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

FORM 10-K

(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, 2017

OR

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

For the transition period from _____to_____

Commission file number: 001-38219

Deciphera Pharmaceuticals, Inc.
(Exact name of registrant as specified in its charter)

Delaware	**30-1003521**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification Number)**
500 Totten Pond Road Waltham, MA (Address of principal executive offices)	**02451 (Zip Code)**

(781) 209-6400

(Registrant's telephone number, including area code)

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

Title of each class	**Name of exchange on which registered**
Common Stock, $0.01 Par Value	**The NASDAQ Global Select 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 and posted on its corporate Web site, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). Yes ☒ No ☐

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

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, 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	☒ (Do not check if a smaller reporting company)	Smaller reporting company	☐
Emerging growth company	☒		

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

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

As of June 30, 2017, the last business day of the registrant's most recently completed second fiscal quarter, there was no established public market for the registrant's Common Stock. The registrant's Common Stock began trading on the NASDAQ Global Select Market on September 28, 2017. The aggregate market value of Common Stock held by non-affiliates of the registrant computed by reference to the price of the registrant's Common Stock as of September 28, 2017 (based on the last reported sale price on the NASDAQ Global Select Market as of such date) was $147.6 million. As of February 28, 2018 there were 32,594,128 shares of Common Stock, $0.01 par value per share, outstanding.

DOCUMENTS INCORPORATED BY REFERENCE

Portions of the registrant's Proxy Statement for its 2018 Annual Meeting of Stockholders, which the registrant intends to file pursuant to Regulation 14A with the Securities and Exchange Commission not later than 120 days after the registrant's fiscal year end December 31, 2017, are incorporated by reference into Part III of this Annual Report on Form 10-K.

Deciphera Pharmaceuticals Inc.
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FORWARD-LOOKING STATEMENTS

This Annual Report on Form 10-K contains forward-looking statements, which reflect our current views with respect to, among other things, our operations and financial performance. All statements other than statements of historical facts contained in this Annual Report on Form 10-K, including statements regarding our strategy, future operations, future financial position, future revenue, projected costs, prospects, plan, objectives of management and expected market growth are forward-looking statements. You can identify these forward-looking statements by the use of words such as “outlook,” “believes,” “expects,” “potential,” “continues,” “may,” “will,” “should,” “seeks,” “approximately,” “predicts,” “intends,” “plans,” “estimates,” “anticipates” or the negative version of these words or other comparable words. Such forward-looking statements are subject to various risks and uncertainties. Accordingly, there are or will be important factors that could cause actual outcomes or results to differ materially from those indicated in these statements. We believe these factors include but are not limited to those described under “Risk Factors” and include, among other things:

- the success, cost and timing of our product development activities and clinical trials, including the timing of our second planned Phase 3 trial for DCC-2618 in GIST;
- our ability to obtain and maintain regulatory approval for DCC-2618 or any of our other current or future drug candidates, and any related restrictions, limitations, and/or warnings in the label of an approved drug candidate;
- our expectations regarding the size of target patient populations for our drug candidates, if approved for commercial use, and any additional drug candidates we may develop;
- our ability to obtain funding for our operations;
- our ability to manufacture sufficient quantities of DCC-2618 to support our planned clinical trials and, if approved, commercialization;
- the commercialization of our drug candidates, if approved;
- our plans to research, develop and commercialize our drug candidates, including the timing of our second planned Phase 3 trial for DCC-2618 in GIST;
- our ability to attract collaborators with development, regulatory and commercialization expertise;
- our expectations regarding our ability to obtain, maintain, enforce and defend our intellectual property protection for our drug candidates;
- future agreements with third parties in connection with the commercialization of DCC-2618, or any of our other current or future drug candidates;
- the size and growth potential of the markets for our drug candidates, and our ability to serve those markets;
- the rate and degree of market acceptance of our drug candidates, as well as the reimbursement coverage for our drug candidates;
- regulatory and legal developments in the United States and foreign countries;
- the performance of our third-party suppliers and manufacturers;
- the success of competing therapies that are or may become available;
- our ability to attract and retain key scientific or management personnel;
- the accuracy of our estimates regarding expenses, future revenues, capital requirements and needs for additional financing;
- our expectations regarding the period during which we qualify as an emerging growth company under the JOBS Act; and
- our use of the proceeds from our initial public offering.

These factors should not be construed as exhaustive and should be read in conjunction with the other cautionary statements that are included in this Annual Report on Form 10-K. You should read this Annual Report on Form 10-K and the documents that we have filed as exhibits to this Annual Report on Form 10-K completely and with the understanding that our actual future results may be materially different from what we expect. The forward-looking statements contained in this Annual Report on Form 10-K are made as of the date of this Annual Report on Form 10-K, and we undertake no obligation to publicly update or review any forward-looking statement, whether as a result of new information, future developments or otherwise.

PART I

Except where the context otherwise requires or where otherwise indicated, the terms "Deciphera," "we," "us," "our," "our company," "the company," and "our business" refer to Deciphera Pharmaceuticals, Inc. and its consolidated subsidiaries.

ITEM 1. BUSINESS

Overview

We are a clinical-stage biopharmaceutical company developing new drugs to improve the lives of cancer patients by addressing key mechanisms of drug resistance that limit the rate and durability of response of many cancer therapies. Our targeted, small molecule drug candidates, designed using our proprietary kinase switch control inhibitor platform, inhibit the activation of kinases, an important family of enzymes, that when mutated or over expressed, are known to be directly involved in the growth and spread of many cancers. We have built a diverse pipeline of wholly owned, orally administered drug candidates that includes three clinical-stage and two research-stage programs. We are studying our lead drug candidate DCC-2618 in an ongoing pivotal Phase 3 trial in fourth-line plus treatment of gastrointestinal stromal tumors, or GIST, where there are currently no approved therapies, and in an ongoing Phase 1 trial in patients with advanced malignancies. We presented interim results from this Phase 1 trial in September 2017 at the European Society for Medical Oncology 2017 Congress, or the ESMO 2017 Congress, that demonstrate clinical proof-of-concept at well tolerated doses in 57 heavily pre-treated GIST patients, of which 51 had KIT- or PDGFRα-driven GIST. We are currently enrolling expansion cohorts in this Phase 1 trial to study DCC-2618 in patients with different stages of GIST, as well as in patients with advanced systemic mastocytosis, or ASM, gliomas, including glioblastoma multiforme, or GBM, and other solid tumors driven by KIT or PDGFRα. We expect to report initial data from some of these expansion cohorts in 2018. We expect to initiate enrollment in a second pivotal Phase 3 trial in second-line GIST comparing DCC-2618 to sunitinib in the second half of 2018. Initiation of our Phase 3 trial in second-line GIST is subject to data from our ongoing Phase 1 trial and the U.S. Food and Drug Administration, or FDA, and the European Medicines Agency, or EMA, feedback on the study protocol. We are also developing two other clinical-stage, small molecule drug candidates, DCC-3014 and rebastinib, as immuno-oncology kinase, or immunokinase, switch control inhibitors targeting colony stimulating factor receptor 1, or CSF1R, and TIE2 kinase, respectively. Both drug candidates are in Phase 1 trials. We believe our proprietary kinase switch control inhibitor platform, supported by our experienced management team, enables us to develop advanced, differentiated, kinase inhibitors that may provide significant benefits to cancer patients.

As presented at the ESMO 2017 Congress, in our ongoing Phase 1 trial of DCC-2618, in GIST patients shown to harbor a broad range of KIT and PDGFRα mutations who received at least 100 mg of DCC-2618 daily, we observed 91% (30 of 33 evaluable patients) had a best response of stable disease or partial response, or PR (defined as tumor size reduction of 30% or more), by Response Evaluation Criteria in Solid Tumors, or RECIST. In addition, we observed a disease control rate, or DCR, defined as patients with either stable disease or a PR, of 76% at 12 weeks in 25 patients and 57% at 24 weeks in 21 patients. Disease control includes stable disease, PRs and complete responses measured by computerized tomography, or CT scan, or magnetic resonance imaging, or MRI scan, and assessed by RECIST. DCR is the proportion of treated patients that exhibit disease control at a point in time. Based on the results presented at the 2017 American Society of Clinical Oncology Annual Meeting, or the 2017 ASCO Meeting, and analysis of the plasma drug exposure achieved at the various doses and regimens administered, we have selected a dose of 150 mg once daily, or QD, for our DCC-2618 pivotal trials.

Kinase inhibitors have become an important class of cancer therapies. Since the first FDA approval of a kinase inhibitor in 2001, a total of 39 kinase inhibitors have been approved in the United States for the treatment of cancer, and kinase inhibitor drugs represented approximately $20 billion in 2016 worldwide pharmaceutical sales. Despite the success of this drug class, there remains a significant opportunity for advanced kinase inhibitors that address the shortcomings of current therapies, including limited durability of response caused by

development of resistance mutations and off-target toxicities that limit dose and, consequently, target inhibition. In addition, currently approved kinase inhibitors target fewer than 10% of the over 500 known human kinases. There remains a substantial opportunity to develop novel inhibitors that target therapeutically relevant kinases.

Our proprietary kinase switch control inhibitor platform combines our deep insight into the biology of kinases with our library of drug-like compounds that we specifically designed to interact with a specific region of the kinase called the switch pocket. The transformation of a kinase from a switched-off, or inactivated, state to a switched-on, or activated, state is dependent upon the interaction of one region of the kinase called the activation switch with another region called the switch pocket. The interaction between the activation switch and the switch pocket is a common mechanism among all kinases; however, the molecular structure of the activation switch and the switch pocket varies among kinases allowing for the rational design of molecules that inhibit a specific kinase or specific kinases.

Our drug candidates directly target the conformation-controlling switch that kinases rely on for activation and inhibit the kinase from switching on. We believe that no kinase inhibitors on the market or active in clinical development directly target the switch pocket region. By using our proprietary approach to target the switch pocket, we can design inhibitors that are more broadly active against the target kinase, covering both wild-type, or non-mutant, and mutant forms, or that are spectrum-selective against several chosen kinases, all while minimizing off-target toxicity. We believe that our drug candidates will contribute to higher and more durable rates of response compared to other kinase inhibitors even upon accumulation of mutations that would render the kinase resistant to other kinase inhibitors. Our drug candidates bind directly into the switch pocket at the site where the activation switch binds. As a result, we believe that any mutations that occur in the switch pocket region that would diminish the activity of our drug candidates likely also would produce a weakly activated or inactive kinase.

We believe the results from the Phase 1 trial of our lead drug candidate DCC-2618 provide strong evidence of the power of our proprietary kinase switch control inhibitor platform. Patients enrolled in this trial have advanced malignancies and generally have been treated previously with a series of three or more kinase inhibitors. While the kinase inhibitors these patients have been treated with target some clinically relevant initiating, or primary, mutations in KIT and PDGFRα and drug resistance-causing, or secondary, mutations in KIT, they fail to inhibit all primary and secondary mutations in these kinases involved in GIST. As a result, almost all patients treated with these kinase inhibitors eventually suffer from disease progression. We designed our lead drug candidate DCC-2618 to inhibit the full spectrum of the known mutant or amplified KIT and PDGFRα kinases that drive cancers such as GIST.

In addition to DCC-2618, we are developing two other clinical-stage drug candidates using our platform, DCC-3014 and rebastinib. These drug candidates target immunokinases involved in the suppression of the immune response to tumors. DCC-3014 is a potent and highly selective inhibitor of CSF1R, a kinase that controls the survival and function of certain immunosuppressive tumor associated macrophages, or TAMs. Mutations in CSF1R also are associated with certain hematological malignancies including chronic myelomonocytic leukemia and acute myeloblastic leukemia. In February 2017, we initiated a Phase 1 dose escalation trial of DCC-3014 in up to 55 patients with advanced malignancies, including solid or hematologic malignancies where CSF1R is known or suspected to contribute to the growth or spread of cancer. We expect to report data from this Phase 1 trial in the second half of 2018. We also plan to explore DCC-3014 in combination with other immuno-oncology, or I/O, therapies. Rebastinib is an orally administered and highly potent and selective inhibitor of TIE2, which plays an important role in regulating tumor angiogenesis, invasiveness, metastasis, and immunotolerance. We plan to investigate rebastinib in combination with chemotherapy and/or checkpoint inhibitors. Rebastinib is currently in an investigator sponsored Phase 1b combination trial with paclitaxel or eribulin in patients with advanced breast cancer.

We have assembled a management team with extensive experience in the discovery, development and commercialization of cancer therapeutics, including in senior roles at leading pharmaceutical companies. We are

supported by our board of directors and scientific advisory board, who contribute their deep understanding of drug discovery and development, as well as expertise in building public companies and business development. We believe that our team is ideally positioned to leverage our highly differentiated platform to develop and commercialize advanced kinase inhibitors that will have significant benefit for cancer patients.

Our Strategy

Our objective is to develop and commercialize innovative drugs that address the serious unmet medical needs of cancer patients caused by drug resistance or immunotolerance. The principal components of our strategy include:

- ***Rapidly develop and commercialize our lead drug candidate, DCC-2618, in fourth-line plus GIST.*** We are currently advancing DCC-2618 through clinical development in multiple cancer types. We are initially targeting fourth-line plus GIST, a market opportunity where there are no approved therapies and a high unmet medical need. In January 2018, we announced the initiation of a pivotal Phase 3 trial in fourth-line plus GIST. We believe that this approach offers a faster, lower risk path to commercialization than initially seeking approval for DCC-2618 in second-line GIST, where there is already an approved therapy.
- ***Expand the market opportunity for DCC-2618 by pursuing development in second-line GIST, ASM, gliomas, including GBM, and other solid tumors driven by KIT or PDGFR***α. We plan to initiate a randomized, controlled, pivotal Phase 3 trial in second-line GIST comparing treatment with DCC-2618 to sunitinib, the currently approved standard of care in second-line GIST, in the second half of 2018. Based upon the activity observed in the expansion cohorts of our Phase 1 trial of DCC-2618, we may conduct additional pivotal trials in ASM, gliomas, including GBM, and other solid tumors driven by KIT or PDGFRα. We believe that this approach offers an opportunity to significantly expand the commercial potential of DCC-2618 over time.
- ***Develop our immunokinase inhibitors, DCC-3014 and rebastinib, as combination therapies.*** We believe kinase inhibitors have potential application in combination with other anti-cancer therapies due to their anticipated synergistic effect with other immuno-oncology therapies, such as anti-PD1 and anti-PD-L1 therapies, as well as chemotherapy. DCC-3014 is currently in a Phase 1 trial in patients with advanced solid and hematologic malignancies, and we expect to report data from this trial in the second half of 2018. Rebastinib is currently in an investigator-sponsored Phase 1b combination trial with paclitaxel or eribulin in patients with advanced breast cancer. Based on data combining DCC-3014 or rebastinib with anti-PD1 antibodies or anti-tubulin chemotherapy in preclinical studies, we are evaluating opportunities for further development of these drug candidates in combination with other I/O therapies or chemotherapy.
- ***Expand the application of our proprietary kinase switch control inhibitor platform to advance additional drug candidates into clinical development.*** We believe there is a significant opportunity to utilize our kinase switch control inhibitor platform to discover and develop novel kinase inhibitor drug candidates that are directed to other tumor-targeted kinases, immunokinases and kinases critical to other mechanisms that contribute to the growth and spread of cancer. We believe that our platform allows us to identify drug candidates that address mechanisms of drug resistance that limit the clinical utility of many kinase inhibitors. We also believe that our drug candidates should exhibit greater resilience to resistance mutations, offer improved kinase selectivity, or both, compared to existing kinase inhibitors. We are also advancing the preclinical development of additional programs and expect to initiate advanced preclinical studies in one of these programs in 2018.
- ***Evaluate strategic opportunities to accelerate development timelines and maximize the commercial potential of our drug candidates.*** We currently have worldwide rights to all of our drug candidates. We intend to selectively evaluate strategic partnerships for our drug candidates with partners whose development and commercial capabilities complement our own. With respect to our immunokinase

inhibitor drug candidates, where use in combination with other I/O therapies will be an important driver of commercial value, we believe that strategic partnerships are an effective means of developing and commercializing this class of drug candidates.

- ***Establish capabilities to effectively commercialize our drug candidates in the United States.*** We intend to retain commercial rights to our drug candidates in the United States. We intend to build a targeted, specialty sales force in the United States to support the commercialization of DCC-2618 and our other drug candidates, if approved.

Kinases and their Role in Cancer

Kinases play an important role in regulating cellular functions and the communication of cells with their environments. When dysregulated, kinases contribute to the development and progression of diseases including cancer and inflammatory and autoimmune diseases. Since the first FDA approval of the kinase inhibitor imatinib in 2001, kinase inhibitors have become an important class of therapeutics with 39 kinase inhibitors approved in the United States. Kinase inhibitors represented approximately $20 billion in 2016 worldwide pharmaceutical sales. Despite the success of kinase inhibitors as a drug class, the therapeutic potential of individual kinase inhibitors has been limited by the development of drug resistance and by poor potency and selectivity profiles that lead to off-target toxicities or diminished efficacy. In addition, currently approved kinase inhibitors target fewer than 10% of the over 500 known human kinases. We believe there is a substantial opportunity to develop novel kinase inhibitor therapies. Inhibitors of one class of kinases, the immunokinases, may represent a particularly promising approach to target key mechanisms of tumor immunotolerance that limit effectiveness of other I/O therapies.

Within almost all kinases, a molecular control known as the activation switch governs whether the kinase is in the inactive or the active state. Most of the time kinases are in an inactive state and are triggered into the active state when they are needed to direct normal cellular functions. Mutations within kinases, particularly those that involve the activation switch region, can cause uncontrolled kinase signaling within the cell. In addition, kinases may acquire further mutations during treatment with traditional kinase inhibitors that confer resistance to these kinase inhibitors. We designate the region of the gene that encodes the kinase, or exon, when referring to a particular mutation. Kinase activity also may be amplified through the aberrant development of multiple copies of the relevant gene. These aggressively activated mutated or amplified kinases can drive rapid, uncontrolled growth and spread of tumors.

In addition to mutated kinases, certain kinases known as immunokinases also play a role in the development of cancer through the suppression of the immune system. Tumors suppress immune system cells, such as macrophages and T-cells, essentially shutting off their ability to identify and destroy cancer cells. For instance, tumors may suppress the immune system by sending a signal that activates an immunokinase in immune system cells. The activated kinase then initiates internal signaling within the immune system cells to suppress their function and prevent them from identifying and destroying the cancer cells. A recent development in cancer therapy, referred to as immuno-oncology, uses advances in understanding of the control of the immune system to develop drugs that enhance the ability of immune system cells to recognize and attack cancer cells. Based on our preclinical data we believe inhibiting immunokinases has the potential to improve the rate and duration of response of other I/O therapies.

Our Approach: Kinase Switch Control Inhibitors

We created our diverse pipeline of drug candidates entirely in-house using our proprietary kinase switch control inhibitor platform. We developed our platform based on our deep insight into the biology of kinases, which are regulated by control of their shape, or conformation. The transformation of a kinase from an inactive to an activated state is dependent upon the interaction of one region of the kinase called the activation switch with another region called the switch pocket. This activation switch mechanism is common among kinases. Some

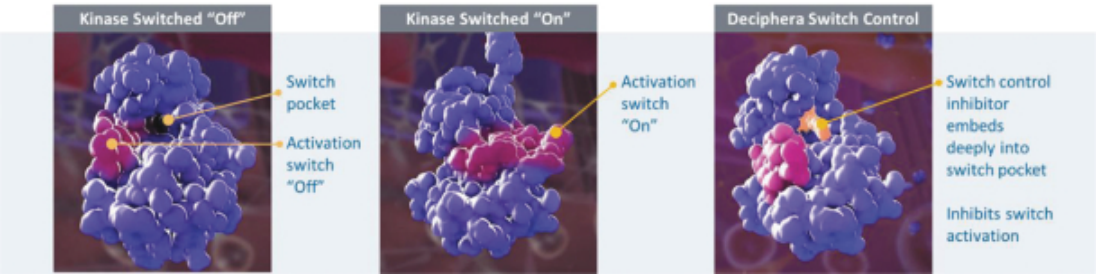
kinases also can be activated if the activity of an inhibitory switch that ordinarily blocks the ability of the activation switch to interact with the switch pocket is diminished or lost. Our drug candidates, which we refer to as kinase switch control inhibitors, directly interfere with the interaction between the activation switch and the switch pocket and prevent kinase activation. While the interaction between the activation switch and the switch pocket is common among kinases, the molecular structure of the activation switch and the switch pocket varies among kinases. We take advantage of this variation to design molecules that inhibit a specific kinase or kinases.

Our proprietary kinase switch control inhibitor platform includes a library of drug-like, kinase switch control compounds. We have determined and assessed more than 100 co-crystal structures where our compounds are bound into the switch pocket of specific kinases. We use this information to identify and optimize candidate molecules that are specifically designed to interact with the switch pocket. By directly targeting the switch pocket, we believe we can design inhibitors that will be broadly active against the target kinase, covering both wild-type and many or all of the known mutant or amplified forms, or spectrum-selective towards several chosen kinases.

We believe that other kinase inhibitors on the market or active in clinical development do not directly target the switch pocket region and that we are the only biopharmaceutical company that is currently developing kinase switch control inhibitors. Using our kinase switch control inhibitor platform, we have developed a diverse pipeline of differentiated, wholly owned, orally administered drug candidates that include three clinical-stage and two research-stage programs. Our kinase switch control inhibitors interact at a molecular level that is distinct from other kinase inhibitors. We believe our drug candidates may contribute to higher and more durable rates of response as compared to other kinase inhibitors, including where multiple mutations confer resistance to these other kinase inhibitors. In addition, because our drug candidates bind directly into the switch pocket at the site where the activation switch binds, we believe mutations in the switch pocket region that could potentially diminish the activity of our drug candidates are likely to result in a weakly activated or inactive kinase.

The image below illustrates activation of the switch pocket and how our switch control inhibitors embed into the switch pocket thereby inhibiting switch activation.

Mechanism of Kinase Switch Control Inhibition

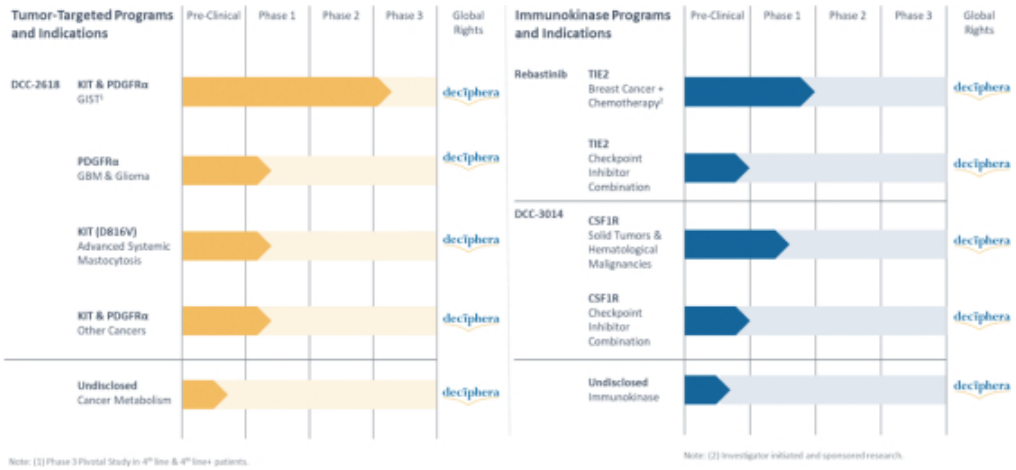


While we believe that our proprietary kinase switch control inhibitor platform offers the benefits described above, there are certain limitations of our platform, including its inability to control inhibition of certain kinases that interfere with access to the switch pocket, including cyclin dependent kinases and specific kinases in the MAPK family (MEK and ERK), which constitute less than 10% of the over 500 known human kinases as well as the inability of our laboratory assays to support high-throughput screening, resulting in limitations on the number of molecules that can be screened.

Our Drug Candidates

We are leveraging our proprietary kinase switch control inhibitor platform to develop a pipeline of highly selective, potent small molecule drug candidates that directly inhibit activation of kinases implicated in the

growth and spread of cancers. Our platform allows to us to rapidly identify new drug candidates to enter preclinical development. We currently retain global development and commercialization rights to our drug candidates, including the lead programs summarized in the following figure:



DCC-2618: A KIT and PDGFRα Inhibitor

We are developing our lead drug candidate DCC-2618, an orally administered kinase switch control inhibitor, for the treatment of GIST, ASM, gliomas, including GBM, and other solid tumors driven by KIT or PDGFRα where significant unmet medical need exists despite currently available therapies. While approved kinase inhibitors control certain initiating and drug resistance-causing mutations in KIT and PDGFRα, the kinases that drive disease progression in most GIST patients, these approved drugs fail to inhibit all known mutations. We designed DCC-2618 to improve the treatment of GIST patients by inhibiting the full spectrum of the known mutations in KIT and PDGFRα. DCC-2618 is a KIT and PDGFRα inhibitor that blocks initiating and resistance KIT mutations in exons 9, 11, 13, 14, 17, and 18 known to be present in GIST patients and the primary mutation in exon 17 that occurs in ASM patients. DCC-2618 similarly inhibits the primary initiating PDGFRα mutations occurring in exons 12 and 18 and also inhibits wild-type PDGFRα that is subject to amplification in cancers such as gliomas, including GBM.

In September 2017, we reported data from the ongoing Phase 1 trial evaluating the safety and tolerability of DCC-2618 in multiple ascending doses in patients with genetically defined advanced malignancies. A primary objective of the dose escalation stage of this trial was to determine a maximum tolerated dose, or MTD, and a recommended Phase 2 dose of oral DCC-2618. At the ESMO 2017 Congress, we reported updated safety data from the ongoing Phase 1 trial from a total of 70 patients. DCC-2618 was well tolerated at all doses up to 400 mg daily. In addition, we reported preliminary efficacy data from 57 patients with GIST who had received an average of 3.3 prior treatments (individual agents). In GIST patients shown to harbor a broad range of KIT and PDGFRα mutations who received at least 100 mg of DCC-2618 daily, we observed 91% (30 of 33 evaluable patients) had a best response of stable disease or PR by RECIST. In addition, we observed a DCR of 76% (19 of 25 patients) at 12 weeks and 57% (12 of 21 patients) at 24 weeks. The best response and DCRs described above are based on investigator assessment of tumor response in a limited number of patients and may not be predictive of or consistent with the results of later trials.

As of October 31, 2017, a total of 125 patients had been dosed with DCC-2618 of which 109 were GIST patients, including 54 GIST patients in three expansion cohorts of the Phase 1 trial, which are enrolling second-line, third-line, and fourth-to-fifth line GIST patients, respectively.

DCC-2618 Mechanism of Action

KIT is activated by the interaction of an activation switch encoded in exons 17 and 18 with the switch pocket. This interaction is negatively controlled by an inhibitory switch in exon 11 that competes for the switch pocket. Loss-of-function mutations in the exon 11 inhibitory switch, which are the primary, or activating, mutations in approximately 90% of KIT-driven GIST patients, allow uncontrolled access to the switch pocket by the activation switch. In drug resistant GIST, unregulated access of the activation switch to the switch pocket is further enhanced by gain-of-function mutations in exon 17 or 18. Such dual loss-of-function mutations in the inhibitory switch and gain-of-function mutation in the activation switch leads to an aggressively activated state that is not comprehensively blocked by conventional KIT inhibitors. By binding into the switch pocket, DCC-2618 provides a structural surrogate for loss-of-function mutations in the exon 11 inhibitory switch. Additionally, binding of DCC-2618 into the switch pocket inhibits even the most aggressive exons 17 and 18 gain-of-function mutation in the activation switch from occupying the switch pocket. This dual mechanism of kinase switch control also enables DCC-2618 to potently inhibit KIT exons 9, 13, and 14 mutations identified in some GIST patients.

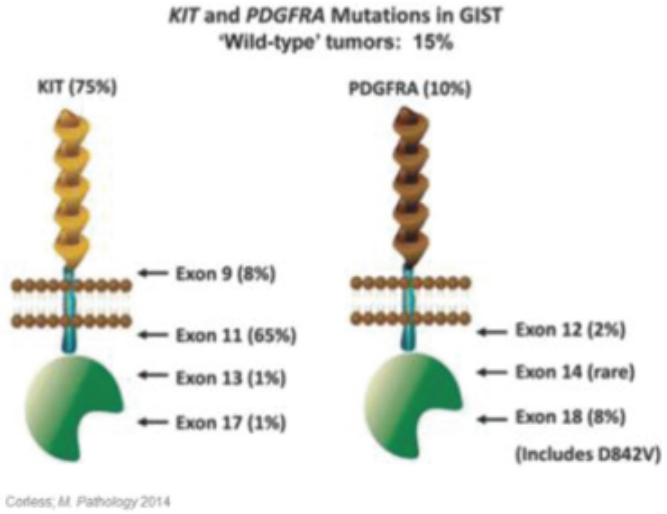
PDGFRα has a similar dual switch mechanism: an exon 18 activation switch and an exon 12 inhibitory switch. DCC-2618 blocks the effects of both loss-of-function PDGFRα exon 12 inhibitory switch mutations and aggressive PDGFRα gain-of-function mutations in the exon 18 activation switch.

Market Opportunity in Gastrointestinal Stromal Tumors (GIST)

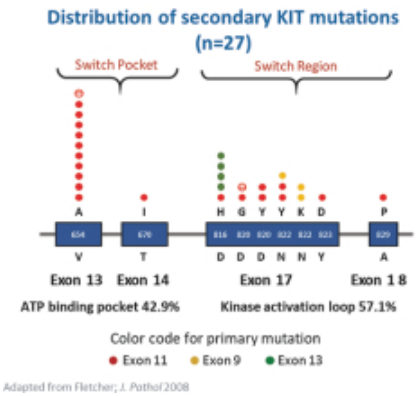
GISTs are the most common sarcoma of the gastrointestinal tract and present most often in the stomach or small intestine. The typical patient is over 40 years old. According to the American Cancer Society, in 2015 approximately 5,000 patients were newly diagnosed with GIST in the United States. Estimates for 5-year survival range from 48% to 90% depending upon the stage of the disease at diagnosis. We believe that the incidence rates for GIST in Europe are similar to the United States, ranging from 10 to 15 per 1,000,000 population.

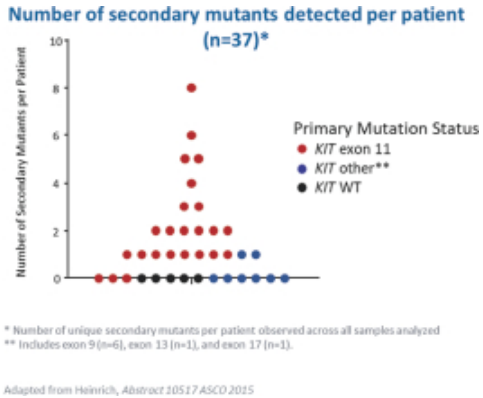
GIST is a disease driven initially by primary mutations in KIT kinase in approximately 75% to 80% of cases or in PDGFRα kinase in approximately 5% to 10% of cases. In about 9% to 15% of all GIST patients, which includes 85% of the cases in children, the disease is not driven by KIT or PDGFRα but by other genetic mutations or alterations. Primary mutations in the KIT gene are found in exon 11 in approximately 65% of GIST patients, in exon 9 in approximately 8% of GIST patients, and less frequently in exon 13 or 17. Primary mutations in the PDGFRα gene are usually found in exon 18 (a mutation referred to as D842V being the most frequent) and more rarely in exon 12. Activation of these kinases caused by primary mutations leads to

uncontrolled cancer cell growth and spread. The diagram below illustrates the mutations that drive GIST, assuming 75% are KIT-driven, the lower end of the range cited by various sources.



