.2 million, which was received in June 2015, due to Teva Pharmaceutical Industries Ltd.'s acquisition of Auspex in May 2015.

The Company determined that all performance obligations in the patent assignment agreement have been satisfied as of the ASC 606 adoption date of January 1, 2018.

Allocable arrangement consideration as of the ASC 606 adoption date was limited to the transaction price consisting of the one-time change of control payment of \$50.2 million. As all promised goods and services in the arrangement were considered satisfied as of the ASC 606 adoption date, the arrangement consideration need not be allocated among the performance obligations because the arrangement consideration was fully recognized as of January 1, 2018.

The arrangement with Auspex contains consideration that is variable in amount, including the potential royalties that may be due upon the commercial success of deuterated pirfenidone products. The \$50.2 million resulting from the change of control payment represents variable consideration that has been received and therefore is not subject to constraint. The Company determined that the sales-and-usage royalty exemption under ASC 606 is not applicable as the Company assigned the intellectual property to Auspex, rather than enter into a license transaction. Accordingly, the consideration related to potential royalties represents variable consideration that is fully constrained due to the significant level of uncertainty related to the development prospects of the assigned patent. As a result, the consideration related to potential royalties will not be recognized until the time at which the constraint is released.

Processa

On October 4, 2017, the Company entered into a License and Option Agreement, or the Option, with Promet Therapeutics, LLC, or Promet, pursuant to which the Company granted Promet an option to obtain an exclusive license to CTP-499, a deuterated analog of 1-(S)-5-hydroxyhexyl-3,7-dimethylxanthine, or HDX, an active metabolite of pentoxifylline, provided certain conditions were met. On October 5, 2017, Promet closed an asset purchase agreement with Heatwurx, Inc., a public company, creating Processa Pharmaceuticals, Inc., or Processa.

CONCERT PHARMACEUTICALS, INC.  
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On March 19, 2018, the Company entered into an Amendment to the Option, or Amendment, and a Securities Purchase Agreement, or Securities Agreement, both with Promet and Processa. Pursuant to the Amendment, the Company granted Promet, who then assigned to Processa, an exclusive, worldwide, royalty-bearing license to develop, manufacture and commercialize CTP-499, now known as PCS-499. Upon transfer of the license and as consideration for the license, the Company received 2,090,301 shares of common stock of Processa, representing approximately 5.4% of the common stock outstanding.

The Company is also eligible to receive royalties on worldwide net sales.

The Amendment contained one performance obligation: an exclusive, worldwide, royalty-bearing license to develop, commercialize and sublicense CTP-499. The Company determined that the transaction price was \$10.5 million, which was based on the fair value of the noncash consideration received on March 19, 2018, which consisted of 2,090,301 shares of publicly traded common stock of Processa. The transaction price of \$10.5 million was allocated to the single performance obligation. The performance obligation was considered satisfied at contract inception as the exclusive license transferred control to the customer at this point in time. Accordingly, revenue of \$10.5 million was recognized during the first quarter of 2018.

Subsequent changes to the fair value of the underlying securities is recognized as unrealized gains or losses on marketable equity securities within the consolidated statement of operations and comprehensive income (loss). The Amendment contains consideration that is variable based on royalties upon the customer's commercial success with the licensed product. The consideration related to royalty milestones is recognized pursuant to the sales- and usage-based exemption under ASC 606. The variable consideration related to royalties will be recognized in the period the products are sold by Processa and the Company has a present right to payment.

For the year ended December 31, 2018, the Company recognized \$10.5 million in revenue related to the transfer of the license.

### 13. Loan Payable and Warrant to Purchase Redeemable Securities

On June 8, 2017, the Company entered into a Loan Agreement with Hercules, pursuant to which Hercules agreed to make available to the Company a Term Loan Facility in the amount of \$30.0 million, subject to certain terms and conditions. The Company borrowed \$30.0 million under the Loan Agreement in one advance. The Company incurred \$0.3 million in loan issuance costs paid directly to the lenders, which was offset against the loan proceeds as a loan discount.