Metastatic KIT-driven GIST is a disease characterized by many mutations in KIT, with over 90% of individual KIT-driven GIST patients harboring multiple mutations that drive progression of their disease. Multiple secondary mutations can arise within an individual patient in different areas or sites of tumor growth. Drug resistant secondary mutations in patients with KIT-driven GIST span exon regions 13 to 18, and in a recent study, 35% of GIST patients had at least two secondary mutations, each as illustrated below.





The complex heterogeneity of KIT mutations within individual tumors and individual patients is a major cause of resistance to existing therapies, which individually only address a subset of the mutations driving disease progression. A kinase inhibitor that could inhibit a broad spectrum of clinically relevant KIT mutations could be of high therapeutic value in the treatment of KIT-driven GIST in patients who are unresponsive to treatment or have grown resistant to treatment. In PDGFRα-driven GIST, there are no approved therapies. The primary PDGFRα mutations are mostly insensitive to imatinib and other drugs approved for GIST. We believe our design of DCC-2618 as a PDGFRα switch control inhibitor may make the appearance of secondary mutations less likely after treatment than with a traditional kinase inhibitor.

First-line Treatments For GIST

Patients diagnosed early with localized GIST generally undergo surgical resection of their tumors. In surgically resected patients considered at a high risk of recurrence and in unresectable or metastatic patients, the kinase inhibitor imatinib is the only approved first line therapy in the United States. Imatinib is typically prescribed in doses of 400 mg or 800 mg daily. Tumors are measured by CT scan and changes in size characterized by RECIST. RECIST criteria define a partial response, or PR, as tumor size reduction of 30% or more, a complete response, or CR, as tumor size reduced by 100%, and progressive disease, or PD, as an increase in tumor size by 20% or more. RECIST criteria define stable disease as that in between a PR or PD. In one Phase 3 trial of GIST patients with unresectable or metastatic disease treated with imatinib, CRs were seen in only about 5% of patients dosed at 400 mg QID and aggregate CRs and PRs, which is defined as an overall objective response rate, or ORR, were seen in approximately 45% of these patients. Patients with PDGFRα-driven GIST are mostly insensitive to imatinib and generally fail to respond to therapy. While imatinib generally is well-tolerated, in one clinical study involving patients receiving 400 mg of imatinib daily, 43% experienced one or more Grade 3 to 5 adverse events, 16% underwent dose reductions and 38% interrupted treatment. Among patients treated with 800 mg of imatinib daily, 58% had dose reductions and 59% interrupted treatment.

Disease progression in advanced GIST is often due to secondary mutations in KIT or PDGFRα that cause resistance to imatinib. Although imatinib is effective against KIT mutations in exon 11 and has some limited efficacy against exon 9 mutations when the dose is increased from 400 mg to 800 mg daily, secondary mutations in KIT in exons 13, 14, 17 and 18 or most primary mutations in PDGFRα confer resistance to imatinib. While more than 80% of GIST patients will see some clinical benefit from imatinib monotherapy, and a small portion of patients have shown progression-free survival, or PFS, up to ten years, greater than 50% of patients will develop PD by two years, and 90% at ten years. Of the approximately 5,000 GIST patients that are reported as newly diagnosed each year in the United States, we estimate that about 65% will experience metastatic disease and 90% will receive first-line treatment with imatinib. Most of these imatinib treated patients, or approximately 2,600, will be eligible to progress to second-line therapy.

Second and Third-line Treatments For GIST

In KIT-driven GIST patients who progress on imatinib the clinical goal is stabilization of their disease. Objective responses, as judged by a RECIST-defined decrease in the size of measurable lesions, are rare and increasingly considered on their own to be poor surrogates for clinical benefit in second- and third-line patients. FDA recognized endpoint for approval of the two approved agents for second- and third-line therapies in GIST was median PFS. We believe that the rate of disease control, which includes patients with stable disease and PRs and CRs, is an appropriate measure of clinically relevant activity and a likely predictor of PFS and durability of treatment. In GIST patients who progress on imatinib, second-line therapy is typically sunitinib, which was approved in 2006 for patients with GIST who had disease progression following treatment with, or intolerance to, imatinib. Sunitinib has greater activity against mutations in KIT exon 9 compared to imatinib and less activity against mutations in KIT exon 11. Additionally, sunitinib shows activity against KIT exon 13 and exon 14 mutations but is not active against mutations in exon 17 and exon 18. Only about half of GIST patients show benefit on sunitinib therapy and the reported PFS is 6.1 months. Unlike treatment with imatinib in first-line therapy, sunitinib rarely produces CRs or PRs per RECIST with an ORR of approximately 7%. Approximately 5% to 10% of GIST patients on sunitinib experienced each of the following Grade 3 or 4 adverse events: hypertension, diarrhea, fatigue, asthenia and hand-foot syndrome. In two large retrospective studies of sunitinib in GIST, 20% of patients experienced adverse events leading to treatment discontinuation. The emergence of KIT mutations in exon 17 or 18 confers resistance to sunitinib.

In 2013, regorafenib received marketing approval in the United Sates for the treatment of adults with metastatic and unresectable GIST who have experienced disease progression on, or intolerance to, imatinib and sunitinib. In addition to being active against KIT mutations in exon 11, regorafenib is the only approved therapy with activity against a subset of KIT mutations in exon 17. However, regorafenib does not inhibit all KIT

mutations in exon 17 or exon 18. The reported median PFS with regorafenib is 4.8 months. Similar to treatment with sunitinib, regorafenib rarely produces CRs or PRs per RECIST as shown by the observed ORR of approximately 4.5%. Approximately 61% of GIST patients on regorafenib experienced at least one Grade 3 or 4 adverse event while on study including hypertension (23%), hand-foot syndrome (20%), and diarrhea (5%). Regorafenib also has shown increased liver toxicity. Liver function tests are recommended prior to initiation of therapy and periodically over the first two months of treatment.

The following table shows reported PFS, ORR, stable disease, and DCRs for imatinib, sunitinib and regorafenib in first-line, second-line, and third-line GIST, respectively, based upon the published results of registrational trials that were presented to FDA for approval of these drugs.

Endpoints From Pivotal Trials of Approved GIST Therapies

	First Line *Imatinib (n=147) (Blanke et al. 2008)*	Second Line *Sunitinib (n=243) (Demetri et al. 2012)*	Third Line *Regorafenib (n=133) (Demetri et al. 2013)*
TTP/PFS (months)	24.0	6.1	4.8
ORR (%)	68.1%	7.0%	4.5%
SD (%)	15.6%	53.0%	48.1%
Disease Control Rate	83.7%*	60.0%*	52.6%**

TTP Time To Progression
PFS Progression Free Survival
ORR Objective Response Rate
SD Stable Disease

* Time point not disclosed
** At 12-weeks

While imatinib, sunitinib and regorafenib, the only kinase inhibitors currently approved for the treatment of GIST, inhibit certain clinically relevant initiating and drug resistance-causing mutations in KIT, these approved drugs each inhibit only a limited subset of KIT and PDGFRα mutations known to occur in GIST patients. Although GIST patients may experience periods of disease control with these treatments, due to the heterogeneous nature of the mutations that drive the disease, many patients continue to progress and ultimately fail all lines of treatment. Of the approximately 5,000 GIST patients newly diagnosed each year in the United States we estimate that about 65% will experience metastatic disease and of these about 2,100 will fail second-line and third-line therapies and become fourth-line patients and about 2,600 will fail first-line therapies and become second-line patients. We estimate the annual incidence of new patients with GIST to be approximately 9,500 in Europe and Japan, of which we estimate that approximately 4,000 and 5,000 will become eligible as fourth- and second-line patients, respectively. Treatment of GIST patients who are resistant to or intolerant of these approved second- and third-line drugs remains an area of high unmet medical need. In addition, there are currently no approved therapeutic options for PDGFRα-driven GIST that potently inhibit D842V mutations, which is the most common mutation. We estimate that approximately 400 and 700 GIST patients have PDGFRα-driven disease in the United States and Europe and Japan combined, respectively. In preclinical assays, DCC-2618 is potently active against the D842V mutation and other PDGFRα primary mutations. We believe that DCC-2618 may offer a potential new treatment for these patients in addition to those patients who failed currently approved kinase inhibitors.

Clinical Development of DCC-2618

Ongoing and Planned Phase 3 Trials for DCC-2618 in GIST

In January 2018, we initiated a pivotal Phase 3 trial, or INVICTUS, comparing treatment with DCC-2618 to placebo in 120 fourth-line plus GIST patients. We expect to initiate a second pivotal Phase 3 trial comparing treatment with DCC-2618 to sunitinib in up to 450 second-line GIST patients in the second half of 2018 subject to discussions with the FDA and EMA. Initiation of our second-line Phase 3 trial is subject to data from our ongoing Phase 1 trial and FDA and EMA feedback on the study protocol.

INVICTUS: Ongoing Phase 3 Trial in Fourth-Line Plus GIST

The primary endpoint in our pivotal Phase 3 trial in fourth-line plus GIST is a clinically meaningful improvement in median PFS in patients treated with DCC-2618 compared to placebo. PFS will be determined by independent radiologic review of CT scans, as assessed by RECIST. A secondary endpoint for INVICTUS is overall survival, or OS. Assuming positive results from this trial, we plan to submit a new drug application, or NDA, to FDA for the use of DCC-2618 in fourth-line GIST patients.

INVICTUS is a 2:1 randomized, double-blind, placebo-controlled trial. Two-thirds of patients are randomized to the DCC-2618 arm and one-third to placebo. We administer 150 mg doses of DCC-2618 or placebo QD in repeated 28-day cycles with best supportive care, or BSC. We will evaluate patients for PFS based upon independent radiologic review of CT scans, as assessed by RECIST. Tumor response assessments per RECIST will be conducted every cycle for the first three cycles and then every two cycles thereafter beginning with the fourth cycle. We expect to enroll patients who have a confirmed diagnosis of GIST and have previously received at least three different kinase inhibitors including imatinib, sunitinib and regorafenib. Patients are treated with DCC-2618 or placebo, in accordance with their randomization, until they develop PD, experience unacceptable toxicity, or withdraw consent. Placebo patients have the opportunity to cross over to DCC-2618 treatment upon PD with placebo. Patients on DCC-2618 have the opportunity to remain on DCC-2618 upon PD.

Planned Phase 3 Trial in Second-Line GIST

We expect that the primary endpoint in our pivotal Phase 3 trial in second-line GIST will be a clinically meaningful improvement in median PFS in patients treated with DCC-2618 compared to sunitinib. PFS will be determined by independent radiologic review of CT scans, as assessed by RECIST. Assuming positive results from this trial, we plan to submit an NDA to FDA for the use of DCC-2618 in second-line GIST patients.

In our pivotal Phase 3 trial in second-line GIST, we expect to enroll patients who have progressed on or are intolerant to imatinib, comparing DCC-2618 against sunitinib. The design for this trial has not yet been finalized and is subject to discussions with FDA and EMA.

Phase 1 Trial of DCC-2618 in GIST and Other Solid Tumors

In September 2017, we reported safety and preliminary efficacy data from the ongoing Phase 1 trial of DCC-2618 in 57 GIST patients and updated safety data in a total of 70 patients, including GIST patients and 13 patients with other genetically defined advanced malignancies. In the dose escalation stage of this Phase 1 trial, we evaluated DCC-2618 in multiple ascending oral doses in 62 patients with advanced malignancies in repeated 28-day cycles. Based upon the results from the dose escalation stage, we have selected a dose of 150 mg once daily, taken with or without food, for the clinical trial expansion stage. We are enrolling patients with select advanced malignancies, including fourth-line plus GIST, second- and third-line GIST, ASM, and gliomas, including GBM, in expansion cohorts of this Phase 1 trial.

At the ESMO 2017 Congress, we presented preliminary efficacy data from the 57 GIST patients treated with DCC-2618 in the ongoing Phase 1 trial, including 47 with KIT-driven GIST, 4 with PDGFRα-driven GIST, one with SDH deficient GIST, and 5 GIST patients whose mutational status was not confirmed as of the data cut-off date of July 28, 2017. In addition, we presented safety data from a total of 70 patients including 57 GIST patients as well as 13 additional non-GIST patients across five different dose cohorts that included 7 GBM patients and one patient each with ASM, astrocytoma, thymic carcinoma, desmoid tumor, gynecological squamous cell carcinoma and adenoid cystic carcinoma.

Dose Escalation Stage of Phase 1 Trial

The primary objectives of the dose escalation stage of the Phase 1 trial were to determine the safety, tolerability and MTD of DCC-2618 and to determine a recommended Phase 2 dose. The secondary objectives

were to determine the pharmacokinetic, or PK, profile of DCC-2618 and to document preliminary evidence of antitumor activity. The safety endpoints of the escalation phase of the Phase 1 trial included dose limiting toxicities, or DLTs, and adverse events. The endpoints for preliminary assessment of antitumor activity included ORR and DCR at 12 weeks. Other endpoints included PFS, which is defined as time from Cycle 1 Day 1 to disease progression or death, for all solid tumor patients.

The Phase 1 trial included a screening visit that was conducted within 28 days prior to the first dose of study drug, a baseline visit, a treatment period of 28-day cycles, an intra-patient dose-escalation (if applicable for some patients), a final study visit, and a follow-up safety visit within 30 days after the last dose of study drug. Patients were excluded from the trial if they received treatment with anticancer therapy, including investigational therapy, within two weeks prior to the administration of study drug, with the exception of hydroxyurea, which was permitted to control white blood cell count in patients with hematological malignancies. Patients who received prior therapies with a half-life longer than three days were required to wait at least 28 days prior to the first administration of study drug.

We administered sequentially increasing doses of DCC-2618 QD or BID, or twice daily, in repeated 28-day cycles, which were evaluated for safety based on pharmacologically guided 3+3 escalation rules where three patients are initially enrolled into a given dose cohort. If no DLT is observed in any of these subjects, the trial proceeds to enroll three subjects into the next higher dose cohort until an MTD is identified or a recommended expansion dose is declared. MTD is determined to be the dose level immediately below the highest dose level tested where two DLTs were observed.

Solid tumors generally were measured by CT or MRI scan, as assessed according to RECIST. Malignant gliomas were measured by MRI scan and assessed by RECIST or Response Assessment in Neuro-Oncology, or RANO, Criteria. We conducted CT or MRI scans, as applicable, of each patient at baseline, at the end of the second 4-week cycle, after 8 weeks of therapy, and every 8 weeks thereafter. Response in systemic mastocytosis, or SM, patients is measured by assessing, among other things, the frequency with which KIT mutation alleles appear in sample tissues.

For pharmacodynamics assessments, the type and amount of mutations in KIT or PDGFRα in cell-free circulating tumor DNA, or cfDNA, isolated from plasma are evaluated. In solid tumor patients, determination of levels of circulating tumor cells, or CTCs, in whole blood are evaluated, while in ASM patients, changes in plasma D816V KIT allele fraction are evaluated. Metabolic response as measured by FDG-PET scans was assessed by European Organisation for Research and Treatment of Cancer criteria in GIST patients.

Ongoing Expansion Stage of Phase 1 Trial

The primary objectives of the expansion stage of the Phase 1 trial are to further evaluate the safety and tolerability of DCC-2618 and to determine the antitumor activity of DCC-2618 in all diseases studied in the trial. The secondary objectives are to determine the PK profile of DCC-2618 and determine allele frequency of KIT and PDGFRα mutations in plasma cfDNA and compare it with mutation allele frequency in GIST tumor tissue at baseline and in response to treatment of DCC-2618. The safety endpoints of the expansion phase of the Phase 1 trial include dose reduction or discontinuation of study drug due to toxicity and adverse events. The endpoints for preliminary assessment of antitumor activity include ORR and DCR at 12 weeks. Other endpoints include PFS for all solid tumor patients. In addition, patient reported outcome measures may be used to confirm the recommended expansion dose.

In the expansion stage, up to 200 patients will be enrolled in six disease specific cohorts for each of second/third-, fourth- and fifth-line KIT- or PDGFRα-driven GIST, SM and other hematologic malignancies, malignant gliomas, and other solid tumors. As of October 31, 2017, a total of 125 patients had been dosed with DCC-2618, of which 109 were GIST patients, including 54 patients in the three expansion cohorts for GIST patients.

The expansion stage of the Phase 1 trial includes a screening visit that is conducted within 28 days prior to the first dose of study drug, a baseline visit, a treatment period of 28-day cycles, a final study visit, and a follow-up safety visit within 30 days after the last dose of study drug. Intra-patient dose escalation is offered to patients upon radiographic progression. Patients are not eligible for the trial if they received treatment with anticancer therapy, including investigational therapy, within two weeks prior to the administration of study drug. Patients who received prior therapies with a half-life longer than three days are required to wait at least 28 days prior to the first administration of study drug.

We will administer DCC-2618 at the recommended expansion dose of 150 mg QD that was determined during the dose escalation stage of the Phase 1 trial. Patients who have disease progression by specified indication response criteria in the expansion stage may escalate to a higher daily dose of DCC-2618 after completion of the second cycle. Tumor and pharmacodynamic assessments in the expansion stage will generally be conducted in the same manner and according to the same criteria as in the dose escalation stage.

Tolerability Results in Dose Escalation and Expansion Stages of Phase 1 Trial

The dose escalation stage of the Phase 1 trial was designed to test the safety and tolerability of DCC-2618 in multiple ascending doses in approximately 50 patients with advanced, genetically defined solid tumors. In summary, the safety and tolerability findings from the dose escalation and expansion stages in 70 patients through the cut-off date of July 28, 2017 were as follows:

- oral doses of 20-200 mg BID, 100-250 mg QD were well tolerated;
- most common treatment emergent adverse events included fatigue, alopecia, anemia, increased lipase and decreased appetite;
- DLTs: two Grade 3 asymptomatic lipase elevation (100 mg BID and 200 mg BID) and one Grade 4 asymptomatic CK elevation (150 mg QD); these DLTs were considered not clinically relevant by investigators, and all DLT patients received waivers from site institutional review boards, or IRBs, to remain on study due to continuing clinical benefit;
- one dose reduction due to asymptomatic Grade 4 lipase elevation (100 mg BID) in Cycle 8; and
- MTD was not reached through 200 mg BID.

DCC-2618 was generally well tolerated at all doses administered and the treatment emergent adverse events by Grades 1/2 and Grades 3/4 are summarized in the table below.

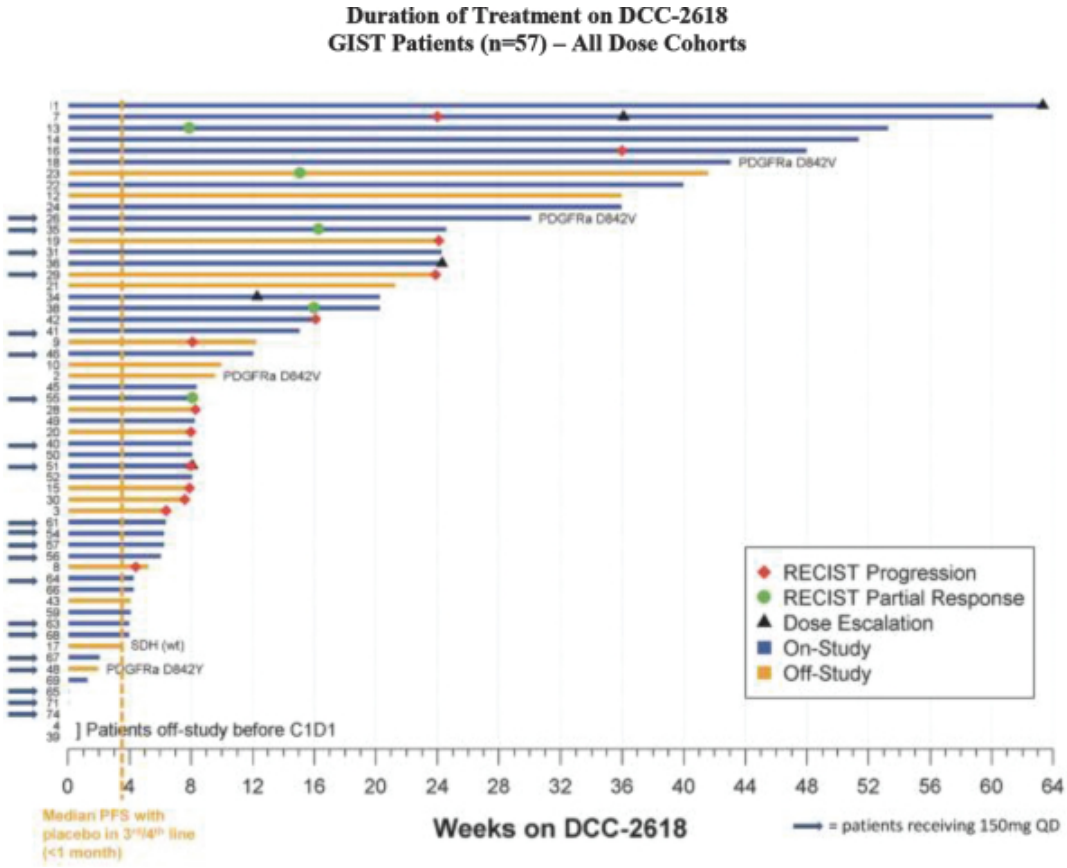
Treatment Emergent Adverse Events in ≥10% Patients (n=70)

Event Term	Total Events	<100 mg/d (n = 8)		≥100 mg/d (n = 62)		Total	150 mg QD (n = 21)		
		G1/2	G3/4	G1/2	G3/4	n = 70	G1/2	G3/4	Total
Lipase increased	33	5	1	15	12	33 (47%)	3	2	5 (24%)
Fatigue	32	6	0	25	1	32 (46%)	5	0	5 (24%)
Anemia	29	1	1	9	18	29 (41%)	0	1	1 (5%)
Decreased appetite	20	1	0	17	1	20 (29%)	3	0	3 (14%)
Diarrhea	16	1	0	15	0	16 (23%)	0	0	0 (0%)
Alopecia	15	1	0	14	0	15 (21%)	4	0	4 (19%)
Hypertension	15	0	1	9	5	15 (21%)	0	0	0 (0%)
Amylase increased	14	3	0	10	1	14 (20%)	1	0	1 (5%)
Myalgia	14	2	0	12	0	14 (20%)	2	0	2 (10%)
Weight decreased	14	1	0	13	0	14 (20%)	1	0	1 (5%)
Dyspnea	13	4	0	8	1	13 (19%)	1	0	1 (5%)
Abdominal pain	11	3	0	7	1	11 (16%)	0	0	0 (0%)
Constipation	11	4	0	7	0	11 (16%)	2	0	2 (10%)
Nausea	11	2	0	9	0	11 (16%)	1	0	1 (5%)
Palmar-plantar erythrodysaesthesia	11	0	0	11	0	11 (16%)	2	0	2 (10%)
Arthralgia	10	2	0	8	0	10 (14%)	0	0	0 (0%)
Blood bilirubin increased	10	1	0	7	2	10 (14%)	0	1	1 (5%)
Rash	8	2	0	6	0	8 (11%)	1	0	1 (5%)

Duration of Treatment of GIST Patients in Dose Escalation Stage of Phase 1 Trial

Since DCC-2618 was specifically designed to treat KIT- and PDGFRα-driven tumors such as GIST and GIST patients made up more than three-quarters of all patients enrolled, we carefully evaluated the potential therapeutic benefit in the 57 GIST patient subgroup. We enrolled patients in the Phase 1 dose escalation stage progressively from November 2015.

The following figure shows the duration of treatment with DCC-2618 as of July 28, 2017, or the cut-off date, for all GIST patients in this trial who had received at least one dose of DCC-2618. Of these patients, 67% (38 of 57) remained active on study. Of the 19 patients who were off study, 47% (9 of 19) discontinued due to PD per RECIST and 53% (10 of 19) for a variety of other reasons.

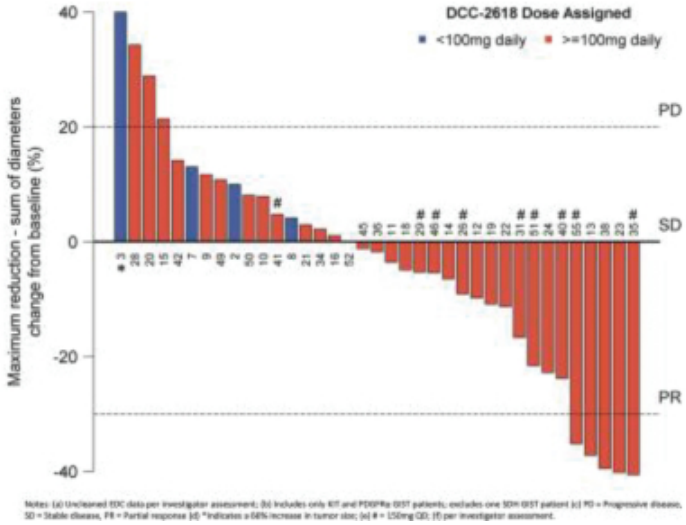


Collectively, these patients had received an average of 3.3 prior treatments (individual agents) with 73% (36 of 49) previously receiving all three approved therapies imatinib, sunitinib, and regorafenib. In the second and third-line pivotal trials of sunitinib and regorafenib, patients with KIT- and PDGFRα-driven GIST had a DCR of 60% (n = 243) and 52.6% (n = 133), respectively. Although we believe that these initial data on duration of treatment from the dose escalation and expansion stages of our Phase 1 trial of DCC-2618 demonstrate single-agent activity, measured by either PR or stable disease, these data are based on results from 57 GIST patients, 51 of which had primary or secondary mutations in KIT or PDGFRα and who had progressed on multiple approved therapies. Our trial sizes to date have been limited. We intend to enroll a larger number of patients in the expansion stage of this Phase 1 trial.

RECIST Responses in KIT- or PDGFRα-driven GIST Patients in Phase 1 Trial

For all GIST patients except for one patient with SDH deficient GIST, who received both a baseline and post-treatment CT scan by the cut-off date (n = 37), the greatest reduction or smallest increase in tumor size from baseline as measured by CT or MRI scan, or best radiology evaluation, for solid malignancies per RECIST is shown in the following figure.

Best Radiographic Response Per RECIST in KIT or PDGFRα GIST Patients (n=37)



Reduction of KIT and PDGFRα Mutant Allele Frequencies in Patients in Phase 1 Trial

Based upon pharmacodynamic data from the Phase 1 trial, we confirmed that the broad KIT and PDGFRα inhibiting profile of DCC-2618 that we observed preclinically translates into the clinic. Circulating tumor cfDNA originates from tumors or from circulating tumor cells and is measured in plasma during treatment. This minimally invasive technique is often referred to as “liquid biopsy.” Next generation sequencing, or NGS, is used to identify and quantify the specific mutations in KIT and PDGFRα that are present in baseline patient samples and those obtained after treatment with DCC-2618. As we reported at the American Association for Cancer Research meeting in April 2017, or AACR 2017, plasma cfDNA revealed a total of 43 KIT mutations in 88% (21 of 24) of patients with KIT mutant GIST, with 20 individual mutations spread across six different exons,

demonstrating the heterogeneity and multiplicity of KIT mutations in these patients. The figure below summarizes these findings.

Heterogeneity and Multiplicity of KIT Mutations in GIST Patients

KIT Exons					
Exon 9	Exon 11	Exon 13	Exon 14	Exon 17	Exon 18
A502_Y503 dup (9)	All unique (8)	V654A (3)	N680K (2)	D820x (6)	A829P (2)
		K642E (1)	D677N (1)	Y823x (4)	
				D816E (3)	
				N822K (2)	
				C809G (1)	
				K826_G827 del (1)	
n = 9	n = 8	n = 4	n = 3	n = 17	n = 2

In addition, at the ESMO 2017 Congress, we reported that treatment with DCC-2618 was shown to markedly reduce cfDNA KIT mutant allele frequencies in 19 evaluable patients indicating DCC-2618 inhibited the full spectrum of identified mutations in exons involved in KIT-driven GIST, including exons 9, 11, 13, 14, 17 and 18, at doses as low as 50 mg BID, or 100 mg daily, in those patients.

These clinical results expand upon results previously presented at the 2017 ASCO Meeting that 100 mg total daily dose of DCC-2618 is sufficient to robustly inhibit a broad range of relevant drug resistance mutations of KIT and PDGFRα, including those mutations with the highest in vitro IC_{90}, values, which is the concentration of DCC-2618 required to inhibit kinase activity by 90%, to DCC-2618, corroborating preclinical studies. Since oral administration of DCC-2618 results in the production of an active metabolite DP-5439, we also evaluated the potency of DP-5439 against these mutations. Potency is measured by the concentration of DCC-2618 or DP-5439 required to inhibit kinase activity by 90%. Therefore, we focused subsequent analyses of potential efficacy on the group of patients who received 100 mg or more per day. This group includes patients who received 50, 100, 150, or 200 mg BID or 100, 150 or 250 mg QD. From the PK analysis, we found 200 mg of DCC-2618 daily (100 mg BID) produced plasma levels or exposure of DCC-2618 and DP-5439 about twice that of 100 mg daily. Higher doses produced comparable plasma levels or drug exposure within an approximately two-fold range.

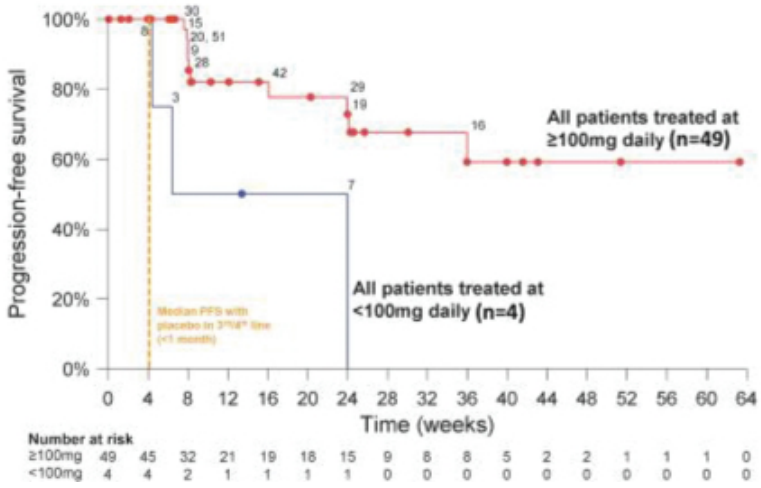
Duration of Disease Control—100 mg or More per Day versus Less than 100 mg per Day

To evaluate the durability of disease control with DCC-2618 in GIST patients, we assessed the duration of disease control in GIST patients to the point where the patient's disease progressed for all KIT- and PDGFRα-driven GIST patients who received more than a single dose of DCC-2618 but less than 100 mg of DCC-2618 daily (n = 4) and those patients who received at least 100 mg of DCC-2618 daily (n = 49). For the patients receiving at least 100 mg of DCC-2618 daily, although a significant number of recently enrolled patients remain censored in this analysis, based on events occurring to date a median PFS has not yet been reached. For the patients receiving less than 100 mg of DCC-2618 daily, the median PFS was 3.5 months. In published trials comparing imatinib rechallenge to placebo and regorafenib to placebo, the reported median PFS in the placebo-treated groups was less than one month.

The following figure shows the percentage of KIT- and PDGFRα-driven GIST patients without PD at each time point in patients who received more than a single dose of DCC-2618 but less than 100 mg of DCC-2618 daily (n = 4) and those patients who received at least 100 mg of DCC-2618 daily (n = 49). Circles represent patients (there may be more than one at a particular timepoint) who had not progressed as of the end of the treatment/study or the last visit date (if still on treatment). Two patients within this group, who demonstrated progressive disease per RECIST, have continued to receive DCC-2618 due to physician assessed clinical benefit.

For one of these patients, who received 30 mg BID for 36 weeks, the dose was increased to 150 mg BID and the patient has since experienced a reduction in tumor size as measured per RECIST.

Progression-Free Survival Rates in KIT- and PDGFRα-driven GIST Patients

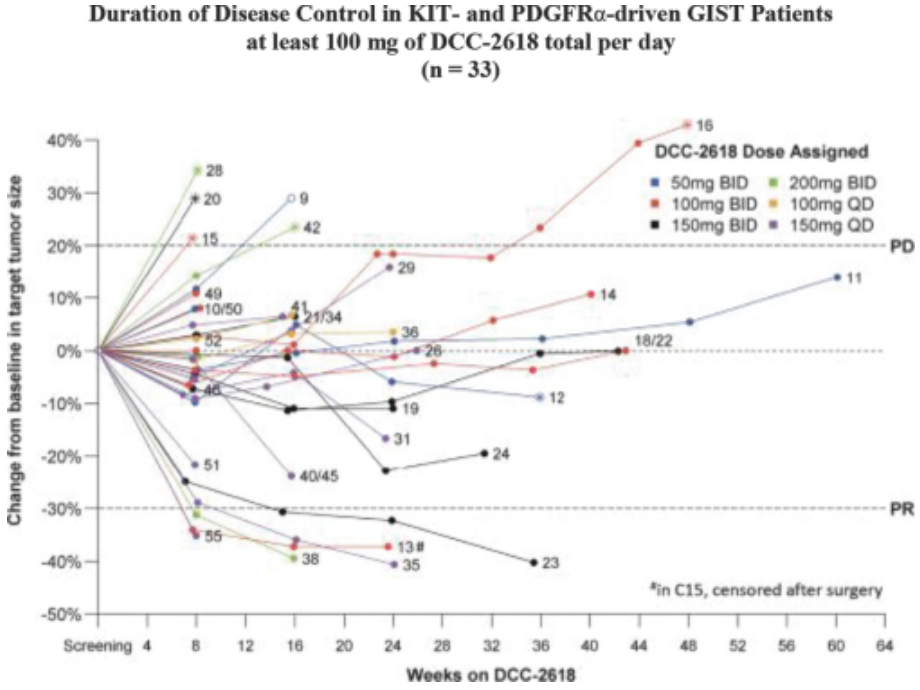


Data for all patients evaluable by the cut-off date were included in the above chart. In a recent randomized placebo-controlled trial comparing imatinib re-challenge to placebo, in patients in a similar third- or fourth-line setting, the median PFS observed in the placebo-treated group was 0.9 months. Similarly, in the randomized placebo-controlled regorafenib pivotal trial in third-line GIST patients, the median PFS observed in the placebo-treated group was also 0.9 months. In its pivotal trial, sunitinib demonstrated a median PFS of 6.1 months.

Disease Control Rate in KIT- and PDGFRα-driven GIST Patients

As presented at the ESMO 2017 Congress, in our ongoing Phase 1 trial of DCC-2618, in GIST patients shown to harbor a broad range of KIT and PDGFRα mutations who received at least 100 mg of DCC-2618 daily, we observed 91% (30 of 33 evaluable patients) had a best response of stable disease or PR. In addition, we observed a DCR of 76% at 12 weeks in 25 patients and 57% at 24 weeks in 21 patients.

The chart below shows durability of response in 33 patients receiving DCC-2618 at doses of at least 100 mg daily, where each cycle has a duration of 4 weeks. The best response and DCRs described above and the tumor assessments in the chart below are based on investigator assessment of tumor response in a limited number of patients and may not be predictive of or consistent with the results of later trials. Closed circles denote that patient was on DCC-2618 at the time of scan. Open circles denote that patient was off DCC-2618 at time of scan. Stars indicate final study visit.

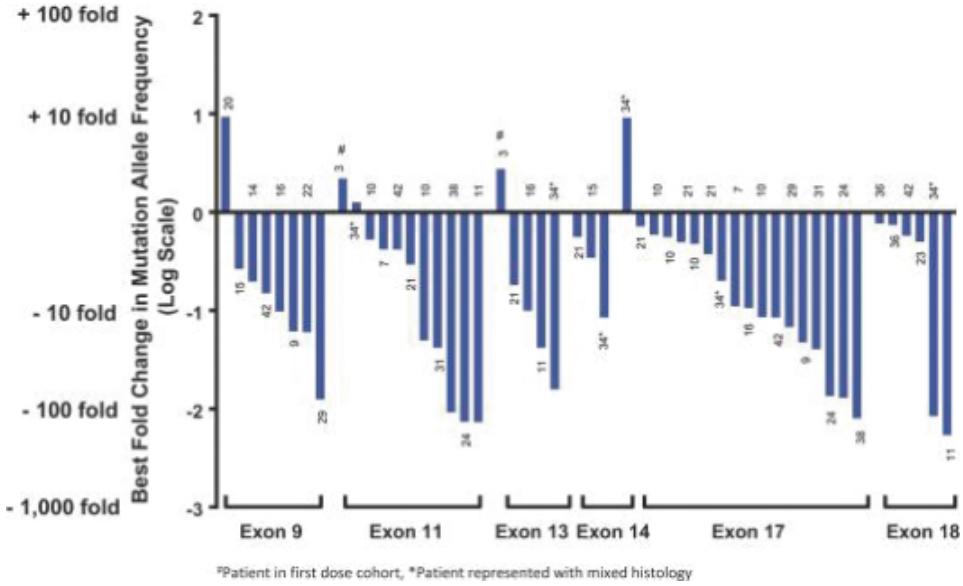


Maximum Change in cfDNA Mutant Allele Frequency in KIT-driven GIST Patients

The following figure, which was included in our ESMO 2017 Congress presentation, shows the maximum change in cfDNA mutant allele frequency, or MAF, by exon in 19 patients with circulating KIT mutations observed at baseline and having data from at least one sample following treatment with DCC-2618. One of the exploratory objectives of our Phase 1 trial was to compare allele fraction of KIT and PDGFRα mutations in plasma cfDNA with mutation allele fraction in GIST tumor tissue and their association with study drug response. We observed in this group of KIT-positive GIST patients that treatment with DCC-2618 resulted in large reductions in the frequency of circulating cfDNA mutant KIT alleles in 89% (17 of 19) of patients. Patient

number 20 experienced an increase in plasma KIT mutation in exon 9, which was coincidental with progressive disease by RECIST at week 12.

Reductions in Circulating MAF of KIT Mutations in all Clinically Relevant Exons
(Note log scale: -1 = 10-fold reduction, -2 = 100-fold reduction)



[#]Patient in first dose cohort, *Patient represented with mixed histology

In summary, whether it is the known primary mutations in KIT that initiate GIST or secondary mutations in KIT that subsequently confer drug resistance, the data presented at the ESMO 2017 Congress, the 2017 ASCO Meeting and AACR 2017 demonstrate potent clinical inhibition of a broad range of these mutations by DCC-2618. The reductions in cfDNA KIT mutant allele frequencies described above were from a total sample of 19 patients with KIT-driven GIST. Our trial sizes to date have been limited. We are enrolling a larger number of patients, which will include additional patients with KIT-driven GIST, in the expansion stage of this Phase 1 trial.

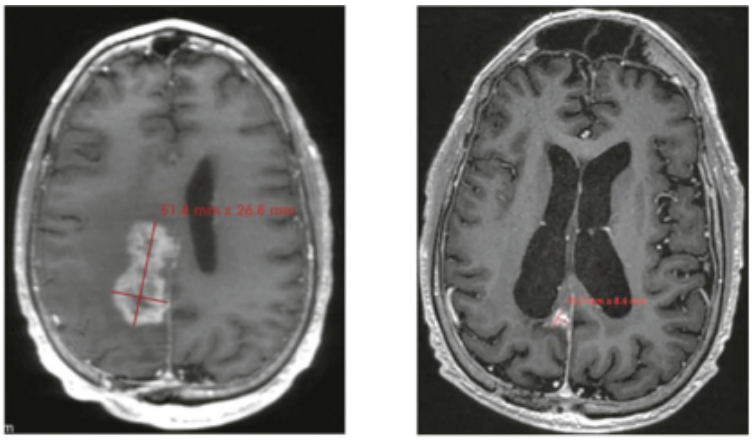
Development of DCC-2618 in Gliomas, including GBM

Gliomas, including GBM, are among the most severe types of brain cancer and according to Central Brain Tumor Registry of the United States, approximately 12,000 patients are projected to be diagnosed in 2016 with estimates for 1-year survival of around 40% and for 2-year survival of 15% to 20%. We believe that data for Europe and Japan suggests similar epidemiology to the United States. In approximately 15% of the patients diagnosed with GBM or glioma, their disease is driven by chromosome 4 genetic amplifications that increase the activity of PDGFRα, KIT and closely related kinases. We estimate that the annual incidence of PDGFRα-driven disease in GBM or glioma patients is approximately 2,400 in the United States and 4,700 in Europe and Japan combined.

At the 22nd Annual Scientific Meeting and Education Day of the Society for Neuro-Oncology in November 2017, we presented data from eight patients diagnosed with malignant gliomas treated with DCC-2618. Of the five evaluable patients, four had GBM. Among these four GBM patients, we observed one PR, as defined by RANO, and three patients with progressive disease. In addition, of the three patients who were non-evaluable, two patients remained on study and one had progressive disease. The GBM patient with the PR has remained on therapy for more than 22 cycles, or 19.4 months and has a 6-fold triple chromosome 4 amplification of three

kinase genes, KIT, PDGFRα and VEGFR2. This patient was diagnosed in February 2015 and prior to entering the Phase 1 trial of DCC-2618, had progressed after three months of standard of care treatment, which included surgical resection, radiation plus concurrent temozolomide followed by adjuvant temozolomide. As of Cycle 23 Day 1, this patient had demonstrated a reduction in tumor size of 94% as shown in the figure below.

Reduction in Tumor Size of GBM Patient by RANO Criteria



In the expansion stage of the Phase 1 trial of DCC-2618, we are enrolling a cohort of GBM or other glioma patients with amplifications and alterations in PDGFRα including the KIT, PDGFRα and VEGFR2 triple amplification. If we observe additional responses in the Phase 1 expansion cohort, we will seek FDA guidance on the potential for an accelerated development plan in this difficult to treat patient population.

Development of DCC-2618 in Systemic Mastocytosis (SM)

Mastocytosis is a disease characterized by an abnormal accumulation of mast cells, a type of white blood cell, located in peripheral tissues and organs. Mast cells store components that mediate inflammatory and allergic responses, such as tryptase, histamine, serotonin and heparin. The disease can be sub-divided into cutaneous mastocytosis where mast cells accumulate only in the skin and SM where mast cells accumulate in at least one organ (with or without skin involvement). In adults, most cases are systemic, frequently with accompanying skin involvement, whereas in children cutaneous mastocytosis is more common and many of the cases resolve spontaneously. While the exact number of patients suffering from all forms of mastocytosis, including urticaria pigmentosa, is not known, it is estimated that about 3,000 patients are newly diagnosed each year in the United States and about 30,000 patients live with the disease in the United States. Within SM there are four main sub-types: indolent and smoldering, or ISM, aggressive SM, SM with associated hematological neoplasm, or SM-AHN, and mast cell leukemia, or MCL. ASM includes aggressive SM, SM-AHN and MCL. We estimate the annual incidence of new patients with ASM to be approximately 1,400 and 2,800 in the United States and Europe and Japan combined, respectively. Rates of survival vary significantly between the various sub-types, from normal in patients with ISM to significantly less than one year in patients with MCL. The chart below

summarizes the prognosis, or likely course of the disease, and survival, or the time from which primary treatment begins, until death.

Subtypes of Systemic Mastocytosis

Sub-type of Systemic Mastocytosis	Prognosis	Survival
Indolent & smoldering (ISM)	Normal life expectancy. Patients typically present with stable disease. Progression to a more severe form occurs in 1-5%.	>20 yrs
Aggressive SM (ASM)	Survival is markedly reduced compared to natural history controls	~3.5 yrs
SM with hematological neoplasm (SM-AHN)	Survival is determined by prognosis of the hematological disorder and is typically substantially reduced as compared to normal controls	~2.5 yrs
Mast Cell Leukemia (MCL)	Patients have a poor prognosis and often progress to multiple organ failure in weeks to months	~0.2 yrs

Approximately 94% of SM patients are reported to have a somatic D816V mutation in KIT. This D816V mutation is a gain-of-function mutation in the KIT activation switch, which leads to unregulated KIT activation. The KIT receptor, which is widely expressed on mast cells, stimulates signaling pathways that control cell growth, differentiation and survival. The gain-of-function mutation, D816V, enables mast cells to proliferate in the absence of normal activation signals. Approved drugs to manage the symptoms include antihistamines, corticosteroids, leukotriene antagonists, mast cell stabilizers, proton pump inhibitors and histamine H2 receptor antagonists, epinephrine salbutamol and other beta-2 agonists. Midostaurin, which targets various kinases including FLT3, PDGFRα, CDK1, Src, KIT and VEGFR, was approved for the treatment of ASM and MCL in April 2017 in the United States based on response rate and duration in a single-arm, open-label study of midostaurin 100 mg orally twice daily. DCC-2618 potently inhibits the D816V mutation. We are enrolling ASM patients within an expansion cohort of our Phase 1 trial.

Preclinical Profile of DCC-2618

We specifically designed DCC-2618, our KIT and PDGFRα switch control inhibitor, to improve the treatment of GIST patients by inhibiting the full spectrum of the known mutant KIT and PDGFRα kinases responsible for initiating the disease, or primary mutations, and the KIT mutations that cause drug resistance or secondary mutations.

The following table lists primary KIT and PDGFRα mutations evaluated in recombinant enzyme assays or in Chinese hamster ovary, or CHO, cells transfected with KIT mutations, together with their sensitivity to inhibition with DCC-2618. Since oral administration of DCC-2618 results in the production of an active metabolite DP-5439, we also evaluated the potency of DP-5439 against these mutations. As shown in the following table, DCC-2618 inhibits a broad spectrum of primary KIT and PDGFRα mutations with nanomolar potency and DP-5439 exhibits a comparable range of potencies. Potency is measured by the concentration of DCC-2618 or DP-5439 required to inhibit kinase activity by 50%, or the inhibitory concentration 50%, or IC_{50}. These preclinical evaluations demonstrate that DCC-2618 is a KIT inhibitor that at nanomolar potencies blocks initiating mutations in exons 9, 11, 13, and 17 known to be present in GIST patients and the mutation in exon 17

that occurs in SM patients. DCC-2618 is also a potent inhibitor of initiating PDGFRα mutations in exons 12 and 18. These results against the broad range of KIT mutations known to occur in GIST patients provides preclinical data that support a broad-spectrum KIT profile observed with DCC-2618 in reducing cfDNA MAF in all clinically relevant exons.

Potency of inhibition of clinically relevant primary KIT and PDGFRα mutations by DCC-2618 and its active metabolite DP-5439

Frequency	KIT and PDGFRα	DCC-2618	DP-5439
% Patients	Primary GIST Mutations	IC50 (nM)[1]	IC50 (nM)[1]
67%	KIT Exon 11 Deletions	3	3
	KIT Exon 11 V560D	8	NT
10%	KIT Exon 9 A-Y Duplication	246	NT
1%	KIT Exon 13 K642E	140	312
1%	KIT Exon 17 D816Y	15	NT
>90%[2]	KIT Exon 17 D816V [2]	25	19
1%	PDGFRα Exon 12 V561D	25	NT
5%	PDGFRα Exon 18 D842V	24	26
1%	PDGFRα Exon 18 842-845 Deletion	77	NT

(1) Values in the table are the concentrations of DCC-2618 or DP-5439 required to achieve 50% inhibition of the kinase indicated. A concentration of 0.51 ng/ml of DCC-2618 is a 1 nanomolar concentration.
(2) Frequency in systemic mastocytosis.

Secondary KIT mutations that confer drug resistance have been identified in exons 13 and 14 in the switch pocket region and in exons 17 and 18 in the switch region. The table below shows that DCC-2618 inhibits these secondary mutations across the full spectrum of known primary mutation subclasses with nanomolar potency in

transfected CHO cells or in cell lines from patients resistant to earlier lines of treatment (indicated by "*"). The active metabolite DP-5439 inhibited this spectrum of mutations with a similar nanomolar potency.

Potency of inhibition of clinically relevant secondary KIT mutations by DCC-2618 and its active metabolite DP-5439

KIT Secondary GIST Mutations		DCC-2618	DP-5439
Primary KIT Mutation	Secondary KIT Mutation	IC50 (nM)(1)	IC50 (nM)(1)
Exon 9 Duplication	Exon 13 V654A	30	NT
Exon 9 Duplication	Exon 13 N655S	63	60
Exon 9 Duplication	Exon 14 N680K	47	129
Exon 9 Duplication	Exon 17 D816G	20	48
Exon 9 Duplication	Exon 17 D820G	16	54
Exon 9 Duplication	Exon 17 D820E	31	99
Exon 9 Duplication	Exon 17 NB22K	17	47
Exon 9 Duplication	Exon 17 N822Y	22	44
Exon 9 Duplication	Exon 17 N822H	25	169
Exon 11 Deletion	Exon 13 V654A*	7	15
Exon 11 Del SKV557C	Exon 14 T670I	183	121
Exon 11 Del SKV557C	Exon 17 D820Y	6	21
Exon 11 Del SKV557C	Exon 17 D820A	7	26
Exon 11 V560D	Exon 13 V654A	189	191
Exon 11 V560D	Exon 14 T670I	221	88
Exon 11 V560D	Exon 17 D820A *	53	150
Exon 11 V560D	Exon 17 N822K	23	49
Exon 11 V560D	Exon 17 Y823D	14	NT
Exon 11 V560D	Exon 18 A829P	87	NT

NT = Not Tested *GIST patient derived cell line

(1) Values in the table are the concentrations of DCC-2618 or DP-5439 required to achieve 50% inhibition of the kinase indicated. A concentration of 0.51 ng/ml of DCC-2618 is a 1 nanomolar concentration.

The data presented above demonstrate DCC-2618's ability to inhibit known KIT and PDGFRα initiating mutations and KIT drug resistance mutations. We also evaluated the potential resilience of DCC-2618 to new gain-of-function resistance mutations to kinase switch control inhibition. To that end, we performed saturation mutagenesis studies in cells to examine the emergence of new mutations in KIT following exposure to DCC-2618 or imatinib. The results demonstrate that, while new drug resistance mutations are produced rapidly in response to imatinib exposure (four new mutations secondary to exposure were recorded), we observed no gain-of-function mutations resistant to DCC-2618. These results further support the broad-spectrum KIT inhibitor profile of DCC-2618 and its potential resilience to the emergence of mutations resistant to kinase switch control inhibition.

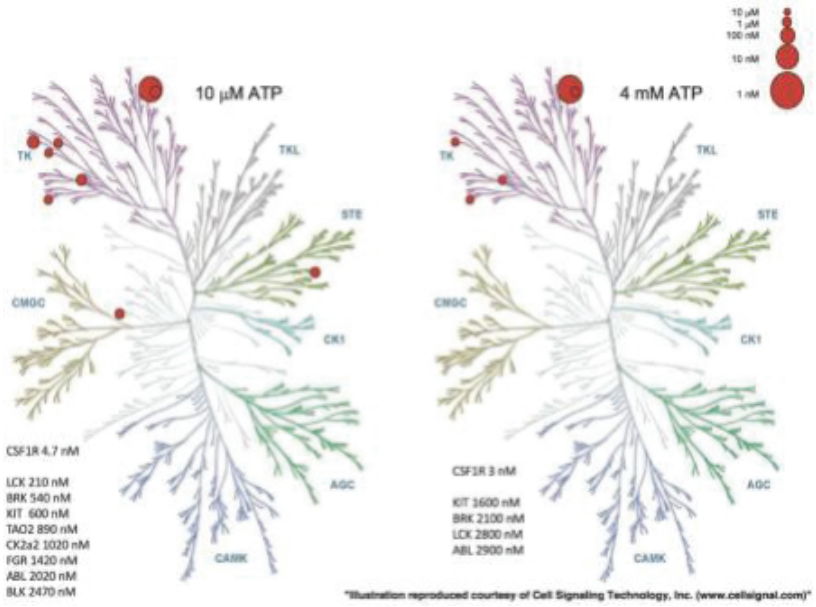
DCC-3014—A Potent and Highly Selective Inhibitor of CSF1R

DCC-3014 is an orally administered, potent and highly selective inhibitor of CSF1R, also known as FMS. DCC-3014 was designed to selectively bind to the unique switch pocket in CSF1R. It has greater than 100-fold selectivity for CSF1R over the closely related kinases FLT3, KIT, PDGFRα, PDGFRß and VEGFR2 and has an even greater selectivity for CSF1R over approximately 300 other human kinases that we tested.

We evaluated the selectivity of DCC-3014 for CSF1R in a standard biochemical assay, called a kinome screen assay. The kinome screen assay assesses the concentrations of DCC-3014 at which it inhibits CSF1R and other kinases. We conducted this assay in the presence of adenosine tri-phosphate, or ATP. ATP is essential for kinase activation, and the presence of higher concentrations of ATP increases the activity of kinases. The potency of traditional kinase inhibitors is often highly sensitive to increased ATP.

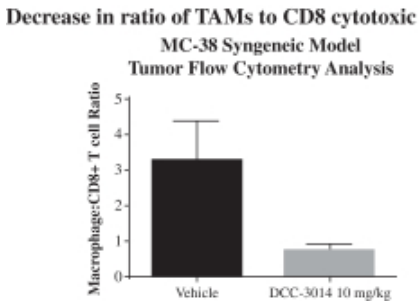
The following figure on the left depicts the results of the kinome screen assay using a 10 micromolar concentration of ATP, a standard low concentration typically used in this assay. Each kinase in the assay is depicted as a point at the end of a branch in the figure, and each kinase inhibited by DCC-3014 is depicted as a red dot and listed in the key to the figure. For each kinase that DCC-3014 inhibited in the kinome screen assay, we then assessed the inhibiting activity in a separate biochemical assay using a 4 millimolar concentration of ATP, equivalent to that present in human cells. The following figure on the right depicts the results of this second assay. As shown below, DCC-3014 inhibits CSF1R at concentrations much lower than the concentrations at which it inhibits other kinases, and this selective inhibition of CSF1R was more pronounced at the higher concentrations of ATP typically found in human cells. The increase in selectivity is a feature of kinase switch control inhibition, which is not affected by high ATP concentration for targeted kinases.

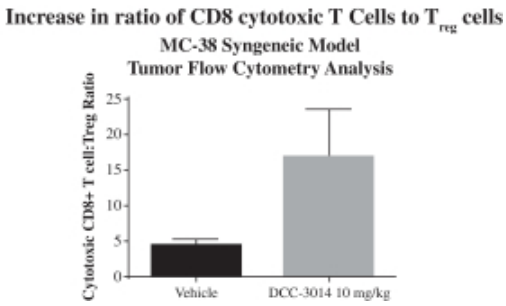
DCC-3014 Exhibits High Selectivity for CSF1R at Low, Screening Concentrations of ATP (10 µM) and Even Higher Selectively at Cellular Levels of ATP (4 mM)



CSF1R is a receptor for the ligands Macrophage Colony-stimulating Factor, or MCSF, and interleukin 34 (IL34). CSF1R controls the differentiation and function of macrophages, a type of white blood cell that engulfs and digests cellular debris, foreign substances, microbes and cancer cells. These macrophages are also programmed to either activate the immune system to fight a cancer (so-called M1 macrophages) or programmed to inactivate the immune system and promote tumor growth (so-called M2 macrophages). Pro-tumoral M2 macrophages have been shown to infiltrate certain tumors including cancers of the breast, cervix, pancreas, bladder and brain where poor prognosis correlates with the density of these TAMs. Tumors induce TAMs to suppress a natural immune response mediated by cytotoxic T-cells, a type of lymphocyte that would otherwise eradicate the tumor; a process known as macrophage checkpoints. In animal models of several cancers,

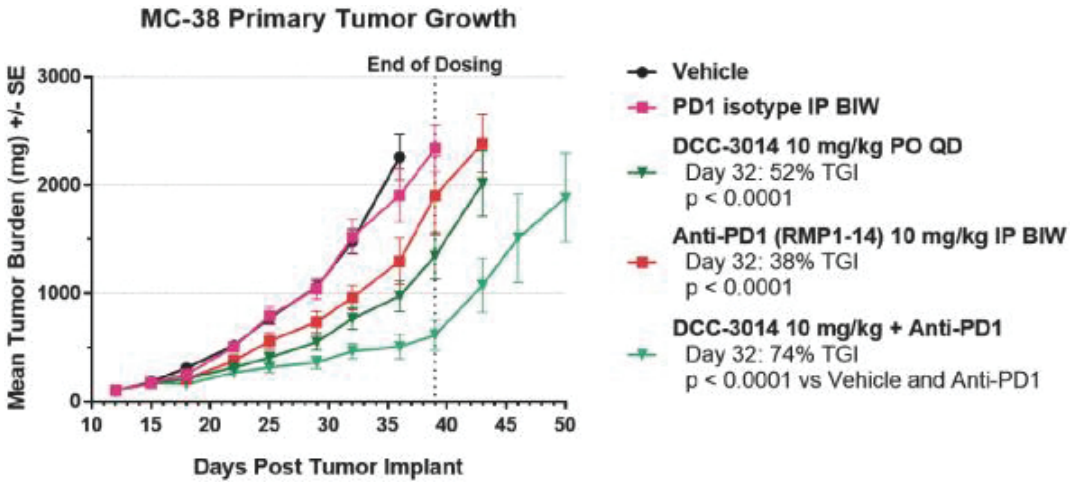
DCC-3014 has demonstrated potent macrophage checkpoint inhibition by blocking TAM-mediated immunosuppression as both a single agent and in combination with PD1 checkpoint inhibitors. TAMs are immunosuppressive and the greater the number in the tumor microenvironment, the lower the immune system's ability to attack the cancer. TAMs mediate their immunosuppressive function by decreasing the levels of the tumor-fighting CD8 cytotoxic T-cells. In the first figure below, control treatment with vehicle (placebo) results in a high TAM to CD8 T-cell ratio (black bar), indicative of high numbers of infiltrating TAMs that suppress the number of tumor fighting CD8 T-cells. This ratio is dramatically reversed (gray bar) after treatment with DCC-3014 to a tumor fighting state. In the second figure below, the ratio of the number of tumor fighting CD8 T-cells to the number of T_{reg} cells is illustrated. Whereas CD8 T-cells are tumor fighting immune cells, T_{reg} cells are immunosuppressive T-cells that, like TAMs, suppress the ability of the immune system to attack the cancer. Treatment with vehicle (placebo) results is a low ratio of CD8 T-cells to T_{reg} cells, resulting in an immunosuppressed immune cell infiltration to the tumor site (black bar). Treatment with DCC-3014 reverses this ratio to an enhanced tumor fighting state illustrated by the higher ratio of tumor fighting CD8 T-cells to immunosuppressive T_{reg} cells (gray bar).





The following figure illustrates macrophage checkpoint inhibition by DCC-3014 in a syngeneic colorectal cancer model, which has a high influx of TAMs. In this experiment, we assess the growth of murine colorectal cancer cells in mice treated with vehicle control, a control antibody, an anti-PD1 antibody, DCC-3014 or DCC-3014 in combination with an anti-PD1 antibody. The treatment period was 39 days. Tumor size was measured at 12 days after implantation and then every three or four days thereafter. The inhibiting effect of DCC-3014, dosed

alone or in combination with an anti-PD1 antibody, was greater than that of vehicle control, control antibody or anti-PD1 antibody treatment alone.



Unlike anti-CSF1R antibodies where over 10,000-fold increases in MCSF have been observed, DCC-3014 produces only a modest increase (less than 10-fold) in preclinical models potentially reducing the risk of rebound or other effects mediated by elevated MCSF. In addition, as an orally administered small molecule DCC-3014 offers significant flexibility in dose titration and a more convenient, patient-preferred route of administration compared to anti-CSF1R antibodies, which require injection. In February 2017, we began enrolling patients in a Phase 1 dose escalation trial of DCC-3014, which is expected to enroll up to 55 patients with advanced malignancies, including solid or hematologic malignancies where TAMs are known or suspected to contribute to the growth and spread of the cancer.

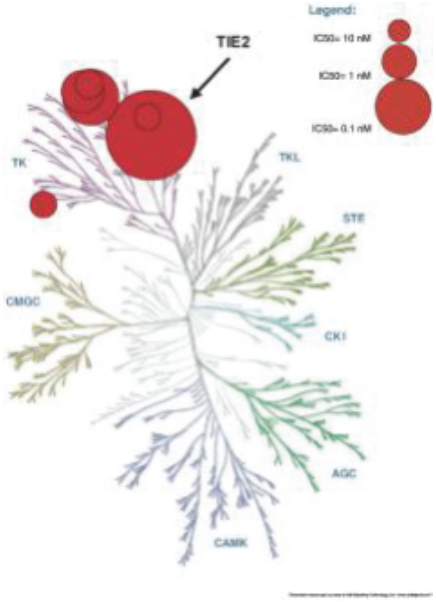
Rebastinib—TIE2 Inhibitor

Rebastinib is an orally administered, potent and selective inhibitor of the TIE2 immunokinase, the receptor for angiopoietins, an important family of vascular growth factors. Rebastinib binds potently into the switch pocket of TIE2, stabilizing the inhibitory switch and displacing the activation switch to block TIE2 signaling. TIE2 has an important role in regulating tumor angiogenesis, invasiveness, metastasis, and immunotolerance in a manner analogous to CSF1R. Whereas CSF1R is expressed on TAMs in certain cancers, there is a different and distinct population of protumoral M2 macrophages in which TIE2 is active, known as TIE2 expressing macrophages, or TEMs.

Rebastinib is in clinical development for the treatment of multiple solid tumors and in combination with chemotherapy in an investigator sponsored Phase 1b trial.