The advance under the Loan and Security Agreement bore interest at a variable rate of the greater of 8.55% and an amount equal to 8.55% plus the prime rate of interest minus 4.50%. Through September 7, 2017, the Term Loan Facility had an interest rate of 8.55%. Pursuant to the Loan Agreement, the Company had the option to prepay the principal of the Loan Agreement at any time subject to a prepayment charge; however the prepayment charge was waived upon the completion of the sale of CTP-656 to Vertex, discussed further in Note 12, and the prepayment of the Term Loan Facility after the 90th day following the closing date of the Loan Agreement but prior to the six month anniversary of the closing date of the Loan Agreement.

On September 7, 2017, the Company paid a total of \$30.8 million to Hercules, representing the principal, accrued and unpaid interest, fees, costs and expenses outstanding under the Loan Agreement. The payoff amount included a final end of term charge to Hercules in the amount of \$0.7 million, reduced from the \$1.5 million end of term charge required had the debt been outstanding to maturity. Upon the payment of the \$30.8 million pursuant to a payoff letter between the Company and Hercules, all outstanding indebtedness and obligations of the Company owed to Hercules under the Loan Agreement were paid in full, and the Loan Agreement was terminated. As a result of the debt

extinguishment, the Company recognized a loss of \$1.4 million during the year ended December 31, 2017.

In connection with the entry into the Loan Agreement, the Company issued warrants (the "Warrants") to certain entities affiliated with Hercules, exercisable for an aggregate of 61,273 shares of the Company's common stock at an exercise price of \$12.24 per share. The Warrants have a five year term, expiring June 8, 2022, and may be exercised on a cashless basis. The Hercules Warrants had a total relative fair value of \$0.5 million upon issuance and were recorded as a debt discount.

Pursuant to ASC 480, Distinguishing Liabilities from Equity and ASC 815, Derivatives and Hedging, the Warrants were classified as equity and were initially measured at relative fair value. Subsequent changes to fair value will not be recognized so long as the instrument continues to be equity classified. To determine the relative fair value, the Company measured the fair value of the Warrants as of June 8, 2017 using the Black-Scholes-Merton option pricing model. The significant assumptions

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CONCERT PHARMACEUTICALS, INC.  
NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

used in estimating the fair value of the Warrants include the volatility of the stock underlying the warrants, risk-free interest rate, and estimated life of the warrant. The Company used the following weighted-average assumptions:

|                          |         |
|--------------------------|---------|
| Expected volatility      | 73.71 % |
| Expected term (in years) | 5       |
| Risk-free interest rate  | 1.75 %  |
| Expected dividend yield  | — %     |

Consistent with the Company's weighted-average assumptions used in determining the fair value of options, expected volatility was estimated using a weighted-average of the Company's historical volatility of its common stock and the historical volatility of the common stock of a group of similar companies that were publicly traded.

#### 14. Disgorgement of Profits

On December 28, 2017, the Company received \$3.6 million due to a disgorgement of short-swing profits arising from the sales of the Company's stock by a greater than 10% stockholder pursuant to Section 16(b) of the Securities and Exchange Act of 1934. The funds disgorged to the Company were based on a formulaic computation as proscribed by the 1934 Act as a result of security activities that generally fall under the Section 16(b) rules.

The sales of the Company's stock was conducted without the knowledge of the Company, and the disgorgement profits were unrelated to the Company's primary business operations. Furthermore, under Section 16(b) of the Securities and Exchange Act of 1934, the Company was legally entitled to receive the disgorged profits without any corresponding obligations owed by the Company and no shares or other benefits were given to BVF by the Company in exchange for the disgorgement proceeds. As a result, the disgorgement receipt was recognized in other income for the fiscal year ended December 31, 2017.

#### 15. 401(k) Retirement Plan

In January 2008, the Company established the Concert Pharmaceuticals 401(k) Retirement Plan (the 401(k) Plan) in which substantially all of its permanent employees are eligible to contribute a percentage of base wages up to an amount not to exceed an annual statutory maximum. The Company matches 50% of the first 6% of an employee's contributions subject to statutory limits.