We evaluated the selectively of rebastinib for TIE2 in a kinome screen assay. The kinome screen assay assesses the concentrations of rebastinib at which it inhibits TIE2 and other kinases. Rebastinib has greater than 100-fold selectively for TIE2. The following figure depicts the results of the kinome screen assay using a 10 micromolar concentration of ATP, a standard low concentration typically used in this assay. Each kinase in the

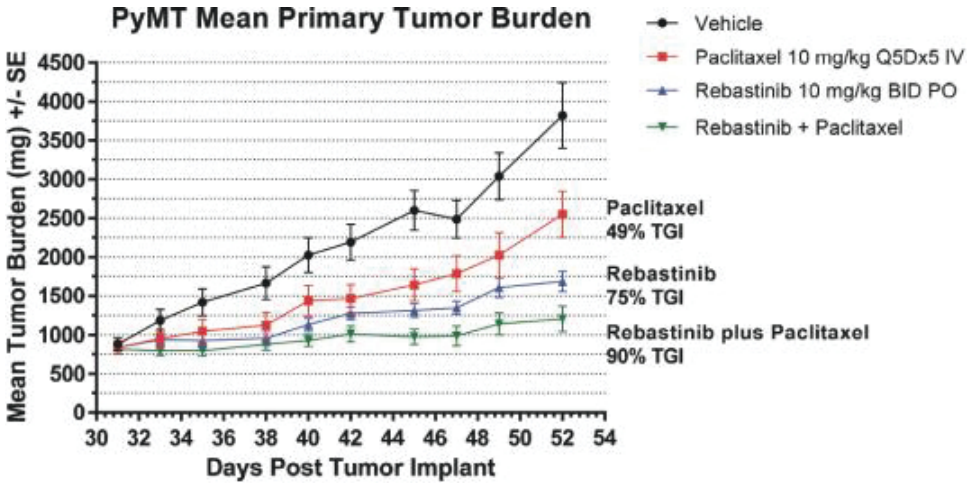
assay is depicted as a point at the end of a branch in the figure, and each kinase inhibited by rebastinib is depicted as a red dot and listed in the legend to the figure.



We evaluated the activity of rebastinib in a polyoma middle-T antigen, or PyMT, syngeneic mouse model in which murine breast cancer growth and mestasis can be assessed. In this model, tumor growth leads to metastasis, which is known to be modulated by TEMs. We examined multiple dosing schedules of rebastinib in combination with paclitaxel, an inhibitor of microtubule dynamics. In these preclinical studies, treatment with rebastinib significantly decreased tumor growth in the PyMT syngeneic mouse breast cancer model, reduced blood microvessel density and inhibited the TEMs. Treatment with rebastinib also significantly reduced the number of circulating tumor cells and metastases.

The figure below depicts the results of our evaluation of rebastinib in the PyMT syngeneic mouse breast cancer model. We treated the mice with vehicle, paclitaxel, rebastinib or a combination of rebastinib and paclitaxel. Tumor size was measured by weight at 31 days after initiation and every two or three days thereafter.

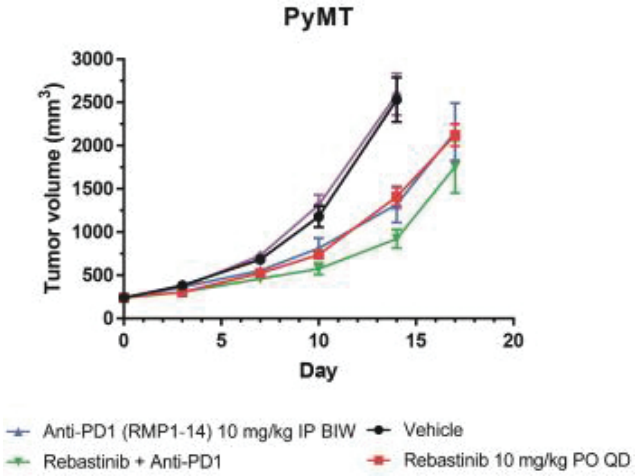
Treatment with rebastinib, either alone or in combination with paclitaxel, controlled tumor growth to a greater extent than vehicle or paclitaxel alone.



In addition to activity as a TIE2 inhibitor, a higher concentration of rebastinib also demonstrates activity as a switch control inhibitor of the kinases BCR-ABL1 and FLT3, which are believed to be involved in chronic or acute myeloid leukemia respectively. We investigated rebastinib in a Phase 1, single-agent trial in relapsed or refractory chronic (BCR-ABL+) or acute myeloid leukemia (FLT3-ITD+) patients. Although clinical activity was observed in this Phase 1 trial, clinical benefit was insufficient to justify continued development in either disease. However, PD analyses provided strong evidence of TIE2 inhibition in these patients. The primary objectives of this trial were to investigate the safety of rebastinib and establish the MTD. 57 patients received treatment with rebastinib. The trial established the MAD dose level as 200 mg BID and MTD as 150 mg BID. Dose limiting toxicities were dysarthria, or slurred or slow speech, muscle weakness, and peripheral neuropathy. Treatment emergent Grade 3 adverse events were reported by 35 patients (61%) and treatment emergent Grade 4 adverse events were reported by four patients (7%). One patient experienced an adverse event resulting in death, and the relationship to treatment is unknown. The most common adverse events (incidence of at least 30%) included dry mouth (47%), constipation (44%), fatigue (39%), muscular weakness (37%), headache (35%), nausea (33%) and dizziness (30%). Rebastinib exhibits 100-fold more potent inhibition of TIE2 compared to BCR-ABL in vitro. Our current clinical plans include exploring its potent TIE2 immunokinase inhibitory properties. In a Phase 1 clinical trial, biomarker data has demonstrated rebastinib-induced increases in the TIE2 ligand angiopoietin 2, secondary to TIE2 inhibition. Rebastinib is currently in an investigator sponsored Phase 1b trial in 24 patients with metastatic breast cancer in combination with paclitaxel or erubilin. Based on the more potent TIE2 inhibition of rebastinib, we are planning to explore the clinical development of rebastinib in multiple solid tumors in combination with chemotherapy or immuno-oncology agents at 50 mg and 100 mg BID doses of rebastinib.

The figure below illustrates macrophage checkpoint inhibition by rebastinib in the PyMT syngeneic mouse breast cancer model. In this experiment, we assessed the growth of murine breast cancer cells in mice treated with vehicle control, a control antibody, an anti-PD1 antibody, rebastinib or rebastinib in combination with an anti-PD1 antibody. The treatment period was 18 days. Tumor size was measured at 11 days after implantation and then every three or four days thereafter. The inhibiting effect of rebastinib dosed in combination with an

anti-PD1 antibody was greater than that of vehicle control, control antibody or anti-PD1 antibody or rebastinib treatment alone.



Platform Development and Preclinical Pipeline

We intend to leverage our proprietary kinase switch control inhibitor platform to advance additional drug candidates into clinical development. Our discovery programs are focused on novel immunokinases, kinases critical to autophagy and cancer cell metabolism, and kinases known to selectively drive cancer cell growth and survival. We are advancing the preclinical development of additional programs and expect to initiate further preclinical studies in one of these programs in 2018.

Commercial Operations

For DCC-2618, we intend to establish our own commercial and marketing organization in the United States and to selectively establish partnerships in markets outside the United States. We intend to build a specialist sales force to target physicians who are high prescribers of treatments for invasive solid tumors. We expect that the sales force will be supported by sales management, internal sales support, an internal marketing group and distribution support. Additionally, we expect that the sales and marketing teams will manage relationships with key accounts such as managed care organizations, group purchasing organizations, hospital systems, physician group networks, and government accounts. To develop the appropriate commercial infrastructure, we expect to invest significant amounts of financial and management resources, some of which will be committed prior to approval of DCC-2618, which we may never obtain.

For our other drug candidates in oncology, we intend to retain commercialization rights in the United States and leverage our commercial and marketing organization for DCC-2618, assuming we obtain regulatory approval in the United States. For certain drug candidates, such as DCC-3014 and rebastinib, we will consider entering into relationships with strategic partners that enable the expansion of the ongoing clinical development, while retaining significant value for our shareholders. These pharmaceutical company partnerships could focus on specific patient populations and their caregivers, on regional development, or on distribution and sales.

Manufacturing and Supply

We do not own or operate, and currently have no plans to establish, any manufacturing facilities. We currently rely on third parties for the manufacture of our drug candidates for preclinical and clinical testing, as well as for commercial manufacture of any drugs that we may commercialize. To date, we have obtained active

pharmaceutical ingredients, or APIs, and clinical drug supply for DCC-2618, DCC-3014 and rebastinib for our preclinical and ongoing and planned Phase 1 and Phase 3 testing from third-party manufacturers. We obtain our supplies from these manufacturers on a purchase order basis and do not have a long-term supply arrangement in place. We do not currently have arrangements in place for redundant supply for active pharmaceutical ingredients, or API, and drug product. We do not currently have a validated process or supply arrangements in place for the quantities of DCC-2618 that we anticipate we would require for our planned commercialization of DCC-2618, if approved. For all of our drug candidates, we intend to identify and qualify manufacturers to provide the API and drug product prior to submission of a new drug application to FDA or a marketing authorization application to the European Medicines Agency.

All our drug candidates are compounds of low molecular weight, generally called small molecules. As drug substances, they can be manufactured from readily available starting materials in reliable and reproducible synthetic processes that are amenable to scale-up. Some, including DCC-2618, may require specialized processing to optimize the drug product. We expect to continue to develop drug candidates that can be produced cost-effectively at contract manufacturing facilities.

We generally expect to rely on third parties for the manufacture of any companion diagnostics we may develop.

Competition

The pharmaceutical and biotechnology industries are characterized by rapidly advancing technologies, intense competition and a strong emphasis on proprietary drugs. While we believe that our technology, development experience and scientific knowledge provide us with competitive advantages, we face potential competition from many different sources, including major pharmaceutical, specialty pharmaceutical and biotechnology companies, academic institutions and governmental agencies and public and private research institutions. Any drug candidates that we successfully develop and commercialize will compete with existing drugs and new drugs that may become available in the future.

We compete in the segments of the pharmaceutical, biotechnology and other related markets that address inhibition of kinases in cancer and other rare genetic diseases. There are other companies working to develop therapies in the field of kinase inhibition for cancer and other diseases. These companies include divisions of large pharmaceutical companies and biotechnology companies of various sizes.

Many of the companies against which we are competing or against which we may compete in the future have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals and marketing approved drugs than we do. Mergers and acquisitions in the pharmaceutical, biotechnology and diagnostic 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 could see a reduction or elimination in our commercial opportunity if our competitors develop and commercialize drugs that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than any drugs that we or our collaborators may develop. Our competitors also may obtain FDA or other regulatory approval for their drugs more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we or our collaborators are able to enter the market. The key competitive factors affecting the success of all of our drug candidates, if approved, are likely to be their efficacy, safety, convenience, price, the effectiveness of companion diagnostics, the level of generic competition and the availability of reimbursement from government and other third-party payors.

If we receive approval for the drug candidates in our priority programs for the indications we are targeting, they will compete with the drugs discussed below and will likely compete with other drugs that are currently in clinical trials.

Competition for DCC-2618

We are initially developing DCC-2618 for GIST, gliomas, including GBM, and ASM patients with mutations in KIT and PDGFRα. Certain forms of lung and skin cancer and some leukemias express high levels of KIT or PDGFRα or contain mutations in these kinases and we also intend to evaluate the potential clinical benefit of DCC-2618 in patients with these cancers.

In GIST, the current approved standards of care for unresectable or metastatic patients are first-line imatinib, followed by second-line sunitinib upon imatinib progression, followed by third-line regorafenib upon sunitinib progression. If DCC-2618 receives marketing approval for GIST, we may also face competition from drug candidates in clinical trials including Arog Pharmaceuticals, Inc., Blueprint Medicines Corporation, Plexxikon Inc., a wholly owned subsidiary of Daiichi Sankyo Company, Limited, ARIAD Pharmaceuticals, Inc., a wholly owned subsidiary of Takeda Pharmaceuticals U.S.A., Bristol-Myers Squibb Company and Xencor, Inc.

For ASM, the only approved drugs that inhibit KIT are imatinib for patients without the KIT D816V mutation or mutational status unknown and midostaurin (Novartis AG). If DCC-2618 receives marketing approval, in addition to midostaurin it may face competition from other drug candidates in clinical trials for ASM, including drug candidates from AB Science S.A., Blueprint Medicines Corporation, Plexxikon Inc., a wholly owned subsidiary of Daiichi Sankyo Company, Limited.

Competition for Rebastinib

We are initially developing rebastinib, a TIE2 inhibitor, to control immunosuppressive tumor-associated macrophages expressing TIE2 to assist the immune system in targeting tumor cells. While rebastinib is a novel molecule, we may face competition from drug candidates in clinical trials that are not TIE2 inhibitors, but aim to achieve similar effects on the immune system. These include small molecule drug candidates in clinical trials from Array BioPharma Inc., Novartis AG, Plexxikon Inc., a wholly owned subsidiary of Daiichi Sankyo Company, Limited, and from antibody therapeutics from Amgen Inc., Eli Lilly and Company, Roche Holding Ltd, Five Prime Therapeutics, Inc., Novartis AG, and Syndax Pharmaceuticals, Inc.

Competition for DCC-3014

We are initially developing DCC-3014, an inhibitor of CSF1R, to control immunosuppressive tumor-associated macrophages to assist the immune system in targeting tumor cells. If DCC-3014 receives marketing approval, it may face competition from other drug candidates in clinical trials that target CSF1R, including small molecule drug candidates in clinical trials from Array BioPharma, Inc., Novartis AG, Plexxikon Inc., a wholly owned subsidiary of Daiichi Sankyo Company, Limited, and from antibody therapeutics including those from Amgen Inc., Eli Lilly and Company, Roche Holding Ltd, Five Prime Therapeutics, Inc., Novartis AG, and Syndax Pharmaceuticals, Inc.

Intellectual Property

We strive to protect the proprietary technologies that we believe are important to our business, including pursuing and maintaining patent protection intended to cover the composition of matter of our drug candidates, for example, DCC-2618, DCC-3014 and rebastinib, their methods of use, related technologies and other inventions that are important to our business. In addition to patent protection, we also rely on trade secrets to protect aspects of our business that are not amenable to, or that we do not consider appropriate for, patent protection, including our proprietary kinase switch control inhibitor platform.

Our commercial success depends in part upon our ability to obtain and maintain patent and other proprietary protection for our drug candidates and other commercially important technologies, inventions and know-how related to our business, defend and enforce our intellectual property rights, in particular, our patent rights, preserve the confidentiality of our trade secrets and operate without infringing valid and enforceable intellectual property rights of others.

The patent positions for biotechnology and pharmaceutical companies like us are generally uncertain and can involve complex legal, scientific and factual issues. We cannot predict whether the patent applications we are currently pursuing will issue as patents in any particular jurisdiction or whether the claims of any issued patents will provide sufficient proprietary protection from competitors. In addition, the coverage claimed in a patent application can be significantly reduced before a patent is issued, and its scope can be reinterpreted and even challenged after issuance. As a result, we cannot guarantee that any of our drug candidates will be protected or remain protectable by enforceable patents. Moreover, any patents that we hold may be challenged, circumvented or invalidated by third parties. For more information regarding the risks related to our intellectual property please see “Risk Factors—Risks Related to Our Intellectual Property.”

As of February 28, 2018, we owned at least 11 issued U.S. patents, 62 issued foreign patents, four pending U.S. patent applications and 46 pending foreign patent applications.

With regard to DCC-2618, as of February 28, 2018, we owned two issued U.S. patents with composition of matter and method of use claims directed to DCC-2618 and its use. The issued U.S. patents are expected to expire in 2032, without taking a potential patent term extension into account. In addition, we have patents that have been granted in various jurisdictions including Australia, Europe, Japan, South Korea, and Singapore, which are expected to expire in 2032, without taking potential patent term extensions into account, and at least nine pending patent applications in various other countries and regions in North America, South America and Asia, which, if issued, are expected to expire in 2032, without taking potential patent term extensions into account.

With regard to DCC-3014, as of February 28, 2018, we owned one issued U.S. patent with composition of matter and method of use claims directed to DCC-3014 and its use. The issued U.S. patent is expected to expire in 2034, without taking a potential patent term extension into account. In addition, we have at least 17 patents that have been granted in various European countries (e.g., Austria, Belgium, France, Germany, the United Kingdom, Spain, Italy and Denmark), China and Japan, and at least 14 pending patent applications in various other countries and regions in North America, South America, and Asia, which, if issued, are expected to expire in 2034, without taking potential patent term extensions into account.

With regard to rebastinib, as of February 28, 2018, we own three issued U.S. patents with composition of matter and method of use claims directed to rebastinib and its use. The issued U.S. patents are expected to expire in between 2027 and 2034, without taking a potential patent term adjustment or extension into account. In addition, we own approximately 15 patents that have been granted in various different countries including Australia, Canada, China, Europe, Hong Kong, Indonesia, Israel, Japan, South Korea, Mexico, Russian Federation, Philippines, and Singapore, which are expected to expire in 2027, without taking potential patent term adjustments or extensions into account, and approximately ten pending patent applications in various other countries and regions, for example in Australia, Brazil, China, India, and Indonesia, which, if issued, are expected to expire in 2027 and 2033, without taking potential patent term adjustments or extensions into account.

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

In the United States, the term of a patent covering an FDA-approved drug may, in certain cases, be eligible for a patent term extension under the Hatch-Waxman Act as compensation for the loss of patent term during FDA regulatory review process. The period of extension may be up to five years, but cannot extend the

remaining term of a patent beyond a total of 14 years from the date of product approval. Only one patent among those eligible for an extension and only those claims covering the approved drug, a method for using it, or a method for manufacturing it may be extended. Similar provisions are available in Europe and in certain other jurisdictions to extend the term of a patent that covers an approved drug. It is possible that issued U.S. patents covering DCC-2618, DCC-3014, and rebastinib may be entitled to patent term extensions. If our drug candidates receive FDA approval, we intend to apply for patent term extensions, if available, to extend the term of patents that cover the approved drug candidates. We also intend to seek patent term extensions in any jurisdictions where they are available, however, there is no guarantee that the applicable authorities, including FDA, will agree with our assessment of whether such extensions should be granted, and even if granted, the length of such extensions.

In addition to patent protection, we also rely on trade secret protection for our proprietary information that is not amenable to, or that we do not consider appropriate for, patent protection, including, for example, our proprietary kinase switch control inhibitor platform and certain aspect of our manufacturing processes and aspects of our kinase switch control platform. However, trade secrets can be difficult to protect. Although we take steps to protect our proprietary information, including restricting access to our premises and our confidential information, as well as entering into agreements with our employees, consultants, advisors and potential collaborators, such individuals may breach such agreements and disclose our proprietary information including our trade secrets, and we may not be able to obtain adequate remedies for such breaches. In addition, third parties may independently develop the same or similar proprietary information or may otherwise gain access to our proprietary information. As a result, we may be unable to meaningfully protect our trade secrets and proprietary information. For more information regarding the risks related to our intellectual property please see “Risk Factors—Risks Related to Our Intellectual Property.”

Government Regulation

Government authorities in the United States at the federal, state and local level and in other countries extensively regulate, among other things, the research and clinical development, testing, manufacture, quality control, approval, labeling, packaging, storage, record-keeping, promotion, advertising, distribution, post-approval monitoring and reporting, marketing, pricing and export and import of drug products, such as those we are developing. Generally, before a new drug can be marketed, considerable data demonstrating its quality, safety and efficacy must be obtained, organized into a format specific to each regulatory authority, submitted for review and approved by the regulatory authority.

Drugs are also subject to other federal, state and local statutes and regulations. The process of obtaining regulatory approvals and the subsequent compliance with appropriate federal, state, local and foreign statutes and regulations require the expenditure of substantial time and financial resources. Failure to comply with the applicable regulatory requirements at any time during the product development process, approval process or after approval, may subject an applicant to administrative or judicial sanctions. These sanctions could include, among other actions, the regulatory authority’s refusal to approve pending applications, withdrawal of an approval, clinical holds, untitled or warning letters, voluntary product recalls or withdrawals from the market, product seizures, total or partial suspension of production or distribution injunctions, disbarment, fines, refusals of government contracts, restitution, disgorgement, or civil or criminal penalties. Any agency or judicial enforcement action could have a material adverse effect on us.

U.S. Drug Development

In the United States, FDA regulates drugs under the Federal Food, Drug, and Cosmetic Act, or FDCA, and its implementing regulations. Drugs are also subject to other federal, state and local statutes and regulations. Our drug candidates must be approved by FDA through the NDA process before they may be legally marketed in the

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

- completion of extensive preclinical, sometimes referred to as nonclinical laboratory tests, animal studies and formulation studies all performed in accordance with applicable regulations, including FDA's good laboratory practice, or GLP, regulations;
- submission to FDA of an investigational new drug application, or IND, which must become effective before human clinical trials may begin and must be updated annually;
- performance of adequate and well-controlled human clinical trials in accordance with applicable IND and other clinical trial-related regulations, sometimes referred to as good clinical practices, or GCPs, to establish the safety and efficacy of the proposed drug for its proposed indication;
- submission to FDA of an NDA, for a new drug;
- a determination by FDA within 60 days of its receipt of an NDA to file the NDA for review;
- satisfactory completion of an FDA pre-approval inspection of the manufacturing facility or facilities at which the API and finished drug product are produced to assess compliance with FDA's current good manufacturing practice requirements, or cGMP;
- potential FDA audit of the clinical trial sites that generated the data in support of the NDA; and
- FDA review and approval of the NDA prior to any commercial marketing or sale of the drug in the United States.

The data required to support an NDA are generated in two distinct development stages: preclinical and clinical. For new chemical entities, the preclinical development stage generally involves synthesizing the active component, developing the formulation and determining the manufacturing process, as well as carrying out non-human toxicology, pharmacology and drug metabolism studies in the laboratory, which support subsequent clinical testing. The conduct of the preclinical tests must comply with federal regulations, including GLPs. The sponsor must submit the results of the preclinical tests, together with manufacturing information, analytical data, any available clinical data or literature and a proposed clinical protocol, to FDA as part of the IND. An IND is a request for authorization from FDA to administer an investigational drug product to humans. The central focus of an IND submission is on patient safety and the general investigational plan and the protocol(s) for human trials. The IND automatically becomes effective 30 days after receipt by FDA, unless FDA raises concerns or questions regarding the proposed clinical trials and places the IND on clinical hold within that 30-day time period. In such a case, the IND sponsor and FDA must resolve any outstanding concerns or questions before the clinical trial can begin. FDA also may impose a partial clinical hold that would limit a trial, for example, to certain doses or for a certain length of time or to a certain number of subjects. FDA may also impose clinical holds on a drug candidate at any time before or during clinical trials due to safety concerns or non-compliance. Accordingly, we cannot be sure that submission of an IND will result in FDA allowing clinical trials to begin, or that, once begun, issues will not arise that could cause the trial to be suspended or terminated.

The clinical stage of development involves the administration of the drug candidate to human subjects under the supervision of qualified investigators, generally physicians not employed by or under the trial sponsor's control, in accordance with GCPs, which include the requirement that all research subjects provide their informed consent for their participation in any clinical trial. Clinical trials are conducted under protocols detailing, among other things, the objectives of the clinical trial, dosing procedures, subject selection and exclusion criteria, and the parameters to be used to monitor subject safety and assess efficacy. Each protocol, and any subsequent amendments to the protocol, must be submitted to FDA as part of the IND. Further, each clinical trial must be reviewed and approved by an independent institutional review board, or IRB, at or servicing each institution at which the clinical trial will be conducted. An IRB is charged with protecting the welfare and rights of trial participants and considers such items as whether the risks to individuals participating in the clinical trials are minimized and are reasonable in relation to anticipated benefits. The IRB also approves the informed consent

form that must be provided to each clinical trial subject or his or her legal representative and must monitor the clinical trial until completed. There are also requirements governing the reporting of ongoing clinical trials and completed clinical trial results to public registries.

Clinical trials are generally conducted in three sequential phases that may overlap or be combined, known as Phase 1, Phase 2 and Phase 3 trials. Phase 1 trials generally involve a small number of healthy volunteers who are initially exposed to a single dose and then multiple doses of the drug candidate. The primary purpose of these clinical trials is to assess the metabolism, pharmacologic action, side effects, tolerability and safety of the drug. Phase 2 clinical trials typically involve studies in disease-affected patients to determine the dose required to produce the desired benefits. At the same time, safety and further PK and PD information is collected, as well as identification of possible adverse effects and safety risks and preliminary evaluation of efficacy. Phase 3 trials generally involve large numbers of patients at multiple sites (from several hundred to several thousand subjects) and are designed to provide the data necessary to demonstrate the efficacy of the drug for its intended use, its safety in use, and to establish the overall benefit/risk relationship of the drug and provide an adequate basis for drug approval. The duration of treatment is often extended to mimic the actual use of a drug during marketing. Generally, two adequate and well-controlled Phase 3 trials are required by FDA for approval of an NDA.

A pivotal study is a clinical study that adequately meets regulatory agency requirements for the evaluation of a drug candidate's efficacy and safety such that it can serve as the primary basis for approval of the drug. Generally, pivotal studies are also Phase 3 studies but may be Phase 2 studies if the trial design provides a well-controlled and reliable assessment of clinical benefit, particularly in situations where there is an unmet medical need. Post-approval trials, sometimes referred to as Phase 4 clinical trials, may be conducted after initial marketing approval. These trials are used to gain additional experience from the treatment of patients in the intended therapeutic indication. In certain instances, FDA may mandate the performance of Phase 4 clinical trials.

Progress reports detailing the results of the clinical trials must be submitted at least annually to FDA and written IND safety reports must be submitted to FDA and the investigators for serious and unexpected adverse reactions, any finding from other clinical studies, tests in laboratory animals, or in vitro testing that suggests a significant risk for human subjects, or any clinically important increase in the rate of a serious suspected adverse reaction over that listed in the protocol or investigator brochure. Phase 1, Phase 2 and Phase 3 trials may not be completed successfully within any specified period, if at all. FDA, the IRB, or the clinical trial sponsor may suspend or terminate a clinical trial at any time on various grounds, including a finding that the research subjects or patients are being exposed to an unacceptable health risk. Similarly, an IRB can suspend or terminate approval of a clinical trial at its institution if the clinical trial is not being conducted in accordance with the IRB's requirements or if the drug 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 a data safety monitoring board or committee. This group provides authorization for whether or not a trial may move forward at designated check points based on access to certain data from the trial. We may also suspend or terminate a clinical trial based on evolving business objectives and/or competitive climate. Concurrent with clinical trials, companies usually complete additional animal studies and must also develop additional information about the chemistry and physical characteristics of the drug as well as finalize a process for manufacturing the drug in commercial quantities in accordance with cGMP requirements. The manufacturing process must be capable of consistently producing quality batches of the drug candidate and, among other things, cGMPs impose extensive procedural, substantive and recordkeeping requirements to ensure and preserve the long-term stability and quality of the final drug product. Additionally, appropriate packaging must be selected and tested and stability studies must be conducted to demonstrate that the drug candidate does not undergo unacceptable deterioration over its shelf life.

NDA and FDA Review Process

Following trial completion, trial data are analyzed to assess safety and efficacy. The results of preclinical studies and clinical trials are then submitted to FDA as part of an NDA, along with proposed labeling for the drug

and information about the manufacturing process and facilities that will be used to ensure drug quality, results of analytical testing conducted on the chemistry of the drug, and other relevant information. The NDA is a request for approval to market the drug and must contain adequate evidence of safety and efficacy, which is demonstrated by extensive preclinical and clinical testing. Data may come from company-sponsored clinical trials intended to test the safety and efficacy of a use of a drug, or from a number of alternative sources, including studies initiated by investigators. To support marketing approval, the data submitted must be sufficient in quality and quantity to establish the safety and efficacy of the investigational drug product for a particular indication or indications to the satisfaction of FDA. The submission of an NDA is subject to the payment of substantial user fees; a waiver of such fees may be obtained under certain limited circumstances. FDA approval of an NDA must be obtained before a drug may be offered for sale in the United States.

Under the Prescription Drug User Fee Act, or PDUFA, as amended, each NDA must be accompanied by a user fee. FDA adjusts the PDUFA user fees on an annual basis. According to FDA's fee schedule, effective from October 1, 2017 through September 30, 2018, the user fee for an application requiring clinical data, such as an original NDA, is $2,421,495. PDUFA also imposes an annual prescription drug product program fee for human drugs ($304,162). Fee waivers or reductions are available in certain circumstances, including a waiver of the application fee for the first application filed by a small business. Additionally, no user fees are assessed on NDAs for products designated as orphan drugs, unless the product also includes a non-orphan indication.

Within 60 days following submission of an original NDA, FDA reviews the application to determine if it is substantially complete before the agency accepts it for filing. FDA may refuse to file any NDA that it deems incomplete or not properly reviewable at the time of submission, including for failure to pay required fees, and may request additional information. In this event, the application must be resubmitted with the additional information. The resubmitted application also is subject to review before FDA accepts it for filing. FDA typically makes a decision on whether to accept an NDA for filing within 60 days of receipt. Once the submission is accepted for filing, FDA begins an in-depth, substantive review of the NDA. Under the performance goals established under PDUFA, FDA has agreed to review 90% of standard NDAs for new molecular entities (NMEs) in ten months from the filing date and 90% of priority NME NDAs in six months from the filing date. The goals for reviewing standard and priority non-NME NDAs are ten months and six months, respectively, measured from the submission date of the application. FDA does not always meet its PDUFA goal dates for standard and priority NDAs, and the review process is often significantly extended by FDA requests for additional information or clarification.

After the NDA submission is accepted for filing, FDA reviews the NDA to determine, among other things, whether the proposed drug is safe and effective for its intended use, and whether the drug is being manufactured in accordance with cGMP to assure and preserve the drug's identity, strength, quality and purity. FDA may refer applications for novel drugs or drug candidates that present difficult questions of safety or efficacy to an advisory committee, typically a panel that includes clinicians and other experts, for review, evaluation and a recommendation as to whether the application should be approved and under what conditions. FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions. In the course of its review, FDA may re-analyze the clinical trial data, which could result in extensive discussions between FDA and the applicant during the review process. The review and evaluation of an NDA by FDA is extensive and time consuming and may take longer than originally planned to complete, and we may not receive a timely approval, if at all.

Before approving an NDA, FDA typically conducts a pre-approval inspection of the manufacturing facilities for the new drug to determine whether they comply with cGMPs. FDA will not approve the drug unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the drug within required specifications. In addition, before approving an NDA, FDA may also audit data from clinical trials to ensure compliance with GCP requirements. After FDA evaluates the application, manufacturing process and manufacturing facilities where the drug product and/or its API will be produced, it may issue an approval letter or a Complete Response Letter. An approval letter

authorizes commercial marketing of the drug with specific prescribing information for specific indications. A Complete Response Letter indicates that the review cycle of the application is complete and the application is not ready for approval. A Complete Response Letter usually describes all of the specific deficiencies in the NDA identified by FDA. The Complete Response Letter may require additional clinical data and/or an additional pivotal clinical trial(s), and/or other significant, expensive and time-consuming requirements related to clinical trials, preclinical studies or manufacturing. If a Complete Response Letter is issued, the applicant may either resubmit the NDA, addressing all of the deficiencies identified in the letter, challenge the determination set forth in the letter by requesting a hearing, or withdraw the application. Even if such data and information are submitted, FDA may ultimately decide that the NDA does not satisfy the criteria for approval. Data obtained from clinical trials are not always conclusive and FDA may interpret data differently than we interpret the same data.

There is no assurance that FDA will ultimately approve a drug product for marketing in the United States and we may encounter significant difficulties or costs during the review process. If a drug receives marketing approval, the approval may be significantly limited to specific diseases, dosages, or patient subgroups, or the indications for use may otherwise be limited, which could restrict the commercial value of the drug. Further, FDA may require that certain contraindications, warnings, precautions, or adverse events be included in the drug labeling or may condition the approval of the NDA on other changes to the proposed labeling, development of adequate controls and specifications, or a commitment to conduct post-market testing or clinical trials and surveillance to monitor the effects of approved drugs. For example, FDA may require Phase 4 testing which involves clinical trials designed to further assess a drug's safety and may require testing and surveillance programs to monitor the safety of approved drugs that have been commercialized. FDA may also place other conditions on approvals including the requirement for a Risk Evaluation and Mitigation Strategy, or REMS, to assure the safe use of the drug. If FDA concludes a REMS is needed, the sponsor of the NDA must submit a proposed REMS. FDA will not approve the NDA without an approved REMS, if required. A REMS could include medication guides, physician communication plans, or elements to assure safe use, such as restricted distribution methods, patient registries and other risk minimization tools. Any of these limitations on approval or marketing could restrict the commercial promotion, distribution, prescription or dispensing of drugs. Drug approvals may be withdrawn for non-compliance with regulatory standards or if problems occur following initial marketing.

Special FDA Expedited Review and Approval Programs

FDA has various programs, including Fast Track Designation, priority review, accelerated approval and Breakthrough Therapy Designation, that are intended to expedite or simplify the process for the development and FDA review of drugs that are intended for the treatment of serious or life-threatening diseases or conditions and demonstrate the potential to address unmet medical needs. The purpose of these programs is to provide important new drugs to patients earlier than under standard FDA review procedures. To be eligible for a Fast Track Designation, FDA must determine, based on the request of a sponsor, that a drug is intended to treat a serious or life-threatening disease or condition and demonstrates the potential to address an unmet medical need. FDA will determine that a product will fill an unmet medical need if it will provide a therapy where none exists or provide a therapy that may be potentially superior to existing therapy based on efficacy or safety factors.

FDA may give a priority review designation to drugs intended to treat serious conditions that offer major advances in treatment, or provide a treatment where no adequate therapy exists. A priority review means that the goal for FDA to review an application is six months, rather than the standard review of ten months under current PDUFA guidelines. These six and ten-month review periods are measured from the "filing" date rather than the receipt date for NDAs for new molecular entities, which typically adds approximately two months to the timeline for review and decision from the date of submission. Most products that are eligible for Fast Track Designation are also likely to be considered appropriate to receive a priority review.

In addition, drugs studied for their safety and effectiveness in treating serious or life-threatening illnesses and that provide meaningful therapeutic benefit over existing treatments may receive accelerated approval and

may be approved on the basis of adequate and well-controlled clinical trials establishing that the drug has an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity or prevalence of the condition and the availability or lack of alternative treatments. As a condition of approval, FDA may require a sponsor of a drug receiving accelerated approval to perform post-marketing studies to verify and describe the predicted effect on irreversible morbidity or mortality or other clinical endpoint and to submit promotional materials for preapproval and pre-use review. In addition, the drug may be subject to accelerated withdrawal procedures.

Moreover, a sponsor can request designation of a drug candidate as a "breakthrough therapy." A breakthrough therapy is defined as a drug that is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. Drugs designated as breakthrough therapies are also eligible for accelerated approval. FDA must take certain actions, such as holding timely meetings and providing advice, intended to expedite the development and review of an application for approval of a breakthrough therapy.

Even if a product qualifies for one or more of these programs, FDA may later decide that the product no longer meets the conditions for qualification or decide that the time period for FDA review or approval will not be shortened. Furthermore, Fast Track Designation, priority review, accelerated approval and Breakthrough Therapy Designation, do not change the standards for approval and may not ultimately expedite the development or approval process.

Pediatric Trials

Under the Pediatric Research Equity Act, or PREA, as amended, an NDA or supplement to an NDA for a drug that includes a new active ingredient, new indication, new dosage form, new dosing regimen or new route of administration must contain data to assess the safety and efficacy of the drug for the claimed indications in all relevant pediatric subpopulations and to support dosing and administration for each pediatric subpopulation for which the drug is safe and effective. FDA may grant deferrals for submission of data or full or partial waivers.

A sponsor who is planning to submit a marketing application for a drug subject to PREA must submit an initial Pediatric Study Plan, or PSP, within sixty days of an end-of-Phase 2 meeting or as may be agreed between the sponsor and FDA. The initial PSP must include an outline of the pediatric study or studies that the sponsor plans to conduct, including study objectives and design, age groups, relevant endpoints and statistical approach, or a justification for not including such detailed information, and any request for a deferral of pediatric assessments or a full or partial waiver of the requirement to provide data from pediatric studies along with supporting information. FDA and the sponsor must reach agreement on the PSP. A sponsor can submit amendments to an agreed-upon initial PSP at any time if changes to the pediatric plan need to be considered based on data collected from preclinical studies, early phase clinical trials, and/or other clinical development programs.

Post-Marketing Requirements

Following approval of a new drug, a pharmaceutical company and the approved drug are subject to continuing regulation by FDA, including, among other things, registration and listing, monitoring and recordkeeping activities, reporting to the applicable regulatory authorities of adverse experiences with the drug, providing the regulatory authorities with updated safety and efficacy information, drug sampling and distribution requirements, and complying with promotion and advertising requirements, which include, among others, standards for direct-to-consumer advertising, restrictions on promoting drugs for uses or in patient populations

that are not described in the drug's approved labeling (known as "off-label use"), limitations on industry-sponsored scientific and educational activities, and requirements for promotional activities involving the internet. Although physicians may prescribe legally available drugs for off-label uses. FDA takes the position that manufacturers may not market or promote such off-label uses. Modifications or enhancements to the drug or its labeling or changes of the site or process of manufacture are often subject to the approval of FDA and other regulators, which may or may not be received or may result in a lengthy review process.

Prescription drug advertising is subject to federal, state and foreign regulations. In the United States, FDA regulates prescription drug promotion, including direct-to-consumer advertising. Prescription drug promotional materials must be submitted to FDA in conjunction with their first use. Any distribution of prescription drugs and pharmaceutical samples must comply with the U.S. Prescription Drug Marketing Act, or the PDMA, a part of the FDCA.

In the United States, once a drug is approved, its manufacture is subject to comprehensive and continuing regulation by FDA. FDA regulations require that drugs be manufactured in specific facilities per the NDA approval and in accordance with cGMP. We rely, and expect to continue to rely, on third parties for the production of clinical and commercial quantities of our drugs in accordance with cGMP regulations. cGMP regulations require among other things, quality control and quality assurance as well as the corresponding maintenance of records and documentation and the obligation to investigate and correct any deviations from cGMP. Drug manufacturers and other entities involved in the manufacture and distribution of approved drugs are required to register their establishments with FDA and certain state agencies, and are subject to periodic unannounced inspections by FDA and certain state agencies for compliance with cGMP and other laws. Accordingly, manufacturers must continue to expend time, money, and effort in the area of production and quality control to maintain cGMP compliance. These regulations also impose certain organizational, procedural and documentation requirements with respect to manufacturing and quality assurance activities. NDA holders using contract manufacturers, laboratories or packagers are responsible for the selection and monitoring of qualified firms, and, in certain circumstances, qualified suppliers to these firms. These firms and, where applicable, their suppliers are subject to inspections by FDA at any time, and the discovery of violative conditions, including failure to conform to cGMP, could result in enforcement actions that interrupt the operation of any such facilities or the ability to distribute drugs manufactured, processed or tested by them. Discovery of problems with a drug after approval may result in restrictions on a drug, manufacturer, or holder of an approved NDA, including, among other things, recall or withdrawal of the drug from the market, and may require substantial resources to correct.

FDA also may require post-approval testing, sometimes referred to as Phase 4 testing, risk minimization action plans and post-marketing surveillance to monitor the effects of an approved drug or place conditions on an approval that could restrict the distribution or use of the drug. Discovery of previously unknown problems with a drug or the failure to comply with applicable FDA requirements can have negative consequences, including adverse publicity, judicial or administrative enforcement, untitled or warning letters from FDA, mandated corrective advertising or communications with doctors, and civil or criminal penalties, among others. Newly discovered or developed safety or effectiveness data may require changes to a drug's approved labeling, including the addition of new warnings and contraindications, and also may require the implementation of other risk management measures, including a REMS or the conduct of post-marketing studies to assess a newly discovered safety issue. Also, new government requirements, including those resulting from new legislation, may be established, or FDA's policies may change, which could delay or prevent regulatory approval of our drugs under development.

Other Regulatory Matters

Manufacturing, sales, promotion and other activities following drug approval are also subject to regulation by numerous regulatory authorities in addition to FDA, including, in the United States, the Centers for Medicare & Medicaid Services, other divisions of the Department of Health and Human Services, the Drug

Enforcement Administration for controlled substances, the Consumer Product Safety Commission, the Federal Trade Commission, the Occupational Safety & Health Administration, the Environmental Protection Agency and state and local governments. In the United States, sales, marketing and scientific/educational programs must also comply with state and federal fraud and abuse laws. Pricing and rebate programs must comply with the Medicaid rebate requirements of the U.S. Omnibus Budget Reconciliation Act of 1990 and more recent requirements in the Patient Protection and Affordable Care Act as amended by the Health Care and Education Reconciliation Act of 2010, or ACA. If drugs are made available to authorized users of the Federal Supply Schedule of the General Services Administration, additional laws and requirements apply. The handling of any controlled substances must comply with the U.S. Controlled Substances Act and Controlled Substances Import and Export Act. Drugs must meet applicable child-resistant packaging requirements under the U.S. Poison Prevention Packaging Act. Manufacturing, sales, promotion and other activities are also potentially subject to federal and state consumer protection and unfair competition laws.

We are subject to numerous foreign, federal, state and local 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. In addition, our leasing and operation of real property may subject us to liability pursuant to certain U.S. environmental laws and regulations, under which current or previous owners or operators of real property and entities that disposed or arranged for the disposal of hazardous substances may be held strictly, jointly and severally liable for the cost of investigating or remediating contamination caused by hazardous substance releases, even if they did not know of and were not responsible for the releases.

The distribution of pharmaceutical drugs is subject to additional requirements and regulations, including extensive record-keeping, licensing, storage and security requirements intended to prevent the unauthorized sale of pharmaceutical drugs.

The failure to comply with regulatory requirements subjects firms to possible legal or regulatory action. Depending on the circumstances, failure to meet applicable regulatory requirements can result in criminal prosecution, fines or other penalties, injunctions, voluntary recall or seizure of drugs, total or partial suspension of production, denial or withdrawal of product approvals, or refusal to allow a firm to enter into supply contracts, including government contracts. In addition, even if a firm complies with FDA and other requirements, new information regarding the safety or efficacy of a product could lead FDA to modify or withdraw product approval. Prohibitions or restrictions on sales or withdrawal of future products marketed by us could materially affect our business in an adverse way.

Changes in regulations, statutes or the interpretation of existing regulations could impact our business in the future by requiring, for example: (i) changes to our manufacturing arrangements; (ii) additions or modifications to product labeling; (iii) the recall or discontinuation of our products; or (iv) additional record-keeping requirements. If any such changes were to be imposed, they could adversely affect the operation of our business.

U.S. Patent Term Restoration and Marketing Exclusivity

Depending upon the timing, duration and specifics of FDA approval of our drug candidates, some of our U.S. patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, commonly referred to as the Hatch-Waxman Amendments. The Hatch-Waxman Amendments permit a patent restoration term of up to five years as compensation for patent term lost during product development and FDA regulatory review process. However, patent term restoration cannot extend the remaining term of a patent beyond a total of 14 years from the product's approval date. The patent term restoration period is generally one-half the time between the effective date of an IND and the submission date of an NDA plus the time between the submission date of an NDA and the approval of that application. Only one patent may be extended and only those claims covering the approved drug, a method for using it, or a method for manufacturing it may be extended and the application for the extension must be submitted prior to the expiration of the patent. The USPTO, in consultation with FDA, reviews and approves the application for any patent term

extension or restoration. In the future, we intend to apply for restoration of patent term for one of our currently owned or licensed patents to add patent life beyond its current expiration date, depending on the expected length of the clinical trials and other factors involved in the filing of the relevant NDA.

Marketing exclusivity provisions under the FDCA can also delay the submission or the approval of certain marketing applications for competing products. The FDCA provides a five-year period of non-patent marketing exclusivity within the United States to the first applicant to obtain approval of an NDA for a new chemical entity. A drug is a new chemical entity if FDA has not previously approved any other new drug containing the same active moiety, which is the molecule or ion responsible for the action of the drug substance. During the exclusivity period, FDA may not accept for review an abbreviated new drug application, or ANDA, or a 505(b)(2) NDA submitted by another company for another drug based on the same active moiety, regardless of whether the drug is intended for the same indication as the original innovator drug or for another indication. However, an application may be submitted after four years if it contains a certification of patent invalidity or non-infringement to one of the patents listed with FDA by the innovator NDA holder. The FDCA also provides three years of marketing exclusivity for an NDA, or supplement to an existing NDA, if new clinical investigations, other than bioavailability studies, that were conducted or sponsored by the applicant are deemed by FDA to be essential to the approval of the application, for example new indications, dosages or strengths of an existing drug. This three-year exclusivity covers only the modification for which the drug received approval on the basis of the new clinical investigations and does not prohibit FDA from approving ANDAs or 505(b)(2) applications for drugs containing the active agent for the original indication or condition of use. Five-year and three-year exclusivity will not delay the submission or approval of a full NDA. However, an applicant submitting a full NDA would be required to conduct or obtain a right of reference to all of the preclinical studies and adequate and well-controlled clinical trials necessary to demonstrate safety and effectiveness. Orphan drug exclusivity, as described below, may offer a seven-year period of marketing exclusivity, except in certain circumstances. Pediatric exclusivity is another type of regulatory market exclusivity in the United States. Pediatric exclusivity, if granted, adds six months to existing exclusivity periods and patent terms. This six-month exclusivity, which runs from the end of other exclusivity protection or patent term, may be granted based on the voluntary completion of a pediatric trial in accordance with an FDA-issued “Written Request” for such a trial.

Orphan Drug Designation

FDA may grant Orphan Drug Designation to drugs intended to treat a rare disease or condition that affects fewer than 200,000 individuals in the United States, or if it affects more than 200,000 individuals in the United States, there is no reasonable expectation that the cost of developing and marketing the drug for this type of disease or condition will be recovered from sales in the United States. In the European Union, the European Commission, after receiving the opinion of the EMA’s Committee for Orphan Medicinal Products, or COMP, grants Orphan Drug Designation to promote the development of products that are intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating conditions affecting not more than five in 10,000 persons in the European Union Community. Additionally, designation is granted for products intended for the diagnosis, prevention or treatment of a life-threatening, seriously debilitating or serious and chronic condition and when, without incentives, it is unlikely that sales of the drug in the European Union would be sufficient to justify the necessary investment in developing the drug or biological product.

In the United States, Orphan Drug Designation entitles a party to financial incentives such as opportunities for grant funding towards clinical trial costs, tax advantages and user-fee waivers. In addition, if a product receives the first FDA approval for the indication for which it has orphan designation, the product is entitled to orphan drug exclusivity, which means FDA may not approve any other application to market the same drug for the same indication for a period of seven years, except in limited circumstances, such as a showing of clinical superiority over the product with orphan exclusivity.

In the European Union, Orphan Drug Designation also entitles a party to financial incentives such as reduction of fees or fee waivers and ten years of market exclusivity is granted following drug or biological

product approval. This period may be reduced to six years if the Orphan Drug Designation criteria are no longer met, including where it is shown that the product is sufficiently profitable not to justify maintenance of market exclusivity.

Orphan drug designation must be requested before submitting an application for marketing approval. Orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process.

Regulation of Diagnostic Tests

Some of our drug candidates may require use of a diagnostic to identify appropriate patient populations for our products. These diagnostics, often referred to as companion diagnostics, are medical devices, often in vitro devices, which provide information that is essential for the safe and effective use of a corresponding drug. In the United States, the FDCA and its implementing regulations, and other federal and state statutes and regulations govern, among other things, medical device design and development, preclinical and clinical testing, premarket clearance or approval, registration and listing, manufacturing, labeling, storage, advertising and promotion, sales and distribution, export and import, and post-market surveillance. Unless an exemption applies, diagnostic tests require marketing clearance or approval from FDA prior to commercial distribution. The two primary types of FDA marketing authorization applicable to a medical device are premarket notification, also called 510(k) clearance, and premarket approval, or PMA approval. We expect that any companion diagnostic developed for our drug candidates will utilize the PMA pathway.

PMA applications must be supported by valid scientific evidence, which typically requires extensive data, including technical, preclinical, clinical and manufacturing data, to demonstrate to FDA's satisfaction the safety and effectiveness of the device. For diagnostic tests, a PMA application typically includes data regarding analytical and clinical validation studies. As part of its review of the PMA, FDA will conduct a pre-approval inspection of the manufacturing facility or facilities to ensure compliance with the Quality System Regulation, or QSR, which requires manufacturers to follow design, testing, control, documentation and other quality assurance procedures. FDA review of an initial PMA may require several years to complete. If FDA evaluations of both the PMA application and the manufacturing facilities are favorable, FDA will either issue an approval letter or an approvable letter, which usually contains a number of conditions that must be met in order to secure the final approval of the PMA. If FDA's evaluation of the PMA or manufacturing facilities is not favorable, FDA will deny approval of the PMA or issue a not approvable letter. A not approvable letter will outline the deficiencies in the application and, where practical, will identify what is necessary to make the PMA approvable. FDA may also determine that additional clinical trials are necessary, in which case the PMA approval may be delayed for several months or years while the trials are conducted and then the data submitted in an amendment to the PMA. Once granted, PMA approval may be withdrawn by FDA if compliance with post approval requirements, conditions of approval or other regulatory standards is not maintained or problems are identified following initial marketing.

On August 6, 2014, FDA issued a final guidance document addressing the development and approval process for "In Vitro Companion Diagnostic Devices." According to the guidance, for novel drugs such as our drug candidates, a companion diagnostic device and its corresponding drug should be approved or cleared contemporaneously by FDA for the use indicated in the therapeutic product labeling. The guidance also explains that a companion diagnostic device used to make treatment decisions in clinical trials of a drug generally will be considered an investigational device, unless it is employed for an intended use for which the device is already approved or cleared. If used to make critical treatment decisions, such as patient selection, the diagnostic device generally will be considered a significant risk device under FDA's Investigational Device Exemption, or IDE, regulations. Thus, the sponsor of the diagnostic device will be required to comply with the IDE regulations. According to the guidance, if a diagnostic device and a drug are to be studied together to support their respective approvals, both products can be studied in the same investigational study, if the study meets both the requirements of the IDE regulations and the IND regulations. The guidance provides that depending on the details of the study plan and subjects, a sponsor may seek to submit an IND alone, or both an IND and an IDE.

In the EEA, in vitro medical devices are required to conform with the essential requirements of the EU Directive on in vitro diagnostic medical devices (Directive No 98/79/EC, as amended). To demonstrate compliance with the essential requirements, the manufacturer must undergo a conformity assessment procedure. The conformity assessment varies according to the type of medical device and its classification. For low-risk devices, the conformity assessment can be carried out internally, but for higher risk devices it requires the intervention of an accredited EEA Notified Body. If successful, the conformity assessment concludes with the drawing up by the manufacturer of an EC Declaration of Conformity entitling the manufacturer to affix the CE mark to its products and to sell them throughout the EEA.

European Drug Development

In Europe, our future drugs may also be subject to extensive regulatory requirements. As in the United States, medicinal products can only be marketed if a marketing authorization from the competent regulatory agencies has been obtained.

Similar to the United States, the various phases of preclinical and clinical research in Europe are subject to significant regulatory controls. Although the EU Clinical Trials Directive 2001/20/EC has sought to harmonize the European Union clinical trials regulatory framework, setting out common rules for the control and authorization of clinical trials in the European Union, the European Union Member States have transposed and applied the provisions of the Directive differently. This has led to significant variations in the member state regimes. Under the current regime, before a clinical trial can be initiated it must be approved in each of the European Union countries where the trial is to be conducted by two distinct bodies: the National Competent Authority, or NCA, and one or more Ethics Committees, or ECs. Under the current regime all suspected unexpected serious adverse reactions to the investigated drug that occur during the clinical trial have to be reported to the NCA and ECs of the Member State where they occurred.

In 2014, a new Clinical Trials Regulation 536/2014, replacing the current Directive, was adopted. The new Regulation will become directly applicable in all EU Member States (without national implementation) once the EU Portal and Database are fully functional. It is expected that the Regulation will apply in 2019. The new Regulation seeks to simplify and streamline the approval of clinical trials in the European Union. For example, the sponsor shall submit a single application for approval of a clinical trial via the EU Portal. As part of the application process, the sponsor shall propose a reporting Member State, who will coordinate the validation and evaluation of the application. The reporting Member State shall consult and coordinate with the other concerned Member States. If an application is rejected, it can be amended and resubmitted through the EU Portal. If an approval is issued, the sponsor can start the clinical trial in all concerned Member States. However, a concerned Member State can in limited circumstances declare an “opt-out” from an approval. In such a case, the clinical trial cannot be conducted in that Member State. The Regulation also aims to streamline and simplify the rules on safety reporting, and introduces enhanced transparency requirements such as mandatory submission of a summary of the clinical trial results to the EU Database.

European Drug Review and Approval

In the European Economic Area, or EEA, which is comprised of the 28 Member States of the European Union plus Norway, Iceland and Liechtenstein, medicinal products can only be commercialized after obtaining a Marketing Authorization, or MA. There are two types of marketing authorizations.

The first is the Community MA, which is issued by the European Commission through the Centralized Procedure, based on the opinion of the Committee for Medicinal Products for Human Use, or CHMP, of the EMA and which is valid throughout the entire territory of the EEA. The Centralized Procedure is mandatory for certain types of drugs, such as biotechnology medicinal drugs, orphan medicinal drugs, and medicinal drugs containing a new active substance indicated for the treatment of AIDS, cancer, neurodegenerative disorders, diabetes, auto-immune and viral diseases. The Centralized Procedure is optional for drugs containing a new

active substance not yet authorized in the EEA, or for drugs that constitute a significant therapeutic, scientific or technical innovation or which are in the interest of public health in the European Union.

National MAs, which are issued by the competent authorities of the Member States of the EEA and only cover their respective territory, are available for drugs not falling within the mandatory scope of the Centralized Procedure. Where a drug has already been authorized for marketing in a Member State of the EEA, this National MA can be recognized in another Member States through the Mutual Recognition Procedure. If the drug has not received a National MA in any Member State at the time of application, it can be approved simultaneously in various Member States through the Decentralized Procedure. Under the Decentralized Procedure an identical dossier is submitted to the competent authorities of each of the Member States in which the MA is sought, one of which is selected by the applicant as the Reference Member State, or RMS. The competent authority of the RMS prepares a draft assessment report, a draft summary of the drug characteristics, or SPC, and a draft of the labeling and package leaflet, which are sent to the other Member States (referred to as the Member States Concerned) for their approval. If the Member States Concerned raise no objections, based on a potential serious risk to public health, to the assessment, SPC, labeling, or packaging proposed by the RMS, the drug is subsequently granted a national MA in all the Member States (i.e., in the RMS and the Member States Concerned).

Under the above described procedures, before granting the MA, the EMA or the competent authorities of the Member States of the EEA make an assessment of the risk-benefit balance of the drug on the basis of scientific criteria concerning its quality, safety and efficacy.

European Chemical Entity Exclusivity

In Europe, new chemical entities, sometimes referred to as new active substances, 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 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 11 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.

Rest of the World Regulation

For other countries outside of the Europe and the United States, such as countries in Eastern Europe, Latin America or Asia, the requirements governing the conduct of clinical trials, drug licensing, pricing and reimbursement vary from country to country. In all cases the clinical trials must be conducted in accordance with GCP requirements and the applicable regulatory requirements and the ethical principles that have their origin in the Declaration of Helsinki.

If we fail to comply with applicable foreign regulatory requirements, we may be subject to, among other things, fines, suspension or withdrawal of regulatory approvals, product recalls, seizure of products, operating restrictions and criminal prosecution.

Coverage and Reimbursement

Sales of our drugs will depend, in part, on the extent to which our drugs will be covered by third-party payors, such as government health programs, commercial insurers and managed healthcare organizations, as well as the level of reimbursement such third-party payors provide for our products. Patients and providers are unlikely to use our products unless coverage is provided and reimbursement is adequate to cover a significant portion of the cost of our products in which our products are used. These third-party payors are increasingly reducing reimbursements for medical drugs and services.

In the United States, no uniform policy of coverage and reimbursement for drugs or biological products exists, and one payor's determination to provide coverage and adequate reimbursement for a product does not assure that other payors will make a similar determination. Accordingly, decisions regarding the extent of coverage and amount of reimbursement to be provided for any of our products candidates, if approved, will be made on a payor-by-payor basis. As a result, the coverage determination process may be a time-consuming and costly process that will require us to provide scientific and clinical support for the use of our products to each payor separately, with no assurance that coverage and adequate reimbursement will be obtained.

Additionally, the containment of healthcare costs has become a priority of federal and state governments, and the prices of drugs have been a focus in this effort. The U.S. government, state legislatures and foreign governments have shown significant interest in implementing cost-containment programs, including price controls, restrictions on reimbursement and requirements for substitution of generic drugs. Adoption of price controls and cost-containment measures, and adoption of more restrictive policies in jurisdictions with existing controls and measures, could further limit our net revenue and results. Decreases in third-party reimbursement for our drug candidates, if approved, or a decision by a third-party payor to not cover our drug candidates could reduce physician usage of such drugs and have a material adverse effect on our sales, results of operations and financial condition.

The Medicaid Drug Rebate Program requires pharmaceutical manufacturers to enter into and have in effect a national rebate agreement with the Secretary of the Department of Health and Human Services as a condition for states to receive federal matching funds for the manufacturer's outpatient drugs furnished to Medicaid patients. The Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010, or collectively the ACA, made several changes to the Medicaid Drug Rebate Program, including increasing pharmaceutical manufacturers' rebate liability by raising the minimum basic Medicaid rebate on most branded prescription drugs from 15.1% of average manufacturer price, or AMP, to 23.1% of AMP and adding a new rebate calculation for "line extensions" (i.e., new formulations, such as extended release formulations) of solid oral dosage forms of branded products, creating a new methodology by which rebates owed are calculated for drugs that are inhaled, infused, instilled, implanted or injected, as well as potentially impacting their rebate liability by modifying the statutory definition of AMP. The ACA also expanded the universe of Medicaid utilization subject to drug rebates by requiring pharmaceutical manufacturers to pay rebates on Medicaid managed care utilization and by enlarging the population potentially eligible for Medicaid drug benefits. Pricing and rebate programs must also comply with the Medicaid rebate requirements of the U.S. Omnibus Budget Reconciliation Act of 1990.

The Medicare Prescription Drug, Improvement, and Modernization Act of 2003, or the MMA, established the Medicare Part D program to provide a voluntary prescription drug benefit to Medicare beneficiaries. Under Part D, Medicare beneficiaries may enroll in prescription drug plans offered by private entities that provide coverage of outpatient prescription drugs. Unlike Medicare Parts A and B, Part D coverage is not standardized. Part D prescription drug plan sponsors are not required to pay for all covered Part D drugs, and each drug plan can develop its own drug formulary that identifies which drugs it will cover and at what tier or level. However, while all Medicare drug plans must give at least a standard level of coverage set by Medicare, Part D prescription drug plan sponsors are not required to pay for all covered Part D drugs, and each drug plan can develop its own drug formulary that identifies which drugs it will cover and at what tier or level. These Part D prescription drug formularies must include drugs within each therapeutic category and class of covered Part D drugs, though not necessarily all the drugs in each category or class. Any formulary used by a Part D prescription drug plan must be developed and reviewed by a pharmacy and therapeutic committee. Government payment for some of the costs of prescription drugs may increase demand for drugs for which we obtain marketing approval. However, any negotiated prices for our drugs covered by a Part D prescription drug plan will likely be lower than the prices we might otherwise obtain. Moreover, while the MMA applies only to drug benefits for Medicare beneficiaries, private payors often follow Medicare coverage policy and payment limitations in setting their own payment rates. Any reduction in payment that results from the MMA may result in a similar reduction in payments from non-governmental payors.

For a drug product to receive federal reimbursement under the Medicaid or Medicare Part B programs or to be sold directly to U.S. government agencies, the manufacturer must extend discounts to entities eligible to participate in the 340B drug pricing program. The required 340B discount on a given product is calculated based on the AMP and Medicaid rebate amounts reported by the manufacturer. As of 2010, the ACA expanded the types of entities eligible to receive discounted 340B pricing, although, under the current state of the law, with the exception of children's hospitals, these newly eligible entities will not be eligible to receive discounted 340B pricing on orphan drugs. In addition, as 340B drug pricing is determined based on AMP and Medicaid rebate data, the revisions to the Medicaid rebate formula and AMP definition described above could cause the required 340B discount to increase.

The American Recovery and Reinvestment Act of 2009 provides funding for the federal government to compare the effectiveness of different treatments for the same illness. The plan for the research was published in 2012 by the Department of Health and Human Services, the Agency for Healthcare Research and Quality and the National Institutes for Health, and periodic reports on the status of the research and related expenditures are made to Congress. Although the results of the comparative effectiveness studies are not intended to mandate coverage policies for public or private payors, it is not clear what effect, if any, the research will have on the sales of our drug candidates, if any such drug or the condition that they are intended to treat are the subject of a trial. It is also possible that comparative effectiveness research demonstrating benefits in a competitor's drug could adversely affect the sales of our drug candidate. If third-party payors do not consider our drugs to be cost-effective compared to other available therapies, they may not cover our drugs after approval as a benefit under their plans or, if they do, the level of payment may not be sufficient to allow us to sell our drugs on a profitable basis.

In recent years, additional laws have resulted in direct or indirect reimbursement reductions for certain Medicare providers, including:

- The Budget Control Act of 2011, among other things, created measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least $1.2 trillion for the years 2013 through 2021, was unable to reach required goals, thereby triggering the legislation's automatic reduction to several government programs. These changes included aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, which went into effect in April 2013 and will remain in effect through 2025 unless additional Congressional action is taken.
- The American Taxpayer Relief Act of 2012, 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.
- The Middle Class Tax Relief and Job Creation Act of 2012 required that the Centers for Medicare & Medicaid Services reduce the Medicare clinical laboratory fee schedule by 2% in 2013, which served as a base for 2014 and subsequent years. In addition, effective January 1, 2014, CMS also began bundling the Medicare payments for certain laboratory tests ordered while a patient received services in a hospital outpatient setting.

These laws, and future state and federal healthcare reform measures may be adopted in the future, any of which may result in additional reductions in Medicare and other healthcare funding and otherwise affect the prices we may obtain for any product candidates for which we may obtain regulatory approval or the frequency with which any such product candidate is prescribed or used.

In addition, in some foreign countries, the proposed pricing for a drug must be approved before it may be lawfully marketed. The requirements governing drug pricing vary widely from country to country. For example, the European Union provides options for its member states to restrict the range of medicinal drugs for which their national health insurance systems provide reimbursement and to control the prices of medicinal drugs for human use. A member state may approve a specific price for the medicinal drug or it may instead adopt a system of

direct or indirect controls on the profitability of the company placing the medicinal drug on the market. There can be no assurance that any country that has price controls or reimbursement limitations for pharmaceutical drugs will allow favorable reimbursement and pricing arrangements for any of our drugs. Historically, drugs launched in the European Union do not follow price structures of the United States and generally tend to be significantly lower.