The Company made matching contributions under the 401(k) Plan of \$0.3 million, \$0.3 million and \$0.3 million for the years ended December 31, 2018, 2017 and 2016, respectively.

#### 16. Quarterly Financial Information (unaudited)

|  | Three Months Ended                    |                  |                       |                      |
|--|---------------------------------------|------------------|-----------------------|----------------------|
|  | March 31,<br>2018                     | June 30,<br>2018 | September 30,<br>2018 | December 31,<br>2018 |
|  | (in thousands, except per share data) |                  |                       |                      |
|  | (unaudited)                           |                  |                       |                      |
| Revenue                                | \$10,479                              | \$2              | \$ 11                 | \$ 13                |
| Operating expenses                     | 14,286                                | 14,376           | 17,351                | 20,076               |
| Loss from operations                   | (3,807 )                              | (14,374 )        | (17,340 )             | (20,063 )            |
| Other (expense) income, net            | (656 )                                | 1,329            | (29 )                 | (771 )               |
| Provision for income taxes             | —                                     | (280 )           | (18 )                 | (15 )                |
| Net loss                               | \$(4,463 )                            | \$(13,325 )      | \$(17,387 )           | \$(20,849 )          |
| Net loss per share - basic and diluted | \$(0.19 )                             | \$(0.57 )        | \$(0.74 )             | \$(0.89 )            |



CONCERT PHARMACEUTICALS, INC.  
NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

|                                      | Three Months Ended                                   |                  |                       |                      |
|--------------------------------------|--|------------------|-----------------------|----------------------|
|                                      | March 31,<br>2017                                    | June 30,<br>2017 | September 30,<br>2017 | December 31,<br>2017 |
|                                      | (in thousands, except per share data)<br>(unaudited) |                  |                       |                      |
| Revenue                              | \$20   | \$15             | \$ 143,844            | \$ 12                |
| Operating expenses                   | 13,490   | 12,992           | 12,011                | 12,749               |
| Income (Loss) from operations        | (13,470 )  | (12,977 )        | 131,833               | (12,737 )            |
| Other income (expense), net          | 137  | (50 )            | (1,591 )              | 4,194                |
| (Provision) Benefit for income taxes | —  | —                | (2,177 )              | 2,477                |
| Net income (loss)                    | \$(13,333)   | \$(13,027)       | \$ 128,065            | \$ (6,066 )          |
| Net income (loss) per share—basic    | \$(0.60 )  | \$(0.58 )        | \$ 5.61               | \$ (0.26 )           |
| Net income (loss) per share—diluted  | \$(0.60 )  | \$(0.58 )        | \$ 5.44               | \$ (0.26 )           |

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

The term “disclosure controls and procedures,” as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended, or the Exchange Act, refers to controls and procedures that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in the Securities and Exchange Commission’s rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to the company’s management, including its principal executive and principal financial officers, or persons performing similar functions, as appropriate to allow timely decisions regarding required disclosure. Our management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and our management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Our disclosure controls and procedures are designed to provide reasonable assurance of achieving their control objectives. Our management, with the participation of our Chief Executive Officer and Chief Financial Officer, has evaluated the effectiveness of our disclosure controls and procedures as of December 31, 2018, the end of the period covered by this Annual Report on Form 10-K. Based upon such evaluation, our Chief Executive Officer and Chief Financial Officer have concluded that our disclosure controls and procedures were effective at the reasonable assurance level as of such date.

Management’s Report on Internal Control Over Financial Reporting

Our management is responsible for establishing and maintaining adequate internal control over financial reporting. Internal control over financial reporting is defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act as a process designed by, or under the supervision of, a company’s principal executive officer and principal financial officer, or persons performing similar functions, and effected by a company’s board of directors, management, and other personnel, 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 and includes those policies and procedures that:

pertain to the maintenance of records that in reasonable detail accurately and fairly reflect the transactions and dispositions of a company’s assets;

provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that a company's receipts and expenditures are being made only in accordance with authorizations of the company's management and directors; and provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use or disposition of our assets that could have a material effect on the financial statements.

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Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. 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.