U.S. Healthcare Reform

The ACA has had a significant impact on the healthcare industry. The ACA expanded coverage for the uninsured while at the same time containing overall healthcare costs. With regard to pharmaceutical products, the ACA, among other things, addressed a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected, increased the minimum Medicaid rebates owed by manufacturers under the Medicaid Drug Rebate Program and extended the rebate program to individuals enrolled in Medicaid managed care organizations, established annual fees and taxes on manufacturers of certain branded prescription drugs, and a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 50% point-of-sale discounts off negotiated prices of applicable brand drugs to eligible beneficiaries during their coverage gap period, as a condition for the manufacturer's outpatient drugs to be covered under Medicare Part D.

Some of the provisions of the ACA have yet to be fully implemented, while certain provisions have been subject to reform through legislation and Executive Orders of the President, and to judicial challenges. The U.S. Supreme Court has upheld certain key aspects of the legislation, including a tax-based shared responsibility payment imposed on certain individuals who fail to maintain qualifying health coverage for all or part of a year, which is commonly the requirement that all individuals maintain health insurance coverage or pay a penalty, referred to as the "individual mandate. However, the new presidential administration has indicated that enacting changes to the ACA is a legislative priority, and has discussed repealing and replacing or amending the ACA. In January 2017, Congress voted to adopt a budget resolution for fiscal year 2017, that while not a law, is widely viewed as the first step toward the passage of legislation that would repeal certain aspects of the ACA. While Congress has not passed repeal legislation to date, the 2017 Tax Reform Act includes a provision repealing the individual mandate, effective January 1, 2019. In addition, on January 20, 2017, the President signed an Executive Order directing federal agencies with authorities and responsibilities under the ACA to waive, defer, grant exemptions from, or delay the implementation of any provision of the ACA that would impose a fiscal burden on states or a cost, fee, tax, penalty or regulatory burden on individuals, healthcare providers, health insurers, or manufacturers of pharmaceuticals or medical devices. On October 13, 2017, the President signed an Executive Order terminating the cost-sharing subsidies that reimburse insurers under the ACA. Several state Attorneys General filed suit to stop the administration from terminating the subsidies, but their request for a restraining order was denied by a federal judge in California on October 25, 2017. In addition, CMS has recently proposed regulations that would give states greater flexibility in setting benchmarks for insurers in the individual and small group marketplaces, which may have the effect of relaxing the essential health benefits required under the ACA for plans sold through such marketplaces. Further, each chamber of Congress has put forth multiple bills this year designed to repeal or repeal and replace portions of the ACA. Although none of these measures has been enacted by Congress to date, Congress may consider other legislation to repeal and replace elements of the ACA.

Thus, the full impact of the ACA, any law repealing or replacing elements of it, or the political uncertainty surrounding its repeal or replacement on our business remains unclear. We expect that additional federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that federal and state governments will pay for healthcare drugs and services, and in turn could significantly reduce the projected value of certain development projects and reduce our profitability and may increase our regulatory burdens and operating costs.

Other Healthcare Laws

For our product and any product candidates that obtain regulatory approval and are marketed in the United States, our arrangements with third-party payors, healthcare providers, and customers may expose us 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 any products for which we obtain marketing approval. In addition, we may be subject to health information privacy and security regulation by U.S. federal and state governments and foreign jurisdictions in which we conduct our business. In the United States, these laws include, without limitation, state and federal anti-kickback, false claims, physician transparency, and patient data privacy and security laws and regulations, including but not limited to those described below:

- The federal Anti-Kickback Statute makes it illegal for any person, including a prescription drug manufacturer (or a party on its behalf) to, knowingly and willfully offer, solicit, receive, or pay remuneration, directly or indirectly, in cash or in kind, that is intended to induce or reward the referral of an individual for or the purchase or recommendation of an item or for which payment may be made under federal healthcare programs such as the Medicare and Medicaid programs. Violations of this law are punishable by up to five years in prison, criminal fines, administrative civil money penalties and exclusion from participation in federal healthcare programs. In addition, a person or entity does not need to have actual knowledge of the statute or specific intent to violate it.
- The federal False Claims Act imposes civil penalties, including through civil whistleblower or qui tam actions, against individuals or entities (including manufacturers) 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. Penalties for a False Claims Act violation include three times the actual damages sustained by the government, plus mandatory civil penalties of between $10,781and $21,563 for each separate false claim, the potential for exclusion from participation in federal healthcare programs and the potential implication of various federal criminal statutes. The government may deem manufacturers to have “caused” the submission of false or fraudulent claims by, for example, providing inaccurate billing or coding information to customers or promoting a product off-label. Claims which include items or services resulting from a violation of the federal Anti-Kickback Statute are false or fraudulent claims for purposes of the False Claims Act. Our future marketing and activities relating to the reporting of wholesaler or estimated retail prices for our products, the reporting of prices used to calculate Medicaid rebate information and other information affecting federal, state and third-party reimbursement for our products, and the sale and marketing of our product and any future product candidates, are subject to scrutiny under this law.
- Health Insurance Portability and Accountability Act of 1996, or HIPAA, which imposes criminal and civil liability for knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program, including private payors, knowingly and willfully embezzling or stealing from a healthcare benefit program, willfully obstructing a criminal investigation of a healthcare offense, and knowingly or willfully falsifying, concealing or covering up a material fact or making any materially false, fictitious or fraudulent statement in connection with the delivery of or payment for healthcare benefits, items or services.
- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009 (“HITECH”), and their respective implementing regulations, which imposes, among other things, specified requirements on covered entities and their business associates, relating to the privacy and security of individually identifiable health information, including mandatory contractual terms and required implementation of technical safeguards of such information. HITECH also created new tiers of civil monetary penalties, amended HIPAA to make civil and criminal penalties applicable to business associates, and gave state attorneys general new authority to file civil actions for damage or injunctions in federal courts to enforce the federal HIPAA laws and seek attorneys’ fees and costs associated with pursuing federal civil actions.

- The Physician Payments Sunshine Act, enacted as part of the ACA, as amended by the Health Care and Education Reconciliation Act of 2010, which imposed new annual reporting requirements for certain manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program for certain payments and other "transfers of value" provided to physicians and teaching hospitals, and ownership and investment interests held by physicians and their immediate family members.
- Analogous state and foreign fraud and abuse laws and regulations, such as state anti-kickback and false claims laws, which may be broader in scope and apply regardless of payor. Such laws are enforced by various state agencies and through private actions. Some state laws require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant federal government compliance guidance, require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers, and restrict marketing practices or require disclosure of marketing expenditures. State and foreign laws also govern the privacy and security of health information in certain circumstances. Such data privacy and security laws may differ from one another in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

The scope and enforcement of each of these laws is uncertain and subject to rapid change in the current environment of healthcare reform, especially in light of the lack of applicable precedent and regulations. Federal and state enforcement bodies have recently increased their scrutiny of interactions between healthcare companies and healthcare providers, which has led to a number of investigations, prosecutions, convictions and settlements in the healthcare industry. 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 related governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, damages, fines, imprisonment, disgorgement ,exclusion of drugs from government funded healthcare programs, such as Medicare and Medicaid, reputational harm, additional oversight and reporting obligations if we become subject to a corporate integrity agreement or similar settlement to resolve allegations of non-compliance with these laws 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 similar actions, penalties and sanctions. Ensuring business arrangements comply with applicable healthcare laws, as well as responding to possible investigations by government authorities, can be time- and resource-consuming and can divert a company's attention from the business.

Employees

We have operated by leveraging skilled experts, consultants, contract research organizations, and contractors to manage our clinical operations, under the leadership and direction of our management. We will expand our infrastructure to manage our clinical, finance and commercial operations with additional full-time employees.

As of February 28, 2018, we had 63 full-time employees and 2 part-time employees, 24 of whom hold Ph.D. or M.D. degrees. Of these employees, 52 were engaged in research and development activities and 13 were engaged in general and administrative activities. None of our employees are represented by labor unions or covered by collective bargaining agreements. We consider our relationship with our employees to be good.

Facilities

We currently lease two facilities. Our headquarters are located in 6,883 rentable square feet of office space in Waltham, Massachusetts that is used primarily for our clinical research, regulatory, business development and

administrative functions. We also lease 25,524 square feet of laboratory, office and storage space in Lawrence, Kansas that is used primarily for discovery research, preclinical research, and non-clinical functions. The lease for our headquarters in Waltham, Massachusetts expires in August 2019 and the leases for our space in Lawrence, Kansas each expire in December 2020. We believe that our existing facilities are adequate for our current needs. We expect to lease additional office space for our Massachusetts operations as we increase our staffing levels as a result of anticipated growth in our clinical and regulatory activities as well as additional financial and administrative resources required to support operations as a public company. We believe that any additional space we may require will be available on commercially reasonable terms.

Legal Proceedings

We are not currently a party to any material legal proceedings.

Corporate Information, Initial Public Offering and Organizational Transactions

Deciphera Pharmaceuticals, LLC was formed and commenced operations in 2003. Deciphera Pharmaceuticals, Inc. was incorporated under the laws of Delaware on August 1, 2017, for the sole purpose of completing an initial public offering and related transactions in order to carry on the business of Deciphera Pharmaceuticals, LLC. We are the sole managing member of Deciphera Pharmaceuticals, LLC and conduct all our business through, operate and control all of the businesses and affairs of Deciphera Pharmaceuticals, LLC, our wholly owned subsidiary, directly or through blocker entities which are also wholly owned by us.

On October 2, 2017, we completed the initial public offering of our common stock, or IPO, pursuant to which we issued and sold 7,500,000 shares of our common stock at a price to the public of $17.00 per share. In addition, on October 4, 2017, we issued and sold an additional 666,496 shares of common stock at the initial public offering price of $17.00 per share as a result of the partial exercise by the underwriters of their option to purchase additional shares of common stock, resulting in net proceeds to us of $124.6 million after deducting underwriting discounts, commissions and offering expenses payable by us. On October 2, 2017, immediately prior to the completion of the IPO, the Company engaged in a series of transactions whereby Deciphera Pharmaceuticals, LLC became a wholly owned subsidiary of Deciphera Pharmaceuticals, Inc., a Delaware corporation. As part of the transactions, shareholders of Deciphera Pharmaceuticals, LLC exchanged their shares of Deciphera Pharmaceuticals, LLC for shares of Deciphera Pharmaceuticals, Inc. on a one-for-5.65 basis.

See Part II—Item 7—Management’s Discussion and Analysis of Financial Condition and Results of Operations and Note 1 to the consolidated financial statements included in Part II—Item 8 for more information about the above-mentioned transactions.

We are an “emerging growth company” as defined in the Jumpstart Our Business Startups Act of 2012. We will remain an emerging growth company until the earlier of: (i) the last day of the fiscal year (a) following the fifth anniversary of the completion of the IPO, (b) in which we have total annual gross revenue of at least $1.07 billion, or (c) in which we are deemed to be a large accelerated filer, which means the market value of our common stock that is held by non-affiliates exceeds $700.0 million as of the prior June 30th, and (ii) the date on which we have issued more than $1.0 billion in non-convertible debt during the prior three-year period.

Our principal executive offices are located at 500 Totten Pond Road, Waltham, MA 02451, and our telephone number is (781) 209-6400. Our corporate website address is *www.deciphera.com*. Our website and the information contained on, or that can be accessed through, the website will not be deemed to be incorporated by reference in, and are not considered part of, this Annual Report on Form 10-K. You should not rely on any such information in making your decision whether to purchase our common stock.

Financial Information and Segments

The financial information required under this Item 1 is incorporated herein by reference to the section of this Annual Report titled “Part II—Item 8—Financial Statements and Supplementary Data.” We operate in one

business segment. See Note 2 to our consolidated audited financial statements included in this Annual Report. For financial information regarding our business, see “Part II—Item 7—Management’s Discussion and Analysis of Financial Condition and Results of Operations” of this Annual Report and our consolidated audited financial statements and related notes included elsewhere in this Annual Report.

Available Information

Our Internet address is www.deciphera.com. Our Annual Reports on Form 10-K, Quarterly Reports on Form 10-Q, Current Reports on Form 8-K, including exhibits, proxy and information statements and amendments to those reports filed or furnished pursuant to Sections 13(a), 14, and 15(d) of the Securities Exchange Act of 1934, as amended, or the Exchange Act, are available through the “Investors” portion of our website free of charge as soon as reasonably practicable after we electronically file such material with, or furnish it to, the SEC. Information on our website is not part of this Annual Report on Form 10-K or any of our other securities filings unless specifically incorporated herein by reference. The public may read and copy these materials at the SEC’s Public Reference Room at 100 F Street, NE, Washington, DC 20549. The public may obtain information on the operation of the Public Reference Room by calling the Securities and Exchange Commission, or the SEC, at 1-800-SEC-0330. In addition, our filings with the SEC may be accessed through the SEC’s Interactive Data Electronic Applications system at http://www.sec.gov. All statements made in any of our securities filings, including all forward-looking statements or information, are made as of the date of the document in which the statement is included, and we do not assume or undertake any obligation to update any of those statements or documents unless we are required to do so by law.

ITEM 1A. RISK FACTORS

Our business is subject to numerous risks. You should carefully consider the risks and uncertainties described below together with all of the other information contained in this Annual Report on Form 10-K including our consolidated financial statements and the related notes. If any of the following risks actually occur, our business, prospects, operating results and financial condition could suffer materially. In such event, the trading price of our common stock could decline and you might lose all or part of your investment.

Risks Related to Our Financial Position and Need for Additional Capital

We have incurred significant operating losses since our inception and have not generated any revenue from product sales. We expect to incur continued losses for the foreseeable future and may never achieve or maintain profitability.

Investment in pharmaceutical product development is a highly speculative undertaking and involves a substantial degree of risk. We are a clinical-stage biopharmaceutical company that was formed and commenced operations in 2003. We have no approved products for commercial sale and have not generated any revenue from product sales to date. We continue to incur significant research and development and other expenses related to our ongoing operations. As a result, we have never been profitable and have incurred losses in each year since inception. For the years ended December 31, 2017, 2016 and 2015, we reported a net loss of $50.3 million, $25.9 million and $19.8 million, respectively. As of December 31, 2017, we had an accumulated deficit of $195.9 million.

Since our inception, we have focused substantially all of our efforts and financial resources on developing our proprietary compound library, and the preclinical and clinical development of our drug candidates, DCC-2618, DCC-3014, rebastinib and our former drug candidate, which was discontinued and which we no longer plan to develop. To date, we have funded our operations primarily with proceeds from the sales of our common stock in our initial public offering, sales of preferred shares, proceeds from the issuance of convertible notes, payments received under a concluded collaboration agreement, borrowings under a construction loan and research and development grants from the Kansas Bioscience Authority, or KBA. From our inception through December 31, 2017, we received an aggregate of $388 million in net proceeds from such transactions. As of December 31, 2017, our cash and cash equivalents were $196.8 million.

We expect to incur increasing levels of operating losses over the next several years and for the foreseeable future, particularly as we advance our drug candidates. Our prior losses, combined with expected future losses, have had, and will continue to have, an adverse effect on our stockholders' equity and working capital. We expect our research and development expenses to significantly increase in connection with our ongoing and additional clinical trials for DCC-2618, DCC-3014 and rebastinib, and development of any other future drug candidates we may choose to pursue. In addition, if we obtain marketing approval for DCC-2618, or any of our other drug candidates, we will incur significant sales, marketing and outsourced manufacturing expenses. We will also incur additional costs associated with operating as a public company. As a result, we expect to continue to incur significant and increasing operating losses for the foreseeable future. Because of the numerous risks and uncertainties associated with developing pharmaceutical products, we are unable to predict the extent of any future losses or when we will become profitable, if at all. Even if we do become profitable, we may not be able to sustain or increase our profitability on a quarterly or annual basis.

Our ability to become profitable depends upon our ability to generate revenue. To date, we have not generated any revenue from our drug candidates, and we do not know when, or if, we will generate any revenue. We do not expect to generate significant revenue unless and until we obtain marketing approval for, and begin to sell, DCC-2618, or our other drug candidates. Our ability to generate revenue depends on a number of factors, including, but not limited to, our ability to:

- successfully complete our first Phase 3 clinical trial of DCC-2618 for the treatment of fourth-line plus gastrointestinal stromal tumors, or GIST;

- initiate and successfully complete our second Phase 3 clinical trial of DCC-2618 for the treatment of second-line GIST;
- initiate and successfully complete other later-stage clinical trials that meet their clinical endpoints;
- initiate and successfully complete all safety studies required to obtain U.S. and foreign marketing approval for DCC-2618 as a treatment for GIST or other indications;
- subject to obtaining favorable results from our Phase 3 trials, applying for and obtaining marketing approval for DCC-2618;
- successfully manufacture or contract with others to manufacture DCC-2618 and our other our drug candidates;
- commercialize DCC-2618, if approved, by building a sales force or entering into collaborations with third parties;
- obtain, maintain, protect and defend our intellectual property portfolio; and
- achieve market acceptance of DCC-2618 in the medical community and with third-party payors.

To become and remain profitable, we must succeed in developing, and eventually commercializing, products that generate significant revenue. This will require us to be successful in a range of challenging activities, including completing preclinical testing and clinical trials for our drug candidates, discovering additional drug candidates, establishing arrangements with third parties for the manufacture of clinical supplies of our drug candidates, obtaining marketing approval for our drug candidates and manufacturing, marketing and selling any products for which we may obtain marketing approval. We are only in the preliminary stages of most of these activities. We may never succeed in these activities and, even if we do, may never generate revenues that are significant enough to achieve profitability.

Because of the numerous risks and uncertainties associated with pharmaceutical product development, we are unable to accurately predict the timing or amount of increased expenses we will incur or when, or if, we will be able to achieve profitability. If we are required by the U.S. Food and Drug Administration, or FDA, the European Medicines Agency, or EMA, or other regulatory authorities to perform studies in addition to those currently expected, or if there are any delays in establishing appropriate manufacturing arrangements for, or in completing our clinical trials for, or the development of any of our drug candidates, our expenses could increase materially.

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 cause significant harm to our financial position, adversely impact our stock price and impair our ability to raise capital, expand our business, maintain our research and development efforts, diversify our product offerings or even continue our operations. A decline in the value of our company could also cause you to lose all or part of your investment.

We will require substantial additional funding. If we are unable to raise capital when needed, or on attractive terms, we could be forced to delay, reduce or eliminate our research or drug development programs or any future commercialization efforts.

We expect our expenses to increase substantially in connection with our ongoing activities, particularly as we advance our drug candidates, DCC-2618, DCC-3014 and rebastinib, and seek to identify lead drug candidates in our discovery programs. We expect increased expenses as we continue our research and development and initiate additional clinical trials, and establish arrangements with third parties for the manufacture of clinical and commercial supplies of and seek marketing approval for, our lead program and our other drug candidates. In addition, if we obtain marketing approval for any of our drug candidates, we expect to incur significant commercialization expenses related to product manufacturing, marketing, sales and distribution. Furthermore, we expect to incur additional costs associated with operating as a public company.

Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. If we are unable to raise capital when needed, or on attractive terms, we could be forced to delay, reduce or eliminate our research or drug development programs or any future commercialization efforts.

We believe that our existing cash and cash equivalents, will enable us to fund our operating expenses, capital expenditure requirements and debt service payments into the second half of 2019. We have based this estimate on assumptions that may prove to be wrong, and we could use our capital resources sooner than we currently expect.

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

- the scope, progress, costs and results of our clinical trials of DCC-2618;
- the scope, progress, costs and results of drug discovery, preclinical development and clinical trials for our other drug candidates;
- the number and development requirements of other drug candidates that we pursue, including ones we may acquire from third parties;
- the costs and timing of arrangements with third parties for the manufacture of clinical supplies of DCC-2618 and our other drug candidates;
- the costs, timing and outcome of regulatory review of DCC-2618 and our other drug candidates;
- the costs and timing of future commercialization activities, including product manufacturing, marketing, sales and distribution, for DCC-2618 and any of our other drug candidates for which we obtain marketing approval;
- the revenue, if any, received from commercial sales of DCC-2618 and our other drug candidates for which we obtain marketing approval;
- the costs and timing of preparing, filing and prosecuting any patent applications, maintaining and enforcing our intellectual property rights and defending any intellectual property-related claims;
- our ability to establish collaboration arrangements with other biotechnology or pharmaceutical companies on favorable terms, if at all, for the development or commercialization of our drug candidates; and
- the extent to which we acquire or in-license other drug candidates, technologies and associated intellectual property rights.

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

Raising additional capital may cause dilution to our stockholders, restrict our operations or require us to relinquish rights to our technologies or drug candidates.

Until such time, if ever, as we can generate substantial product revenue, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances and marketing, distribution or licensing arrangements. We do not currently have any committed external source of funds. In addition, we may seek additional capital due to favorable market conditions or strategic considerations, even if we believe we have sufficient funds for our current or future operating plans. To the extent that we raise

additional capital through the sale of equity or convertible debt securities, your ownership interest will be diluted, and the terms of our securities may include liquidation or other preferences that adversely affect your rights as a common stockholder. Debt financing and preferred equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making acquisitions or capital expenditures or declaring dividends.

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

We have a limited operating history, have not successfully completed late-stage clinical trials for any drug candidate, have not generated revenue from product sales or profits and do not expect to generate revenue or profits for the foreseeable future. We may never obtain approval for any of our drug candidates or achieve or sustain profitability.

We commenced operations in 2003. Our operations to date have been limited to organizing and staffing our company, business planning, raising capital, conducting research and development, filing patents, identifying potential drug candidates, including five clinical candidates, undertaking preclinical studies, initiating and conducting clinical trials and establishing arrangements with third parties for the manufacture of initial quantities of our drug candidates. We have not yet demonstrated our ability to successfully complete any Phase 2 or Phase 3 clinical trials, obtain marketing approvals, manufacture a commercial scale product, or arrange for a third party to do so on our behalf, or conduct sales, marketing and distribution activities necessary for successful product commercialization, and we have not generated revenue from product sales or profit from our operations. Consequently, we may never achieve or sustain profitability and any predictions you make about our future success or viability may not be as accurate as they could be if we had an operating history with these activities.

In addition, as a growing business entering into new stages of pharmaceutical development, we may encounter unforeseen expenses, difficulties, complications, delays and other known and unknown factors. We will need to transition at some point from a company with a research and development focus to a company capable of supporting commercial activities. There can be no assurance that we will be successful in such a transition.

Risks Related to the Discovery and Development of Our Drug Candidates

We are early in our development efforts. All of our drug candidates target inhibition of the activation switch in kinases. If we are unable to commercialize our drug candidates or experience significant delays in doing so, our business will be materially harmed.

We currently have no products that are approved for sale and have discontinued one of our programs, which we no longer plan to develop. We are early in our development efforts. Two of our drug candidates are only in Phase 1 clinical trials. We recently initiated our first Phase 3 clinical trial of one of our drug candidates in January 2018. All of our drug candidates target inhibition of the activation switch in kinases. There are no currently approved kinase switch control inhibitors and there can be no assurance that kinase switch control inhibitors will ever receive regulatory approval. Our discontinued drug candidate was also based on inhibition of the activation switch in kinases. Its development was discontinued due to strategic and competitive reasons. There can be no assurance that our current drug candidates will achieve success in their clinical trials or obtain regulatory approval.

Our ability to generate product revenues, which we do not expect will occur for many years, if ever, will depend heavily on the successful development and eventual commercialization of one or more of our drug

candidates. The success of our drug candidates, including DCC-2618, will depend on several factors, including the following:

- successful completion of preclinical studies and clinical trials;
- receipt and related terms of marketing approvals from applicable regulatory authorities;
- raising additional funds necessary to complete clinical development of and commercialize our drug candidates;
- obtaining and maintaining patent, trade secret and other intellectual property protection and regulatory exclusivity for our drug candidates;
- making arrangements with third-party manufacturers, or establishing manufacturing capabilities, for both clinical and commercial supplies of our drug candidates;
- developing and implementing marketing and reimbursement strategies;
- establishing sales, marketing and distribution capabilities and launching commercial sales of our products, if and when approved, whether alone or in collaboration with others;
- acceptance of our products, if and when approved, by patients, the medical community and third-party payors;
- effectively competing with other therapies;
- the ability to obtain clearance or approval of companion diagnostic tests, if required, on a timely basis, or at all;
- obtaining and maintaining third-party coverage and adequate reimbursement;
- protecting and enforcing our rights in our intellectual property portfolio; and
- maintaining a continued acceptable safety profile of the products following approval.

If we do not achieve one or more of these factors in a timely manner or at all, we could experience significant delays or an inability to successfully commercialize our drug candidates, which would materially harm our business. For example, our business could be harmed if results of the expansion stage of our Phase 1 clinical trial of DCC-2618 or our Phase 3 clinical trials of DCC-2618 vary meaningfully from our expectations.

Clinical drug development involves a lengthy and expensive process. We may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of DCC-2618 and our other drug candidates.

We currently have three drug candidates in clinical development and their risk of failure is high. We are unable to predict when or if any of our drug candidates will prove effective or safe in humans or will obtain marketing approval. Before obtaining marketing approval from regulatory authorities for the sale of any drug candidate, we must complete preclinical development and then conduct extensive clinical trials to demonstrate the safety and efficacy of our drug candidates in humans. Clinical testing is expensive, difficult to design and implement and can take many years to complete and is uncertain as to the outcome. A failure of one or more clinical trials can occur at any stage of testing. The outcome of preclinical testing 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. In particular, the small number of patients in our early clinical trials may make the results of these trials less predictive of the outcome of later clinical trials. In addition, although we observed encouraging disease control rates in the dose escalation stage of our Phase 1 trial of DCC-2618, the primary objectives were to determine the safety, tolerability and maximum tolerated dose of DCC-2618 and to determine a recommended Phase 2 dose and not to demonstrate efficacy. The assessments of efficacy from the dose escalation portion of our Phase 1 clinical trial of DCC-2618 were not designed to demonstrate statistical significance and may not be

predictive of the results of further clinical trials of DCC-2618 or any of our other drug candidates. We did not observe a maximum tolerated dose in the dose escalation stage of our Phase 1 trial of DCC-2618. FDA has stated that our plan to initiate our Phase 3 trial prior to the completion of our Phase 1 trial, and with limited dose-response information at the various dose levels, may place our development program at risk if we have not identified the optimal dosing regimen.

We may experience numerous unforeseen events during, or as a result of, clinical trials that could delay or prevent our ability to obtain marketing approval or commercialize our drug candidates, including:

- regulators may not authorize us to commence or continue a clinical trial or may impose a clinical hold or may limit the conduct of a clinical trial through the imposition of a partial clinical hold;
- institutional review boards, or IRBs, may not authorize us or our investigators to commence or continue a clinical trial at a prospective trial site or an IRB may not approve a protocol amendment to an ongoing clinical trial;
- we may experience delays in reaching, or fail to reach, agreement on acceptable clinical trial contracts or clinical trial protocols with prospective trial sites;
- clinical trials for our drug candidates may produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional clinical trials, delay planned trials or abandon product development programs;
- the number of patients required for clinical trials for our drug candidates may be larger than we anticipate, enrollment in these clinical trials may be slower than we anticipate, participants may drop out of these clinical trials at a higher rate than we anticipate or the duration of these clinical trials may be longer than we anticipate;
- our third-party contractors, including investigators, may fail to meet their contractual obligations to us in a timely manner, or at all, or may fail to comply with regulatory requirements;
- we may have to suspend or terminate clinical trials for our drug candidates for various reasons, including a finding that the participants are being exposed to unacceptable health risks;
- our drug candidates may have undesirable side effects or other unexpected characteristics, causing us or our investigators, regulators or IRBs to suspend or terminate the trials;
- the cost of clinical trials for our drug candidates may be greater than we anticipate; and
- the supply or quality of our drug candidates or other materials necessary to conduct clinical trials for our drug candidates may be insufficient or inadequate and result in delays or suspension of our clinical trials.

While we designed DCC-2618 to inhibit the full spectrum of the known mutant or amplified KIT and PDGFRα kinases that drive cancers such as GIST, we may find that patients treated with DCC-2618 have or develop mutations that confer resistance to treatment. We are aware of a secondary mutation in PDGFRα, in a patient not treated with DCC-2618, where the potency of inhibition determined in in vitro assays by DCC-2618 suggests that this mutation may confer resistance to DCC-2618 in patients. We may identify other additional mutations in PDGFRα or mutations in KIT that are resistant to DCC-2618. If patients have or develop resistance to treatment with our drug candidates, we may be unable to successfully complete our clinical trials, obtain regulatory approval of, and commercialize our drug candidates.

Our product development costs will increase if we experience delays in preclinical studies or clinical trials or in obtaining marketing approvals. We do not know whether any of our planned preclinical studies or clinical trials will begin on a timely basis or at all, will need to be restructured or will be completed on schedule, or at all. For example, FDA imposed a partial clinical hold on our ongoing Phase 3 clinical trial of DCC-2618 in fourth-line GIST, which limits enrollment to not more than 50 patients until we submit draft toxicology reports to FDA

and wait 30 days for any FDA response to this submission. Our ongoing Phase 1 trial of DCC-2618 continues to generate additional data that also may be requested by FDA. FDA may request additional information or data and any such requests could result in clinical trial delays. Furthermore, FDA could place a clinical hold, either another partial clinical hold or a full clinical hold, on our DCC-2618 Phase 3 trial if they are not satisfied with the information we provide to them, which could result in delays for the trial. Significant preclinical or clinical trial delays also could shorten any periods during which we may have the exclusive right to commercialize our drug candidates or allow our competitors to bring products to market before we do and impair our ability to successfully commercialize our drug candidates and may harm our business and results of operations.

We may utilize companion diagnostics in our planned clinical trials in the future in order to identify appropriate patient populations for our drug candidates. If a satisfactory companion diagnostic is not commercially available, we may be required to create or obtain one that would be subject to regulatory approval requirements. The process of obtaining or creating such diagnostic is time consuming and costly.

We may ultimately be unable to complete the development and commercialization of our drug candidates.

We may be required to conduct additional clinical trials or other testing of our drug candidates beyond those that we currently contemplate, if we are unable to successfully complete clinical trials for our drug candidates or other testing, obtain results of these trials or tests that are not positive or are only modestly positive or if there are safety concerns. For example, in GIST, we have initiated the first pivotal Phase 3 trial of DCC-2618 in fourth-line plus GIST and plan to initiate a second Phase 3 clinical trial in second-line GIST; however, FDA may require us to conduct a clinical trial in third-line GIST before we may conduct one in second-line GIST. In addition, while we plan to conduct only one pivotal Phase 3 trial for each indication of fourth-line plus GIST and second-line GIST, for a single randomized trial to support submission to FDA of a new drug application, or NDA, the trial must be well-designed, well-conducted, with a favorable risk/benefit ratio, and provide statistically persuasive efficacy findings so compelling that a second trial would be unethical or practically impossible to repeat. In addition, certain data that we have presented to date have been generated and reviewed at the clinical sites and are preliminary. The data may be subject to subsequent central review, and based on that review, there may be changes to these data which may not be favorable. For example, in our ongoing Phase 1 clinical trial of DCC-2618, there have been differences observed between imaging data assessed at the clinical sites in accordance with the Phase 1 protocol and by central review, including some instances where central review's findings regarding the number of objective responses were less favorable than those previously reported. In addition to certain imaging results from our Phase 1 trial, we also plan to have all of the data from our Phase 3 trials of DCC-2618 centrally reviewed. The results from our Phase 3 trials of DCC-2618 in which all data will be subject to central review may be less favorable than the results of the escalation stage of our Phase 1 trial of DCC-2618 that were based on data that has not been subject to central review. Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that have believed their drug candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval of their products.