Under the supervision of and with the participation of our principal executive officer and principal financial officer, our management assessed the effectiveness of our internal control over financial reporting as of December 31, 2018 based on the criteria set forth by the Committee of Sponsoring Organizations of the Treadway Commission (COSO) in Internal Control—Integrated Framework (2013 framework). Based on this assessment, management concluded that our internal control over financial reporting was effective as of December 31, 2018.

#### Changes in Internal Control over Financial Reporting

There was no change in our internal control over financial reporting that occurred during the three months ended December 31, 2018 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

#### ITEM 9B. Other Information

None.

Part III

Item 10. Directors, Executive Officers and Corporate Governance

The information required by Item 10 will be included in our definitive proxy statement relating to our 2019 Annual Meeting of Stockholders, to be filed no later than 120 days after December 31, 2018, and is incorporated herein by reference.

Item 11. Executive Compensation

The information required by Item 11 will be included in our definitive proxy statement relating to our 2019 Annual Meeting of Stockholders, to be filed no later than 120 days after December 31, 2018, and is incorporated herein by reference.

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

The information required by Item 12 will be included in our definitive proxy statement relating to our 2019 Annual Meeting of Stockholders, to be filed no later than 120 days after December 31, 2018, and is incorporated herein by reference.

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

The information required by Item 13 will be included in our definitive proxy statement relating to our 2019 Annual Meeting of Stockholders, to be filed no later than 120 days after December 31, 2018, and is incorporated herein by reference.

Item 14. Principal Accountant Fees and Services

The information required by Item 14 will be included in our definitive proxy statement relating to our 2019 Annual Meeting of Stockholders, to be filed no later than 120 days after December 31, 2018, and is incorporated herein by reference.

Part IV

Item 15. Exhibits and Financial Statement Schedules

(1) Financial Statements

Our consolidated financial statements are set forth in Part II, Item 8 of this Annual Report on Form 10-K and are incorporated herein by reference.

(2) Financial Statement Schedules

Schedules have been omitted since they are either not required or not applicable or the information is otherwise included herein.

(3) Exhibits

The exhibits filed as part of this Annual Report on Form 10-K are listed below.

Item 16. Form 10-K Summary

Not applicable.



Exhibit Index

| Exhibit number | Description   |
|----------------|---|
| 3.1            | <u>Restated Certificate of Incorporation of the Registrant (incorporated by reference to Exhibit 3.1 to the Registrant's current report on Form 8-K (File No. 001-36310) filed with the SEC on February 20, 2014)</u>   |
| 3.2            | <u>Amended and Restated Bylaws of the Registrant (incorporated by reference to Exhibit 3.2 to the Registrant's current report on Form 8-K (File No. 001-36310) filed with the SEC on February 20, 2014)</u>   |
| 3.3            | <u>Amendment to Amended and Restated Bylaws of the Registrant (incorporated by reference to Exhibit 3.3 to the Registrant's previous Annual Report on Form 10-K (File No. 001-36310), filed with the SEC on March 6, 2017)</u>  |
| 4.1            | <u>Specimen certificate evidencing shares of common stock (incorporated by reference to Exhibit 4.1 to the Registrant's registration statement on Form S-1 (File No. 333-193335), filed with the SEC on February 3, 2014)</u>   |
| 10.1           | <u>Third Amended and Restated Registration Rights Agreement, dated as of June 1, 2009, as amended (incorporated by reference to Exhibit 10.1 to the Registrant's registration statement on Form S-1 (File No. 333-193335), filed with the SEC on January 13, 2014)</u>  |
| 10.2           | <u>Warrant to purchase shares of Series C Convertible Preferred Stock issued by the Registrant to Hercules Technology Growth Capital, Inc. (incorporated by reference to Exhibit 10.2 to the Registrant's registration statement on Form S-1 (File No. 333-193335), filed with the SEC on January 13, 2014)</u> |
| 10.3 #         | <u>Amended and Restated 2006 Stock Option and Grant Plan, as amended (incorporated by reference to Exhibit 10.3 to the Registrant's registration statement on Form S-1 (File No. 333-193335), filed with the SEC on January 13, 2014)</u>   |
| 10.4 #         | <u>Form of Incentive Stock Option Agreement under 2006 Stock Option and Grant Plan (incorporated by reference to Exhibit 10.4 to the Registrant's registration statement on Form S-1 (File No. 333-193335), filed with the SEC on January 13, 2014)</u>   |
| 10.5 #         | <u>Form of Nonstatutory Stock Option Agreement under 2006 Stock Option and Grant Plan (incorporated by reference to Exhibit 10.5 to the Registrant's registration statement on Form S-1 (File No. 333-193335), filed with the SEC on January 13, 2014)</u>  |
| 10.6 #         | <u>2014 Stock Incentive Plan (incorporated by reference to Exhibit 10.6 to the Registrant's registration statement on Form S-1 (File No. 333-193335), filed with the SEC on February 3, 2014)</u>   |
| 10.7 #         | <u>Form of Incentive Stock Option Agreement under 2014 Stock Incentive Plan (incorporated by reference to Exhibit 10.7 to the Registrant's registration statement on Form S-1 (File No. 333-193335), filed with the SEC on February 3, 2014)</u>  |
| 10.8 #         | <u>Form of Nonstatutory Stock Option Agreement under 2014 Stock Incentive Plan (incorporated by reference to Exhibit 10.8 to the Registrant's registration statement on Form S-1 (File No. 333-193335), filed with the SEC on February 3, 2014)</u>   |