We intend to change the manufacturing process we are using to make clinical supplies of DCC-2618 for our ongoing clinical trials in anticipation of greater drug requirements for commercialization, if we obtain regulatory approval. We will be required to demonstrate comparability, which may include conducting a bioequivalence study, of DCC-2618 made with the new process t to DCC-2618 we have used in clinical trials to date. If we are unable to establish comparability or bioequivalency, or are unable to agree with FDA on a timely basis regarding the study design necessary to do so, the commercialization of DCC-2618 may be substantially delayed or constrained by supply. In addition, we have not demonstrated that our changed manufacturing process can be run at sufficient scale to support our projected commercial requirements of DCC-2618. If we are unable to manufacture sufficient quantities of DCC-2618 to meet commercial demand, our business and results of operations will be harmed.

We may:

- be delayed in obtaining marketing approval for DCC-2618 or our other drug 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 safety warnings;
- be subject to additional post-marketing testing requirements; or
- have the product removed from the market after obtaining marketing approval.

If we experience delays or difficulties in the enrollment of patients in clinical trials, including in our Phase 3 clinical trials of DCC-2618, our receipt of necessary marketing approvals could be delayed or prevented.

We may not be able to initiate or continue clinical trials for our drug candidates if we are unable to locate and enroll a sufficient number of eligible patients to participate in these trials as required by FDA or similar regulatory authorities outside of the United States. In particular, most of the GIST patients we have enrolled in our Phase 1 trial of DCC-2618 have been fourth-line GIST patients. However, we have enrolled a limited number of second-line GIST patients in our Phase 1 trial and intend to enroll second-line GIST patients in a future Phase 3 trial. We cannot predict how difficult it will be to enroll GIST patients for future trials in earlier lines of therapy such as second- and third-line GIST where alternative therapies already are approved. Therefore, our ability to identify and enroll eligible patients for these clinical trials and possibly other clinical trials may be limited or may result in slower enrollment than we anticipate. In addition, some of our competitors have ongoing, or planned, clinical trials for drug candidates that treat the same indications as our drug candidates, and patients who would otherwise be eligible for our clinical trials may instead enroll in clinical trials for our competitors' drug candidates. Patient enrollment is affected by other factors including:

- the severity of the disease under investigation;
- the eligibility criteria for the trial in question;
- the perceived risks and benefits of the drug candidates under study;
- the efforts to facilitate timely enrollment in clinical trials;
- the patient referral practices of physicians;
- the burden on patients due to inconvenient procedures;
- the ability to monitor patients adequately during and after treatment; and
- the proximity and availability of clinical trial sites for prospective patients.

Our inability to enroll a sufficient number of patients for our clinical trials would result in significant delays and could require us to abandon one or more clinical trials altogether. Enrollment delays in our clinical trials may result in increased development costs for our drug candidates, which would cause significant harm to our financial position, adversely impact our stock price and impair our ability to raise capital.

If serious adverse or unacceptable side effects are identified during the development of our drug candidates, we may need to abandon or limit our development of some of our drug candidates.

If our drug candidates are associated with serious adverse events or undesirable side effects in clinical trials or have characteristics that are unexpected, we may need to abandon their development, limit development to more narrow uses or subpopulations in which the serious adverse events, undesirable side effects or other characteristics are less prevalent, less severe or more acceptable from a risk-benefit perspective or highlight these

risks, side effects or other characteristics in the approved product label. In pharmaceutical development, many drugs that initially show promise in early-stage testing for treating cancer may later be found to cause side effects that prevent further development of the drug. Currently marketed therapies for the treatment of cancer are generally limited to some extent by their toxicity. In addition, some of our drug candidates would be chronic therapies or be used in pediatric populations, for which safety concerns may be particularly important. Use of our drug candidates as monotherapies may also result in adverse events consistent in nature with other marketed therapies. In addition, if used in combination with other therapies in the future, our drug candidates may exacerbate adverse events associated with the therapy. If serious adverse or unexpected side effects are identified during development, we may be required to develop a Risk Evaluation and Mitigation Strategy, or REMS, to mitigate those serious safety risks, which could impose significant distribution and/or use restrictions on our products.

If we are unable to successfully develop companion diagnostic tests for our drug candidates that require such tests, or experience significant delays in doing so, we may not realize the full commercial potential of these drug candidates.

We may develop, either by ourselves or with collaborators, in vitro companion diagnostic tests for our drug candidates for certain indications. To be successful, we or our collaborators will need to address a number of scientific, technical, regulatory and logistical challenges. FDA regulates in vitro companion diagnostics as medical devices that will likely be subject to clinical trials in conjunction with the clinical trials for our drug candidates, and which will require regulatory clearance or approval prior to commercialization. We may rely on third parties for the design, development and manufacture of companion diagnostic tests for our therapeutic drug candidates that require such tests. If these parties are unable to successfully develop companion diagnostics for these therapeutic drug candidates, or experience delays in doing so, the development of these therapeutic drug candidates may be adversely affected, these therapeutic drug candidates may not obtain marketing approval, and we may not realize the full commercial potential of any of these therapeutics that obtain marketing approval. As a result, our business, results of operations and financial condition could be materially harmed.

The failure to obtain required regulatory clearances or approvals for any companion diagnostic tests that we may pursue may prevent or delay approval of any of our drug candidates. Moreover, the commercial success of any of our drug candidates that require a companion diagnostic will be tied to the receipt of any required regulatory clearances or approvals and the continued availability of such tests.

In connection with the clinical development of our drug candidates for certain indications we may work with collaborators to develop or obtain access to in vitro companion diagnostic tests to identify appropriate patients for our drug candidates. We may rely on third parties for the development, testing and manufacturing of these companion diagnostics, the application for and receipt of any required regulatory clearances or approvals, and the commercial supply of these companion diagnostics. Our third-party collaborators may fail to obtain the required regulatory clearances or approvals, which could prevent or delay approval of our drug candidates. In addition, the commercial success of any of our drug candidates that require a companion diagnostic will be tied to and dependent upon the receipt of required regulatory clearances or approvals and the continued ability of such third parties to make the companion diagnostic commercially available to us on reasonable terms in the relevant geographies.

We may expend our limited resources to pursue a particular drug candidate or indication and fail to capitalize on drug candidates or indications that may be more profitable or for which there is a greater likelihood of success.

Because we have limited financial and managerial resources, we focus on research programs and drug candidates that we identify for specific indications. As a result, we may forego or delay pursuit of opportunities with other drug candidates or for other indications that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable

market opportunities. Our spending on current and future research and development programs and drug candidates for specific indications may not yield any commercially viable products. If we do not accurately evaluate the commercial potential or target market for a particular drug candidate, we may relinquish valuable rights to that drug candidate through collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such drug candidate.

We may not be successful in our efforts to discover additional potential drug candidates.

A key element of our strategy is to apply our proprietary knowledge and our understanding of the structure, biology and activity of kinase inhibitors that target the switch pocket to develop drug candidates. The therapeutic discovery activities that we are conducting may not be successful in identifying drug candidates that are useful in treating cancer or other diseases. Our research programs may initially show promise in identifying potential drug candidates, yet fail to yield drug candidates for clinical development for a number of reasons, including:

- the research methodology used may not be successful in identifying potential drug candidates;
- potential drug candidates may, on further study, be shown to have harmful side effects or other characteristics that indicate that they are unlikely to be drugs that will obtain marketing approval or achieve market acceptance; or
- potential drug candidates may not be effective in treating their targeted diseases.

Research programs to identify new drug candidates require substantial technical, financial and human resources. We may choose to focus our efforts and resources on a potential drug candidate that ultimately proves to be unsuccessful. If we are unable to identify suitable drug candidates for preclinical and clinical development, we will not be able to obtain revenues from sale of products in future periods, which likely would result in significant harm to our financial position and adversely impact our stock price.

Risks Related to the Commercialization of Our Drug Candidates

The incidence and prevalence for target patient populations of our drug candidates have not been established with precision. If the market opportunities for our drug candidates are smaller than we estimate or if any approval that we obtain is based on a narrower definition of the patient population, our revenue potential and ability to achieve profitability will be adversely affected.

The precise incidence and prevalence for GIST, advanced systemic mastocytosis, or ASM, gliomas, including glioblastoma multiforme, or GBM, and other solid tumors driven by KIT or PDGFRα are unknown. Our projections of both the number of people who have these diseases, as well as the subset of people with these diseases who have the potential to benefit from treatment with our drug candidates, are based on estimates.

The total addressable market opportunity for DCC-2618, DCC-3014, rebastinib and any other drug candidates we may produce will ultimately depend upon, among other things, the diagnosis criteria included in the final label for each such drug candidate, if our drug candidates are approved for sale for these indications, acceptance by the medical community and patient access, drug pricing and reimbursement. The number of patients in our targeted commercial markets and elsewhere may turn out to be lower than expected, patients may not be otherwise amenable to treatment with our drugs, or new patients may become increasingly difficult to identify or gain access to, all of which would adversely affect our results of operations and our business.

Even if any of our drug 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.

If any of our drug candidates receives marketing approval, it may nonetheless fail to gain sufficient market acceptance by physicians, patients, third-party payors and others in the medical community. For example, current

cancer treatments, such as existing targeted therapies, chemotherapy, and radiation therapy, are well established in the medical community, and doctors may continue to rely on these treatments. If our drug candidates do not achieve an adequate level of acceptance, we may not generate significant product revenues and we may not become profitable. The degree of market acceptance of our drug candidates, if approved for commercial sale, will depend on a number of factors, including:

- the efficacy and potential advantages compared to alternative treatments;
- the prevalence and severity of any side effects, in particular compared to alternative treatments;
- our ability to offer our products for sale at competitive prices;
- the convenience and ease of administration compared to alternative treatments;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- the strength of marketing and distribution support;
- the existence of distribution and/or use restrictions, such as through a REMS;
- the availability of third-party coverage and adequate reimbursement;
- the timing of any marketing approval in relation to other product approvals;
- support from patient advocacy groups;
- the labeling of our products, including any significant use or distribution restrictions or safety warnings; and
- any restrictions on the use of our products together with other medications.

If we are unable to establish sales and marketing capabilities, we may not be successful in commercializing our drug candidates if and when they are approved.

We do not have a sales or marketing infrastructure and have no experience in the sale, marketing or distribution of biopharmaceutical products. To achieve commercial success for any product for which we obtain marketing approval, we will need to establish sales, marketing and distribution capabilities, either ourselves or through collaboration or other arrangements with third parties.

We plan to build our own focused, specialized sales and marketing organization in the United States and to selectively establish partnerships in markets outside the United States to support the commercialization of drug candidates for which we obtain marketing approval and that can be commercialized with such capabilities.

There are risks involved with establishing our own sales and marketing capabilities. For example, recruiting and training a sales force is expensive and time-consuming and could delay any product launch. If the commercial launch of a drug candidate for which we recruit a sales force and establish marketing capabilities is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses. These efforts are expected to be costly, and our investment would be lost if we cannot retain or reposition our sales and marketing personnel.

Factors that may inhibit our efforts to commercialize our products on our own include:

- our inability to recruit, train and retain adequate numbers of effective sales and marketing personnel;
- our inability to raise financing necessary to build our commercialization infrastructure;
- the inability of sales personnel to obtain access to physicians or persuade an adequate number of physicians to prescribe any future products;

- the lack of complementary products to be offered by sales personnel, which may put us at a competitive disadvantage relative to companies with more extensive product lines; and
- unforeseen costs and expenses associated with creating an independent sales and marketing organization.

As a result of any potential partnerships in markets outside of the United States, or if we are unable to establish our own sales and marketing capabilities in the United States and instead enter into arrangements with third parties to perform these services, our product revenues and our profitability, if any, are likely to be lower than if we were to market and sell any products that we develop ourselves. In addition, we may not be successful in entering into arrangements with third parties to market and sell our drug candidates or may be unable to do so on terms that are acceptable to us. We likely will have little control over such third parties, and any of these third parties may fail to devote the necessary resources and attention to sell and market our products effectively. If we do not establish sales and marketing capabilities successfully, either on our own or in collaboration with third parties, we will not be successful in commercializing any of our drug candidates for which we receive marketing approval.

We face substantial competition, which may result in others discovering, developing or commercializing products before or more successfully than we do.

The development and commercialization of new pharmaceutical and biotechnology products is highly competitive. We face competition with respect to our current drug candidates, and will face competition with respect to any drug candidates that we may seek to develop or commercialize in the future, from major pharmaceutical companies, specialty pharmaceutical companies and biotechnology companies worldwide. There are a number of large pharmaceutical and biotechnology companies that currently market and sell products or are pursuing the development of products for the treatment of the disease indications for which we are developing our drug candidates. Some of these competitive products and therapies are based on scientific approaches that are similar to our approach, and others are based on entirely different approaches. Potential competitors also include academic institutions, government agencies and other public and private research organizations that conduct research, seek patent protection and establish collaborative arrangements for research, development, manufacturing and commercialization.

Specifically, there are a large number of pharmaceutical and biotechnology companies developing or marketing treatments for cancer that would be competitive with the drug candidates we are developing, if our drug candidates are approved. Many of these companies are developing cancer therapeutics that are also kinase inhibitors. Although there are currently marketed drugs that have activity in GIST through their targeting of primary and secondary KIT mutants, no currently marketed drug directly targets certain secondary resistance mutations in KIT and PDGFRα, or provides coverage of all KIT and PDGFRα mutants. With respect to DCC-2618, there are a number of large pharmaceutical companies and biotechnology companies marketing small molecule drugs or biologic drugs for the treatment of GIST, including Novartis AG, Pfizer Inc., and Bayer AG. We are also aware of pharmaceutical and biotechnology companies developing drugs for the treatment of GIST and systemic mastocytosis including AB Science S.A., Arog Pharmaceuticals, Inc., ARIAD Pharmaceuticals, Inc., a wholly owned subsidiary of Takeda Pharmaceuticals U.S.A., Blueprint Medicines Corporation, Bristol-Myers Squibb Company, Celldex Therapeutics, Inc., Novartis AG, Plexxikon Inc., a wholly owned subsidiary of Daiichi Sanyko Company, Limited and Xencor, Inc. Some of these competitors are further along in their clinical development programs than we are in ours. Further, there are a large number of pharmaceutical and biotechnology companies developing antibody or small molecule colony stimulating factor receptor 1, or CSF1R, inhibitors that we are seeking to target in our DCC-3014 program, including Array BioPharma Inc., Amgen Inc., Eli Lilly and Company, Bristol-Myers Squibb Company, Five Prime Therapeutics, Inc., Roche Holding Ltd, Novartis AG, Plexxikon Inc. and Syndax Pharmaceuticals, Inc.

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 approved for broader

indications or patient populations, are more convenient or are less expensive than any products that we may develop. Our competitors also may obtain FDA or other marketing approval for their products more rapidly than any approval we may obtain for ours, which could result in our competitors establishing a strong market position before we are able to enter the market. In addition, our ability to compete may be affected in many cases by insurers or other third-party payors seeking to encourage the use of generic products. Generic products are currently on the market for some of the indications that we are pursuing, and additional products are expected to become available on a generic basis over the coming years. If our drug candidates achieve marketing approval, we expect that they will be priced at a significant premium over any competitive generic products.

Many of the companies against which we are competing or against which we may compete in the future have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining marketing approvals and marketing and selling 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 and other early- stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These third parties compete with us in recruiting and retaining qualified scientific, management and sales and marketing personnel, establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs.

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

The regulations that govern marketing approvals, pricing, coverage and reimbursement for new drug products vary widely from country to country. Current and future legislation may significantly change the approval requirements in ways that could involve additional costs and cause delays in obtaining approvals. Some countries require approval of the sale price of a drug before it can be marketed. In many countries, the pricing review period begins after marketing or product licensing approval is granted. To obtain reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost-effectiveness of our drug candidate to other available therapies. In some foreign markets, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. As a result, we might obtain marketing approval for a drug candidate in a particular country, but then be subject to price regulations that delay our commercial launch of the product, possibly for lengthy time periods, and negatively impact the revenues, if any, we are able to generate from the sale of the product in that country. Adverse pricing limitations may hinder our ability to recoup our investment in one or more drug candidates, even if such drug candidates obtain marketing approval.

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

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

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

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

- decreased demand for any drug 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 the related litigation;
- substantial monetary awards to trial participants or patients;
- loss of revenue;
- reduced resources of our management to pursue our business strategy; and
- the inability to commercialize any products that we may develop.

We currently hold $5.0 million in product liability insurance coverage in the aggregate for the United States and certain other jurisdictions, with a per incident limit of $5.0 million, which may not be adequate to cover all liabilities that we may incur. We likely will need to increase our insurance coverage as we expand our clinical trials or if we commence commercialization of our drug candidates. Insurance coverage is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise.

Risks Related to Our Dependence on Third Parties

We may enter into collaborations with third parties for the development and commercialization of our drug candidates. If those collaborations are not successful, we may not be able to capitalize on the market potential of these drug candidates.

We have in the past, and may in the future, seek third-party collaborators for the development and commercialization of some of our drug candidates on a selected basis. Our likely collaborators for any collaboration arrangements include large and mid-size pharmaceutical companies, regional and national pharmaceutical companies and biotechnology companies.

If we do enter into any such arrangements with any third parties, we will likely have limited control over the amount and timing of resources that our collaborators dedicate to the development or commercialization of our drug candidates. Our ability to generate revenues from these arrangements will depend on our collaborators' abilities and efforts to successfully perform the functions assigned to them in these arrangements. Collaborations involving our drug candidates would pose numerous risks to us, including the following:

- collaborators have significant discretion in determining the efforts and resources that they will apply to these collaborations and may not perform their obligations as expected;
- collaborators may deemphasize or not pursue development and commercialization of our drug candidates or may elect not to continue or renew development or commercialization programs based on clinical trial results, changes in the collaborators' strategic focus, including as a result of a sale or disposition of a business unit or development function, or available funding or external factors such as an acquisition that diverts resources or creates competing priorities;
- collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a drug candidate, repeat or conduct new clinical trials or require a new formulation of a drug candidate for clinical testing;
- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our products or drug candidates if the collaborators believe that competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;
- a collaborator with marketing and distribution rights to multiple products may not commit sufficient resources to the marketing and distribution of our product relative to other products;
- collaborators may not properly obtain, maintain, defend or enforce our intellectual property rights or may use our proprietary information and intellectual property in such a way as to invite litigation or other intellectual property related proceedings that could jeopardize or invalidate our proprietary information and intellectual property or expose us to potential litigation or other intellectual property related proceedings;
- disputes may arise between the collaborators and us that result in the delay or termination of the research, development or commercialization of our products or drug candidates or that result in costly litigation or arbitration that diverts management attention and resources;
- collaborations may be terminated and, if terminated, may result in a need for additional capital to pursue further development or commercialization of the applicable drug candidates;
- collaboration agreements may not lead to development or commercialization of drug candidates in the most efficient manner or at all; and
- if a collaborator of ours were to be involved in a business combination, the continued pursuit and emphasis on our product development or commercialization program could be delayed, diminished or terminated.

If we are not able to establish collaborations, we may have to alter our development and commercialization plans.

Our drug development programs and the potential commercialization of our drug candidates will require substantial additional cash to fund expenses. In the past, we have been party to a collaboration agreement, which was concluded before completion because our collaboration partner elected not to pursue the development of the drug candidate beyond Phase 1 clinical trials. For some of our drug candidates, we may in the future decide to collaborate with pharmaceutical and biotechnology companies for the development and potential commercialization of those drug candidates.

We face significant competition in seeking appropriate collaborators. Our ability to reach a definitive agreement for a 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 following:

- the design or results of clinical trials;
- the likelihood of approval by FDA or similar regulatory authorities outside of the United States;
- the potential market for the subject drug candidate;
- the costs and complexities of manufacturing and delivering such drug candidate to patients;
- the potential of competing products;
- the existence of uncertainty with respect to our ownership of technology or other rights, which can exist if there is a challenge to such ownership without regard to the merits of the challenge; and
- industry and market conditions generally.

The collaborator may also consider alternative drug candidates or technologies for similar indications that may be available to collaborate on and whether such a collaboration could be more attractive than the one with us for our drug candidate. We may also be restricted under any license agreements from entering into agreements on certain terms or at all with potential collaborators. 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 and changes to the strategies of the combined company.

We may not be able to negotiate collaborations on a timely basis, on acceptable terms, or at all. If we are unable to do so, we may have to curtail the development of such drug candidate, reduce or delay one or more of our other development programs, delay the potential commercialization or reduce the scope of any sales or marketing activities for such drug candidate, or increase our expenditures and undertake development, manufacturing or commercialization activities at our own expense. If we elect to increase our expenditures to fund development, manufacturing 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 drug candidates or bring them to market and generate product revenue.

We rely, and expect to continue to rely, on third parties to conduct our clinical trials, and those third parties may not perform satisfactorily, including failing to meet deadlines for the completion of such trials.

We currently rely on various third-party clinical research organizations, or CROs, to conduct our ongoing Phase 1 clinical trials for DCC-2618 and DCC-3014 and our ongoing Phase 3 clinical trial of DCC-2618 in fourth-line plus GIST patients and do not plan to independently conduct any clinical trials for our other drug candidates. We expect to continue to rely on third parties, such as CROs, clinical data management organizations, medical institutions and clinical investigators, to conduct our clinical trials. Agreements with these third parties might terminate for a variety of reasons, including a failure to perform by the third parties. If we need to enter into alternative arrangements, that would delay our product development activities.

Our reliance on these third parties for research and development activities will reduce our control over these activities but will not relieve us of our responsibilities. For example, we will 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, FDA requires us to comply with requirements, commonly referred to as 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.

Furthermore, these third parties may have relationships with other entities, some of which may be our competitors. If these third parties do not successfully carry out their contractual duties, meet expected deadlines or conduct our clinical trials in accordance with regulatory requirements or our stated protocols, we will not be able to obtain, or may be delayed in obtaining, marketing approvals for our drug candidates and will not be able to, or may be delayed in our efforts to, successfully commercialize our drug candidates.

Manufacturing pharmaceutical products is complex and subject to product loss for a variety of reasons. We contract with third parties for the manufacture of our drug candidates for preclinical testing and clinical trials and expect to continue to do so for commercialization. This reliance on third parties increases the risk that we will not have sufficient quantities of our drug candidates or products or such quantities at an acceptable cost or quality, which could delay, prevent or impair our development or commercialization efforts.

We do not have any manufacturing facilities. We produce in our laboratory very small quantities of small molecules for evaluation in our research programs. We rely, and expect to continue to rely, on third parties for the manufacture of our drug candidates for preclinical and clinical testing, as well as for commercial manufacture if any of our drug candidates obtain marketing approval. Some of our manufacturers represent our sole source of supply. This reliance on third parties increases the risk that we will not have sufficient quantities of our drug candidates or products or such quantities at an acceptable cost or quality, which could delay, prevent or impair our development or commercialization efforts.

We may be unable to establish any agreements with third-party manufacturers or to do so on acceptable terms. Even if we are able to establish agreements with third-party manufacturers, reliance on third-party manufacturers entails additional risks, including:

- reliance on the third party for regulatory, compliance and quality assurance;
- the possible breach of the manufacturing agreement by the third party;
- the possible misappropriation of our proprietary information, including our trade secrets and know how; and
- the possible termination or nonrenewal of the agreement by the third party at a time that is costly or inconvenient for us.

We have only limited supply arrangements in place with respect to our drug candidates, and these arrangements do not extend to commercial supply. We acquire many key materials on a purchase order basis. As a result, we do not have long-term committed arrangements with respect to our drug candidates and other materials. If we obtain marketing approval for any of our drug candidates, we will need to establish an agreement for commercial manufacture with a third party.

We may depend on the proprietary technology of our third-party manufacturers for certain of our drug candidates, including DCC-2618. If we are not able to negotiate commercial supply terms with any such third-party manufacturers, we may be unable to commercialize our products if they were to be approved. Or, if we are forced to accept unfavorable terms for our relationships with any such third-party manufacturer, our business and financial condition would be materially harmed.

Third-party manufacturers may not be able to comply with current good manufacturing practices, or cGMP, regulations or similar regulatory requirements outside of the United States. Our failure, or the failure of our third-party manufacturers, to comply with applicable regulations could result in sanctions being imposed on us, including clinical holds, fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, license revocation, seizures or voluntary recalls of drug candidates or products, operating restrictions and criminal prosecutions, any of which could significantly and adversely affect supplies of our products.

Our drug candidates and any products that we may develop may compete with other drug candidates and products for access to manufacturing facilities. As a result, we may not obtain access to these facilities on a

priority basis or at all. There are a limited number of manufacturers that operate under cGMP regulations and that might be capable of manufacturing for us.

We have not yet scaled up the commercial manufacturing process for any of our drug candidates. Even minor deviations from normal manufacturing processes could result in reduced production yields, product defects and other supply disruptions. If microbial, viral or other contaminations are discovered in our drug candidates or in the manufacturing facilities in which our drug candidates are made, such manufacturing facilities may need to be closed for an extended period of time to investigate and remedy the contamination.

Any performance failure on the part of our existing or future manufacturers could delay clinical development or marketing approval. We do not currently have arrangements in place for redundant supply or a second source for bulk drug substance. If our current contract manufacturers for preclinical and clinical testing cannot perform as agreed, we may be required to replace such manufacturers. Although we believe that there are several potential alternative manufacturers who could manufacture our drug candidates, we may incur added costs and delays in identifying and qualifying any such replacement manufacturer or be able to reach agreement with any alternative manufacturer.

Our current and anticipated future dependence upon others for the manufacture of our drug candidates or products may adversely affect our future profit margins and our ability to commercialize any products that obtain 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 drug candidates, or if the scope of the patent protection is not sufficiently broad, third parties, including our competitors, could develop and commercialize products similar or identical to ours, and our ability to commercialize our drug candidates successfully may be adversely affected.

Our success depends in large part on our ability to protect our proprietary technologies that we believe are important to our business, including pursuing and maintaining patent protection intended to cover the composition of matter of our drug candidates, for example, DCC-2618, DCC-3014 and rebastinib, their methods of use, related technologies and other inventions that are important to our business. In addition to patent protection, we also rely on trade secrets to protect aspects of our business that are not amenable to, or that we do not consider appropriate for, patent protection, including our proprietary kinase switch control inhibitor platform. If we do not adequately obtain, maintain, protect or enforce our intellectual property, third parties, including our competitors, may be able to erode or negate any competitive advantage we may have, which could harm our business and ability to achieve profitability.

The patent application and approval process is expensive, time-consuming and complex. We may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. It is also possible that we fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection.

Furthermore, 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. The standards applied by the United States Patent and Trademark Office, or USPTO, and foreign patent offices in granting patents are not always applied uniformly or predictably. 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. Thus, we cannot offer any assurances about which, if any, patents will issue, the breadth of any such patents, whether any issued patents will be found invalid and unenforceable or will be threatened by third parties or whether any issued patents will effectively prevent others from commercializing competing technologies and drug candidates.

Our pending patent applications cannot be enforced against third parties practicing the technology claimed in such applications unless and until a patent issues from such applications. 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. Since patent applications in the United States and most other countries are confidential for a period of time after filing, and some remain so until issued, we cannot be certain that we were the first to file or invent (prior to March 16, 2013) any patent application related to our drug candidates. In addition, we enter into non-disclosure and confidentiality agreements with parties who have access to confidential or patentable aspects of our research and development output, such as our employees, collaborators, consultants, advisors and other third parties; however, any of these parties may breach the agreements and disclose such output before a patent application is filed, thereby jeopardizing our ability to seek patent protection. Furthermore, if third parties have filed patent applications related to our drug candidates or technology, an interference proceeding in the United States can be initiated by the USPTO or a third party to determine who was the first to invent any of the subject matter covered by the patent claims of our applications. 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.

Moreover, because the issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, our patents or pending patent applications may be challenged in the courts or patent offices in the United States and abroad. For example, we may be subject to a third-party preissuance submission of prior art to the USPTO or become involved in post-grant review procedures, oppositions, derivations, revocation, reexaminations, inter partes review or interference proceedings, in the United States or elsewhere, challenging our patent rights or the patent rights of others. An adverse determination in any such challenge may result in loss of exclusivity or in our 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. Such challenges also may result in substantial cost and require significant time from our scientists and management, even if the eventual outcome is favorable to us. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations, and prospects.

In addition, given the amount of time required for the development, testing and regulatory review of new drug candidates, our patents protecting such drug candidates might expire before or shortly after such drug candidates are commercialized. As a result, our intellectual property may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours. Moreover, some of our patents may in the future be co-owned with third parties. If we are unable to obtain an exclusive license to any such third-party co-owners' interest in such patents or patent applications, such co-owners may be able to license their rights to other third parties, including our competitors, and our competitors could market competing products and technology. In addition, we may need the cooperation of any such co-owners of our patents in order to enforce such patents against third parties, and such cooperation may not be provided to us. Any of the foregoing could have a material adverse effect on our competitive position, business, financial conditions, results of operations, and prospects.

Our pending and future patent applications may not result in patents being issued that protect our drug 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. Moreover, the coverage claimed in a patent application can be significantly reduced before the patent is issued and its scope can be reinterpreted after issuance. Our competitors and other third parties may be able to circumvent our patents by developing similar or alternative technologies or products in a non-infringing manner. Our competitors and other third parties may also seek approval to market their own products similar to or otherwise competitive with our products. Alternatively, our competitors or other third parties may seek to market generic or biosimilar versions of any approved products and in so doing, 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. Thus, even if we have valid and enforceable patents, these patents still may not provide protection against competing products or processes sufficient to achieve our business objectives.

Furthermore, our patents may be subject to a reservation of rights by one or more third parties. For example, if any of our technologies are developed in the future with government funding, the government may obtain certain rights in any resulting patents, including a non-exclusive license authorizing the government to use the invention or to have others use the invention on its behalf. If the U.S. government then decides to exercise these rights, it is not required to engage us as its contractor in connection with doing so. These rights may also permit the government to disclose our confidential information to third parties and to exercise march-in rights to use or allow third parties to use our licensed technology. The government may also exercise its march-in rights if it determines that action is necessary because we failed to achieve practical application of the government-funded technology, because action is necessary to alleviate health or safety needs, to meet requirements of federal regulations, or to give preference to U.S. industry. In addition, our rights in such government-funded inventions may be subject to certain requirements to manufacture products embodying such inventions in the United States. Any exercise by the government of aforementioned proprietary rights could harm our competitive position, business, financial condition, results of operations, and prospects.

Changes to the patent law in the United States and other jurisdictions could diminish the value of patents in general, thereby impairing our ability to protect our products.