- 10.11 # Form of Restricted Stock Unit Award Granted under 2014 Stock Incentive Plan (incorporated by reference to Exhibit 10.1 to the Registrant's Current Report on Form 8-K (File No. 001-36310), filed with the SEC on July 10, 2017)
- 10.12 Asset Purchase Agreement, dated March 3, 2017, by and between the Registrant and Vertex Pharmaceuticals (Europe) Ltd., as Buyer, and Vertex Pharmaceuticals Inc., as Guarantor (incorporated by reference to Exhibit 10.12 to the Registrant's previous Annual Report on Form 10-K (File No. 001-36310), filed with the SEC on March 6, 2017)
- 10.13 # Form of Director and Officer Indemnification Agreement (incorporated by reference to Exhibit 10.13 to the Registrant's registration statement on Form S-1 (File No. 333-193335), filed with the SEC on January 13, 2014)

| Exhibit number | Description   |
|----------------|---|
| 10.14          | <u>Lease Agreement, dated as of February 12, 2008, by and between the Registrant and One Ledgemont LLC (incorporated by reference to Exhibit 10.15 to the Registrant's registration statement on Form S-1 (File No. 333-193335), filed with the SEC on January 13, 2014)</u>  |
| 10.15          | <u>Amendment of Lease, dated as of August 6, 2014, by and between the Registrant and 128 Spring Street Lexington, LLC (incorporated by reference to Exhibit 10.4 to the Registrant's quarterly report on Form 10-Q (File No. 001-36310), filed with the SEC on August 12, 2014)</u>   |
| 10.16 †        | <u>Development and License Agreement, dated as of February 24, 2012, between the Registrant and Avanir Pharmaceuticals, Inc. (incorporated by reference to Exhibit 10.16 to the Registrant's registration statement on Form S-1 (File No. 333-193335), filed with the SEC on February 3, 2014)</u>  |
| 10.17 †        | <u>Development and License Agreement, dated as of February 26, 2013, between the Registrant and Jazz Pharmaceuticals Ireland Limited (incorporated by reference to Exhibit 10.17 to the Registrant's registration statement on Form S-1 (File No. 333-193335), filed with the SEC on February 3, 2014)</u>                                  |
| 10.18 †        | <u>Amendment No. 1, dated February 26, 2015, to Development and License Agreement dated February 26, 2013 by and between the Registrant and Jazz Pharmaceuticals Ireland Limited (incorporated by reference to Exhibit 10.1 to the Registrant's quarterly report on Form 10-Q (File No. 001-36310), filed with the SEC on May 11, 2015)</u> |
| 10.19 †        | <u>Master Development and License Agreement, dated as of April 4, 2013, among the Registrant, Celgene International Sàrl and Celgene Corporation (incorporated by reference to Exhibit 10.18 to the Registrant's registration statement on Form S-1 (File No. 333-193335), filed with the SEC on February 3, 2014)</u>                      |
| 10.20 †        | <u>Patent Assignment Agreement, dated September 8, 2011, by and between the Registrant and Auspex Pharmaceuticals, Inc. (incorporated by reference to Exhibit 10.2 to the Registrant's quarterly report on Form 10-Q (File No. 001-36310), filed with the SEC on May 11, 2015)</u>  |
| 10.21 #        | <u>Summary of Executive Bonus Program (incorporated by reference to Exhibit 10.19 to the Registrant's registration statement on Form S-1 (File No. 333-193335), filed with the SEC on January 13, 2014)</u>   |
| 10.22          | <u>Lease Agreement, dated as of December 21, 2017, by and between the Registrant and HCP/King Hayden Campus LLC (incorporated by reference to Exhibit 10.10 to the Registrant's annual report on Form 10-K (File No. 001-36310), filed with the SEC on March 1, 2018)</u>   |
| 10.23 #*       | <u>Summary of Director Compensation Program</u>   |
| 21.1*          | <u>Subsidiaries of the Registrant</u>   |
| 23.1*          | <u>Consent of Ernst &amp; Young LLP</u>   |
| 31.1*          |   |

Chief Executive Officer—Certification pursuant to Rule 13a-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002

31.2\* Chief Financial Officer—Certification pursuant to Rule 13a-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002

32.1\*\* Chief Executive Officer—Certification pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002

32.2\*\* Chief Financial Officer—Certification pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002

| Exhibit number | Description  |
|----------------|--|
| 101.INS*       | XBRL Instance Document                                 |
| 101.SCH*       | XBRL Taxonomy Extension Schema Document                |
| 101.CAL*       | XBRL Taxonomy Extension Calculation Linkbase Document  |
| 101.DEF*       | XBRL Taxonomy Extension Definition Linkbase Document   |
| 101.LAB*       | XBRL Taxonomy Extension Label Linkbase Document        |
| 101.PRE*       | XBRL Taxonomy Extension Presentation Linkbase Document |

\*Filed herewith.

\*\*Furnished herewith.

Confidential treatment requested as to certain portions, which portions have been omitted and filed separately with the Securities and Exchange Commission.

# Management contracts or compensatory plans or arrangements required to be filed as an exhibit hereto pursuant to Item 15(a) of Form 10-K.

#### SIGNATURES

Pursuant to the requirements of Section 13 or 15(d) of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized, on February 28, 2019.

CONCERT PHARMACEUTICALS, INC.

By: /s/ Roger D. Tung  
Roger D. Tung, Ph.D.  
President and Chief Executive Officer

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



| Signature  | Title   | Date              |
|--|---|-------------------|
| /s/ Roger D. Tung<br>Roger D. Tung, Ph.D.          | Director, President and Chief Executive Officer (Principal Executive Officer) | February 28, 2019 |
| /s/ Marc Becker<br>Marc Becker                     | Chief Financial Officer (Principal Financial Officer)                         | February 28, 2019 |
| /s/ Ryan Lynch<br>Ryan Lynch                       | Corporate Controller (Principal Accounting Officer)                           | February 28, 2019 |
| /s/ Richard H. Aldrich<br>Richard H. Aldrich       | Chairman  | February 28, 2019 |
| /s/ Thomas G. Auchincloss<br>Thomas G. Auchincloss | Director  | February 28, 2019 |
| /s/ Ronald W. Barrett<br>Ronald W. Barrett, Ph.D.  | Director  | February 28, 2019 |
| /s/ Christine van Heek<br>Christine van Heek       | Director  | February 28, 2019 |
| /s/ Peter Barton Hutt<br>Peter Barton Hutt         | Director  | February 28, 2019 |
| /s/ Wilfred E. Jaeger<br>Wilfred E. Jaeger, M.D.   | Director  | February 28, 2019 |
| /s/ Wendell Wierenga<br>Wendell Wierenga, Ph.D.    | Director  | February 28, 2019 |