As is the case with other biopharmaceutical companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the biopharmaceutical industry involves both technological and legal complexity and is therefore costly, time consuming and inherently uncertain. Changes in either the patent laws or interpretation of the patent laws in the United States could increase the uncertainties and costs surrounding the prosecution of patent applications and the enforcement or defense of issued patents. Recent patent reform legislation in the United States and other countries, including the Leahy-Smith America Invents Act, or the Leahy-Smith Act, signed into law in September 2011, could increase those uncertainties and costs. The Leahy-Smith Act includes a number of significant changes to U.S. patent law. These include provisions that affect the way patent applications are prosecuted, redefine prior art and provide more efficient and cost-effective avenues for competitors to challenge the validity of patents. For example, the Leahy-Smith Act allows third-party submission of prior art to the USPTO during patent prosecution and additional procedures to attack the validity of a patent by USPTO administered post-grant proceedings, including post-grant review, *inter partes* review, and derivation proceedings. In addition, the Leahy-Smith Act has transformed the U.S. patent system from a “first-to-invent” system to a “first-to-file” system in which, assuming that other requirements for patentability are met, the first inventor to file a patent application will be entitled to the patent on an invention regardless of whether a third party was the first to invent the claimed invention. The first-to-file provisions, however, only became effective on March 16, 2013. Accordingly, it is not yet clear what, if any, impact the Leahy-Smith Act will have on the operation of our business. However, the Leahy-Smith Act and its implementation could make it more difficult to obtain patent protection for our inventions and increase the uncertainties and costs surrounding the prosecution of our or our collaboration partners’ patent applications and the enforcement or defense of our or our collaboration

partners' issued patents, all of which could harm our business, results of operations, financial condition and prospects.

The U.S. Supreme Court has ruled on several patent cases in recent years, either narrowing the scope of patent protection available in certain circumstances or weakening the rights of patent owners in certain situations. Additionally, there have been recent proposals for additional changes to the patent laws of the United States and other countries that, if adopted, could impact our ability to enforce our proprietary technology. Depending on future actions by the U.S. Congress, the U.S. courts, the USPTO and the relevant law-making bodies in other countries, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future.

We may become involved in lawsuits or administrative disputes 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, trade secrets 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. Any claims we assert against perceived infringers could provoke these parties to assert counterclaims against us alleging that we infringe their patents or their intellectual property, in addition to counterclaims asserting that our patents are invalid or unenforceable, or both. 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 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. Similarly, if we assert trademark infringement claims, a court may determine that the marks we have asserted are invalid or unenforceable, or that the party against whom we have asserted trademark infringement has superior rights to the marks in question. In this case, we could ultimately be forced to cease use of such trademarks.

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 or 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. There could also be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a material adverse effect on the price of shares of our common stock. 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.

Furthermore, third parties may also raise invalidity or unenforceability claims before administrative bodies in the United States or comparable foreign authorities, even outside the context of litigation. Such mechanisms include re-examination, inter partes review, post-grant review, interference proceedings, derivation proceedings and equivalent proceedings in foreign jurisdictions (e.g., opposition proceedings). Such proceedings could result in revocation, cancellation or amendment to our patents in such a way that they no longer cover and protect our drug candidates. The outcome following legal assertions of invalidity and unenforceability is unpredictable. With respect to the validity of our patents, for example, we cannot be certain that there is no invalidating prior art of which we, our licensors, our patent counsel and the patent examiner were unaware during prosecution. If a third

party were to prevail on a legal assertion of invalidity or unenforceability, we would lose at least part, and perhaps all, of the patent protection on one or more of our drug candidates. Any such loss of patent protection could have a material adverse impact on our business, financial condition, results of operations and prospects.

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

Filing, prosecuting and defending patents with respect to our drug candidates in all countries throughout the world would be prohibitively expensive, and the laws of foreign countries may not protect our rights to the same extent as the laws of the United States. The requirements for patentability may differ in certain countries, particularly in developing countries. In addition, our intellectual property license agreements may not always include worldwide rights. Consequently, competitors and other third parties may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and, further, may export otherwise infringing products to territories where we may obtain patent protection, but where patent enforcement is not as strong as that in the United States. These products may compete with our products in jurisdictions where we do not have any issued or licensed patents or where any future patent claims or other intellectual property rights may not be effective or sufficient to prevent them from competing with us.

Moreover, our ability to protect and enforce our intellectual property rights may be adversely affected by unforeseen changes in foreign intellectual property laws. Additionally, laws of some countries outside of the United States and Europe do not afford intellectual property protection to the same extent as the laws of the United States and Europe. Many companies have encountered significant problems in protecting and defending intellectual property rights in certain foreign jurisdictions. The legal systems of some countries, including India, China and other developing countries, do not favor the enforcement of patents and other intellectual property rights, particularly those relating to biotechnology products, which could make it difficult for us to stop the infringement, misappropriation or other violation of our patents or other intellectual property rights. For example, many foreign countries have compulsory licensing laws under which a patent owner must grant licenses to third parties. Consequently, we may not be able to prevent third parties from practicing our inventions in certain countries outside the United States and Europe. In addition, many countries limit the enforceability of patents against government agencies or government contractors. In these countries, the patent owner may have limited remedies, which could materially diminish the value of such patent. If we are forced to grant a license to third parties with respect to any patents relevant to our business, our competitive position may be impaired, and our business, financial condition, results of operations, and prospects may be adversely affected. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and, further, may export otherwise infringing products to territories where we have patent protection, if our ability to enforce our patents to stop infringing activities is inadequate. These products may compete with our products, and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing, which could have a material adverse effect on our business, financial condition, results of operations and prospects.

Proceedings to enforce our patent rights in foreign jurisdictions, whether or not successful, could result in substantial costs and divert our efforts and resources from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly, could put our patent applications at risk of not issuing, and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate, and the damages or other remedies awarded, if any, may not be commercially meaningful. Furthermore, while we intend to protect our intellectual property rights in major markets for our products, we cannot ensure that we will be able to initiate or maintain similar efforts in all jurisdictions in which we may wish to market our products. Accordingly, our efforts to protect our intellectual property rights in such countries may be inadequate.

If we are sued for infringing, misappropriating or otherwise violating intellectual property rights of third parties, such litigation or disputes could be costly and time consuming and could prevent or delay us from developing or commercializing our drug candidates.

Our commercial success depends, in part, on our ability to develop, manufacture, market and sell our drug candidates without infringing, misappropriating or otherwise violating the intellectual property and other proprietary rights of third parties. If any third party patents or patent applications are found to cover our drug candidates or their methods of use or manufacturing, we may be required to pay damages, which could be substantial, and we would not be free to manufacture or market our drug candidates without obtaining a license, which may not be available on commercially reasonable terms, or at all.

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 drug candidates, including interference proceedings before the USPTO. Third parties may assert infringement, misappropriation or other claims against us based on existing or future intellectual property rights. The outcome of intellectual property litigation and other disputes is subject to uncertainties that cannot be adequately quantified in advance. The pharmaceutical and biotechnology industries have produced a significant number of patents, and it may not always be clear to industry participants, including us, which patents cover various types of products or methods of using or manufacturing products. The coverage of patents is subject to interpretation by the courts, and the interpretation is not always uniform. If we were sued for patent infringement, we would need to demonstrate that our drug candidates, products or methods of use, manufacturing or other applicable activities either do not infringe the patent claims of the relevant patent or that the patent claims are invalid or unenforceable, and we may not be successful in doing so. Proving invalidity is difficult. For example, in the United States, proving invalidity requires a showing of clear and convincing evidence to overcome the presumption of validity enjoyed by issued patents. Even if we believe third-party intellectual property claims are without merit, there is no assurance that a court would find in our favor on questions of infringement, validity, or enforceability. 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 significantly harm our business and operating results. In addition, we may not have sufficient resources to bring these actions to a successful conclusion.

If we are found to infringe, misappropriate or otherwise violate a third party's intellectual property rights and we are unsuccessful in demonstrating that such intellectual property rights are invalid or unenforceable, we could be forced, including by court order, to cease developing, manufacturing or commercializing the infringing drug candidate or product. 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 drug 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 drug 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, financial condition, results of operations and prospects.

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

Some of our employees and consultants are currently or have been previously employed at universities or at other biotechnology or pharmaceutical companies, including our competitors or potential competitors. These employees and consultants may have executed proprietary rights, non-disclosure and non-competition

agreements, or similar agreements, in connection with such other current or previous employment. Although we try to ensure that our employees and consultants do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that we or these individuals have used or disclosed intellectual property, including trade secrets or other proprietary information, of third parties. Litigation may be necessary to defend against such claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel or sustain damages. Such intellectual property rights could be awarded to a third party, and we could be required to obtain a license from such third party to commercialize our technology or products. Such a license may not be available on commercially reasonable terms or at all. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to our management. Any of the foregoing would have a material adverse effect on our business, financial condition, results of operations and prospects.

In addition, while we typically require our employees, consultants and contractors who may be involved in the development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an agreement with each party who in fact develops intellectual property that we regard as our own. In addition such agreements may not be self-executing such that the intellectual property subject to such agreements may not be assigned to us without additional assignments being executed, and we may fail to obtain such assignments. In addition, such agreements may be breached. Accordingly, we may be forced to bring claims against third parties, or defend claims that they may bring against us related to the ownership of such intellectual property. If we fail in prosecuting or defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights. Even if we are successful in prosecuting or defending against such claims, litigation could result in substantial costs and be a distraction to our senior management and scientific personnel, which would have a material adverse effect on our business, financial condition, results of operations and prospects.

The term of our patents may be inadequate to protect our competitive position on our products.

Given the amount of time required for the development, testing and regulatory review of new drug candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. Depending upon the timing, duration and other factors relating to any FDA marketing approval we receive for any of our drug candidates, one or more of our U.S. patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Action of 1984, or Hatch-Waxman Amendments. We expect to seek extensions of patent terms in the United States and, if available, in other countries where we are prosecuting patents. In the United States, the Hatch-Waxman Amendments permit a patent term extension of up to five years beyond the normal expiration of the patent as compensation for patent term lost during the regulatory review process. A patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval, only one patent may be extended and only those claims covering the approved drug, a method for using it, or a method for manufacturing it may be extended, and the application for the extension must be submitted prior to the expiration of the patent. However, the applicable authorities, including FDA and the USPTO in the United States, and any equivalent regulatory authority in other countries, may not agree with our assessment of whether such extensions are available for our patents, may refuse to grant extensions to our patents, or may grant more limited extensions than we request. We may not be granted an extension because of, for example, failing to exercise due diligence during the testing phase or regulatory review process, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents, or otherwise failing to satisfy applicable requirements. If we are unable to obtain patent term extension or the term of any such extension is less than we request, our competitors and other third parties may be able to obtain approval of competing products following our patent expiration and take advantage of our investment in development and clinical trials by referencing our clinical and preclinical data and launch their product earlier than might otherwise be the case. Any of the foregoing would have a material adverse effect on our business, financial condition, results of operations and prospects.

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

Periodic maintenance fees on any issued patent are due to be paid to the USPTO and patent offices in foreign countries in several stages over the lifetime of the patent. The USPTO and patent offices in foreign countries require compliance with a number of procedural, documentary, fee payment and other requirements during the patent application process. In certain circumstances, we rely on our licensing partners to pay these fees due to U.S. and non-U.S. patent agencies and to comply with these other requirements with respect to our licensed patents and patent applications. While an inadvertent lapse can be cured by payment of a late fee or by other means in accordance with the applicable rules, there are situations in which noncompliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of a patent or patent rights in the relevant jurisdiction. Non-compliance events that could result in abandonment or lapse of a patent or patent application include, but are not limited to, failure to respond to official actions within prescribed time limits, non-payment of fees and failure to properly legalize and submit formal documents. In such an event, our competitors and other third parties might be able to enter the market with similar or identical products of technology, which would have a material adverse effect on our business, financial condition, results of operations and prospects.

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 certain of our drug candidates, we also rely on proprietary know-how and trade secret protection and confidentiality agreements to protect proprietary know-how or trade secrets that are not patentable or that we elect not to patent. For example, we have elected to not patent our proprietary kinase switch control inhibitor platform and therefore rely on protecting the proprietary aspects of our platform as a trade secret. We seek to protect our trade secrets and proprietary know-how in part, by entering into non-disclosure and confidentiality agreements with parties who have access to them, such as our employees, consultants, independent contractors, advisors, contract manufacturers, suppliers, collaborators and other third parties. We also enter into confidentiality and invention or patent assignment agreements with employees and certain consultants. However, we cannot guarantee that we have entered into such agreements with each party that may have or have had access to our trade secrets or proprietary know-how. Additionally, our confidentiality agreements and other contractual protections may not be adequate to protect our intellectual property from unauthorized disclosure, third-party infringement or misappropriation. 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, including with respect to our proprietary kinase switch control inhibitor platform, were to be disclosed to or independently developed by a competitor or other third party, our business, financial condition, results of operations and prospects our business and competitive position could be materially harmed.

If we fail to comply with our obligations under any license, collaboration or other agreement, we may be required to pay damages and could lose intellectual property rights that are necessary for developing and protecting our drug candidates.

We rely, in part, on license, collaboration and other agreements. We may need to obtain additional licenses from others to advance our research or allow commercialization of our drug candidates and it is possible that we may be unable to obtain additional licenses at a reasonable cost or on reasonable terms, if at all. The licensing or acquisition of third party intellectual property rights is a competitive area, and several more established

companies may pursue strategies to license or acquire third-party intellectual property rights that we may consider attractive. These established companies may have a competitive advantage over us due to their size, capital resources and greater clinical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to use. We also may be unable to license or acquire third party intellectual property rights on terms that would allow us to make an appropriate return on our investment.

In addition, our present and future licenses, collaborations and other intellectual property related agreements, are likely to impose various development, commercialization, funding, milestone, royalty, diligence, sublicensing, insurance, patent prosecution and enforcement or other obligations on us. If we breach any of these obligations, or use the intellectual property licensed to us in an unauthorized manner, we may be required to pay damages and our licensors may have the right to terminate the license. If our license or other intellectual property related agreements are terminated, we may be required to cease developing and commercializing drug candidates that are covered by the licensed intellectual property. Disputes may arise regarding intellectual property subject to a licensing, collaboration or other agreement, including:

- the scope of rights granted under the agreement and other interpretation related issues;
- the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the agreement;
- the sublicensing of patent and other rights under our collaborative development relationships;
- our diligence obligations under the agreement and what activities satisfy those diligence obligations;
- the inventorship and ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our collaborators; and
- the priority of invention of patented technology.

In addition, the agreements under which we license intellectual property or technology to or from third parties are complex, and certain provisions in such agreements may be susceptible to multiple interpretations. The resolution of any contract interpretation disagreement that may arise could narrow what we believe to be the scope of our rights to the relevant intellectual property or technology, or increase what we believe to be our financial or other obligations under the relevant agreement, either of which could have a material adverse effect on our business, financial condition, results of operations and prospects. Moreover, if disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on commercially acceptable terms, we may be unable to successfully develop and commercialize the affected drug candidates.

In some circumstances, we may not have the right to control the preparation, filing and prosecution of patent applications, or to maintain the patents, covering the technology that we license from third parties. Therefore, we cannot be certain that these patents and applications will be prosecuted, maintained and enforced in a manner consistent with the best interests of our business. If our licensors fail to obtain or maintain such intellectual property, or lose rights to such intellectual property, the rights we have licensed and our exclusivity may be reduced or eliminated and our right to develop and commercialize any of our products that are subject to such licensed rights could be adversely affected.

Moreover, our rights to our in-licensed patents and patent applications may depend, in part, on inter-institutional or other operating agreements between the joint owners of such in-licensed patents and patent applications. If one or more of such joint owners breaches such inter-institutional or operating agreements, our rights to such in-licensed patents and patent applications may be adversely affected. In addition, while we cannot currently determine the amount of the royalty obligations we would be required to pay on sales of future products, if any, the amounts may be significant. The amount of our future royalty obligations will depend on the technology and intellectual property we use in products that we successfully develop and commercialize, if any.

Therefore, even if we successfully develop and commercialize products, we may be unable to achieve or maintain profitability. Any of the foregoing could have a material adverse effect on our competitive position, business, financial conditions, results of operations and prospects.

If we are unable to successfully obtain rights to required third party intellectual property rights or maintain the existing intellectual property rights we have, we may have to abandon development of the relevant program or drug candidate and our business, financial condition, results of operations and prospects could suffer.

Intellectual property rights do not necessarily address all potential threats.

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

- others may be able to make products similar to any drug candidates we may develop or utilize similarly related technologies that are not covered by the claims of the patents that we license or may own in the future;
- we, or our license partners or current or future collaborators, might not have been the first to make the inventions covered by the issued patent or pending patent application that we license or may own in the future;
- we, or our license partners or current or future collaborators, might not have been the first to file patent applications covering certain of our or their inventions;
- others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing, misappropriating or otherwise violating any of our owned or licensed intellectual property rights;
- it is possible that our pending licensed patent applications or those that we may own in the future will not lead to issued patents;
- issued patents that we hold rights to may be held invalid or unenforceable, including as a result of legal challenges by our competitors or other third parties;
- our competitors or other third parties might conduct research and development activities in countries where we do not have patent rights and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- we may not develop additional proprietary technologies that are patentable;
- the patents of others may harm our business; and
- we may choose not to file a patent in order to maintain certain trade secrets or know how, and a third party may subsequently file a patent covering such intellectual property.

Should any of these events occur, they could have a material adverse effect on our business, financial condition, results of operations and prospects.

Risks Related to Regulatory Approval and Marketing of Our Drug Candidates and Other Legal Compliance Matters

We have not received approval to market any of our drug candidates from regulatory authorities in any jurisdiction. Even if we complete the necessary clinical trials, the marketing approval process is expensive, time-consuming and uncertain and may prevent us from obtaining approvals for the commercialization of some or all of our drug candidates. If we are not able to obtain, or if there are delays in obtaining, required regulatory approvals, we will not be able to commercialize our drug candidates, and our ability to generate revenue will be materially impaired.

Our drug candidates and the activities associated with their development and commercialization, including their design, testing, manufacture, safety, efficacy, recordkeeping, labeling, storage, approval, advertising, promotion, sale and distribution are subject to comprehensive regulation by FDA and other regulatory agencies in the United States and by the EMA and similar regulatory authorities outside the United States. Failure to obtain marketing approval for a drug candidate will prevent us from commercializing the drug candidate. Our drug candidates are in the early stages of development and are subject to the risks of failure inherent in drug development. We have not received approval to market any of our drug candidates from regulatory authorities in any jurisdiction. We have only limited experience in conducting and managing the clinical trials, and in submitting and supporting the applications necessary to seek marketing approvals and expect to rely on third-party CROs to assist us in this process. Securing marketing approval requires the submission of extensive nonclinical and clinical data and supporting information to regulatory authorities for each therapeutic indication to establish the drug candidate's safety and efficacy. Securing marketing approval also requires the submission of information about the product manufacturing process to, and inspection of manufacturing facilities by, the regulatory authorities. Our drug candidates may not be effective, may be only moderately effective or may prove to have undesirable or unintended side effects, toxicities or other characteristics that may preclude our obtaining marketing approval or prevent or limit commercial use.

The process of obtaining marketing approvals, both in the United States and abroad, is expensive, may take many years if additional clinical trials are required, if approval is obtained at all, and can vary substantially based upon a variety of factors, including the type, complexity and novelty of the drug candidates involved. For example, we plan to initiate a pivotal Phase 3 trial for DCC-2618 in second-line GIST; however, FDA may require us to conduct a clinical trial in third-line GIST before we may conduct one in second-line GIST. In addition, while we plan to conduct only one pivotal Phase 3 trial for each indication of fourth-line GIST and second-line GIST, for a single randomized trial to support an NDA, the trial must be well-designed, well-conducted, with a favorable risk/benefit ratio, and provide statistically persuasive efficacy findings so compelling that a second trial would be unethical or practically impossible to repeat. Further, changes in marketing approval policies during the development period, changes in or the enactment of additional statutes or regulations, 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 drug candidate. Any marketing approval we ultimately obtain may be limited or subject to restrictions or post-approval commitments that render the approved product not commercially viable. If we experience delays in obtaining approval or if we fail to obtain approval of our drug candidates, the commercial prospects for our drug candidates may be harmed and our ability to generate revenues will be materially impaired.

We may not be able to obtain orphan drug exclusivity for our drug candidates.

Regulatory authorities in some jurisdictions, including the United States and Europe, may in response to a request from the sponsor designate drugs for relatively small patient populations as orphan drugs. Under the Orphan Drug Act, FDA may designate a product as an orphan drug if it is a drug intended to treat a rare disease or condition, which is generally defined as a patient population of fewer than 200,000 individuals annually in the

United States. For example, we have received orphan drug designation for DCC-2618 for the treatment of GIST and GBM in the United States.

Generally, if a product with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, the product is entitled to a period of marketing exclusivity, which precludes the approval of another marketing application for the same drug for the same indication for that time period. The applicable period is seven years in the United States and ten years in Europe. Orphan drug exclusivity may be lost if FDA or EMA determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the drug to meet the needs of patients with the rare disease or condition. In addition, FDA can subsequently approve the same drug for treatment of the same disease or condition if it concludes that the later drug is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care. FDA also can approve a different drug for the same orphan indication, or the same drug for a different indication, during the orphan exclusivity period.

Even if we obtain orphan drug exclusivity for a product's use in a specific indication, that exclusivity may not effectively protect the product from competition.

A fast track designation by FDA may not actually lead to a faster development or regulatory review or approval process.

We intend to seek fast track designation for some of our drug candidates. If a drug is intended for the treatment of a serious or life-threatening condition and the drug demonstrates the potential to address unmet medical needs for this condition, the drug sponsor may apply for FDA fast track designation. FDA has broad discretion whether or not to grant this designation, so even if we believe a particular drug candidate is eligible for this designation, we cannot assure you that FDA would decide to grant it. Even if we do receive fast track designation, we may not experience a faster development process, review or approval compared to conventional FDA procedures. FDA may rescind fast track designation if the drug candidate no longer meets the qualifying criteria for fast track designation, such as if the drug: (1) no longer demonstrates a potential to address unmet medical need or (2) is not being studied in a manner that shows the drug can treat a serious condition and meets an unmet medical need. A drug candidate may no longer demonstrate a potential to address unmet medical need if a new product was approved under a traditional approval that addressed the same need or if emerging clinical data failed to show that the fast track designated drug candidate had the anticipated advantage over available therapy.

A breakthrough therapy designation by FDA for our drug candidates may not lead to a faster development or regulatory review or approval process, and it does not increase the likelihood that our drug candidates will obtain marketing approval.

We may seek a breakthrough therapy designation for some of our drug candidates. A breakthrough therapy is defined as a drug that is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. For drugs and biologics that have been designated as breakthrough therapies, interaction and communication between FDA and the sponsor of the trial can help to identify the most efficient path for clinical development while minimizing the number of patients placed in ineffective control regimens. Drugs designated as breakthrough therapies by FDA are also eligible for accelerated approval.

Designation as a breakthrough therapy is within the discretion of FDA. Accordingly, even if we believe one of our drug candidates meets the criteria for designation as a breakthrough therapy, FDA may disagree and instead determine not to make such designation. In any event, the receipt of a breakthrough therapy designation for a drug candidate may not result in a faster development process, review or approval compared to drugs

considered for approval under conventional FDA procedures and does not assure ultimate approval by FDA. In addition, even if one or more of our drug candidates qualify as breakthrough therapies, FDA may later decide that the products no longer meet the conditions for qualification or decide that the time period for FDA review or approval will not be shortened.

Our failure to obtain marketing approval in foreign jurisdictions would prevent our drug candidates from being marketed abroad, and any approval we are granted for our drug candidates in the United States would not assure approval of drug candidates in foreign jurisdictions.

In order to market and sell our products in the European Union and many other jurisdictions, we 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 that required to obtain FDA approval. The regulatory approval process outside the United States generally includes all of the risks associated with obtaining FDA approval. In addition, in many countries outside the United States, it is required that the product be approved for reimbursement before the product can be approved for sale in that country. We may not obtain approvals from regulatory authorities outside the United States on a timely basis, if at all. Approval by 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 FDA. We may not be able to file for marketing approvals and may not receive necessary approvals to commercialize our products in any market.

Even if we obtain marketing approval for our drug candidates, the terms of approvals and ongoing regulation of our products may limit how we manufacture and market our products and compliance with such requirements may involve substantial resources, which could materially impair our ability to generate revenue.

Even if marketing approval of a drug candidate is granted, an approved product and its manufacturer and marketer are subject to ongoing review and extensive regulation, which may include the requirement to implement a risk evaluation and mitigation strategy or to conduct costly post-marketing studies or clinical trials and surveillance to monitor the safety or efficacy of the product. We must also comply with requirements concerning advertising and promotion for any of our drug candidates for which we 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 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 ensure 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 and our contract manufacturers could be subject to periodic unannounced inspections by FDA to monitor and ensure compliance with cGMPs.

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

Any drug candidate for which we obtain marketing approval will be subject to ongoing enforcement of post-marketing requirements and we could be subject to substantial penalties, including withdrawal of our product from the market, if we fail to comply with all regulatory requirements or if we experience unanticipated problems with our products, when and if any of them are approved.

Any drug candidate for which we obtain marketing approval, along with the manufacturing processes, post-approval clinical data, labeling, advertising and promotional activities for such product, will be subject to continual requirements of and review by FDA and other regulatory authorities. These requirements include, but are not limited to, restrictions governing promotion of an approved product, submissions of safety and other post-marketing information and reports, registration and listing requirements, cGMP requirements relating to manufacturing, quality control, quality assurance and corresponding maintenance of records and documents, and requirements regarding drug distribution and the distribution of samples to physicians and recordkeeping.

FDA and other federal and state agencies, including the Department of Justice, closely regulate compliance with all requirements governing prescription drug products, including requirements pertaining to marketing and promotion of drugs in accordance with the provisions of the approved labeling and manufacturing of products in accordance with cGMP requirements. Violations of such requirements may lead to investigations alleging violations of the Federal Food, Drug, and Cosmetic Act, or the FDCA, and other statutes, including the False Claims Act and other federal and state healthcare fraud and abuse laws as well as state consumer protection laws. Our failure to comply with all regulatory requirements, and later discovery of previously unknown adverse events or other problems with our products, manufacturers or manufacturing processes, may yield various results, including:

- litigation involving patients taking our products;
- restrictions on such products, manufacturers or manufacturing processes;
- 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 or untitled letters;
- withdrawal of the products from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- voluntary recall of products;
- fines, restitution or disgorgement of profits or revenues;
- suspension or withdrawal of marketing approvals;
- damage to relationships with any potential collaborators;
- unfavorable press coverage and damage to our reputation;
- refusal to permit the import or export of our products;
- product seizure; or
- injunctions or the imposition of civil or criminal penalties.

Non-compliance by us or any future collaborator with regulatory requirements, including safety monitoring or pharmacovigilance, and with requirements related to the development of products for the pediatric population can also result in significant financial penalties. Similarly, failure to comply with applicable regulatory requirements regarding the protection of personal information can also lead to significant penalties and sanctions.

Our 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 drug candidates for which we obtain marketing approval. Our future arrangements with third-party payors and customers may expose us 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 any products for which we obtain marketing approval. Restrictions under applicable federal and state healthcare laws and regulations, include the following:

- the federal 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 of, any good or service, for which payment may be made under a federal healthcare program such as Medicare and Medicaid;
- the federal False Claims laws impose criminal and civil penalties, including 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 government 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;
- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, imposes criminal and civil liability for executing a scheme to defraud any healthcare benefit program and 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 of 2010, or collectively the ACA, requires manufacturers of drugs, devices, biologics and medical supplies to report to the Department of Health and Human Services information related to physician payments and other transfers of value and physician ownership and investment interests; and
- analogous state laws and regulations such as state anti-kickback and false claims laws and analogous non-U.S. fraud and abuse laws and regulations, may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers.

In addition, some state laws require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance regulations promulgated by the federal government and may 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 non-U.S. 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.

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, which could have a material adverse effect on our business, results of operations, financial condition and prospects.

Recently enacted and future legislation may increase the difficulty and cost for us to obtain marketing approval of and commercialize our drug candidates and decrease the prices we 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 drug candidates, restrict or regulate post-approval activities and affect our ability to profitably sell any drug candidates for which we 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 applies only to 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, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010, or collectively the ACA, was enacted into law. The ACA is a sweeping law intended to broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against fraud and abuse, add new transparency requirements for the healthcare and health insurance industries, impose new taxes and fees on the health industry and impose additional health policy reforms.

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

- an annual, nondeductible fee on any entity that manufactures or imports specified branded prescription drugs and biologic products;
- 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;
- 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;
- requirements to report financial arrangements with physicians and teaching hospitals;
- a requirement to annually report drug samples that manufacturers and distributors provide to physicians; and

- a Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research.

Some of the provisions of the ACA have yet to be fully implemented, while certain provisions have been subject to reform through legislation and Executive Orders of the President of the United States of America, or the President, and to judicial challenges. In 2012, the U.S. Supreme Court heard challenges to the constitutionality of the individual mandate and the viability of certain provisions of the Healthcare Reform Act. The Supreme Court's decision upheld most of the Healthcare Reform Act and determined that requiring individuals to maintain "minimum essential" health insurance coverage or pay a penalty to the Internal Revenue Service was within Congress's constitutional taxing authority. However, as a result of tax reform legislation enacted into law in late December 2017, the individual mandate has been eliminated. The long ranging effects of the elimination of the individual mandate on the viability of the ACA are unknown at this time.

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

In addition, other legislative changes have been proposed and adopted since the ACA was enacted that, directly or indirectly, affect or are likely to affect, the pharmaceutical industry and the commercialization of our products, including the Budget Control Act of 2011, the American Taxpayer Relief Act of 2012 and the Middle Class Tax Relief and Job Creation Act of 2012. Further, each chamber of Congress put forth multiple bills in 2017 designed to repeal or repeal and replace portions of the ACA. In 2018, Congress may consider other legislation to repeal and replace elements of the ACA. Litigation and legislation over the ACA are likely to continue, with unpredictable and uncertain results. The full impact of the ACA, any law repealing and/or replacing elements of it, and the political uncertainty surrounding any repeal or replacement legislation on our business remains unclear.

We expect that the ACA, as well as other healthcare reform measures that may be adopted in the future, may result in more rigorous coverage criteria and in additional downward pressure on the price that we receive for any approved product. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability, or commercialize our products.

There has been heightened governmental scrutiny over the manner in which manufacturers set prices for their marketed products, which have resulted in several recent Congressional inquiries and proposed bills designed to, among other things, bring more transparency to product pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for products. In addition, the U.S. government, state legislatures, and foreign governments have shown significant interest in implementing cost containment programs, including price-controls, restrictions on reimbursement and requirements for substitution of generic products for branded prescription drugs to limit the growth of government paid healthcare costs. Individual states in the United States have also become increasingly aggressive in passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product

access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing.

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 FDA regulations, guidance or interpretations will be changed, or what the impact of such changes on the marketing approvals of our drug candidates, if any, may be. In addition, increased scrutiny by the U.S. Congress of FDA's approval process may significantly delay or prevent marketing approval, as well as subject us to more stringent product labeling and post-marketing testing and other requirements.

The effects of recently enacted tax legislation and other legislative, regulatory and administrative developments to our business are uncertain. Increased costs related to such developments could adversely affect our financial condition and results of operations.

On December 22, 2017, the President signed into law H.R. 1, informally titled the Tax Cuts and Jobs Act, or the TCJA. The TCJA makes major changes to the taxation of corporations, including reduction of the corporate tax rate from 35% to 21%, limitation of the tax deduction for interest expense to 30% of adjusted taxable income (except for certain small businesses), limitation of the deduction for net operating losses to 80% of annual taxable income and elimination of net operating loss carrybacks, one time taxation of offshore earnings at reduced rates regardless of whether they are repatriated, elimination of U.S. tax on foreign earnings (subject to certain important exceptions), immediate deductions for certain new investments instead of deductions for depreciation expense over time, and modifying or repealing many business deductions and credits. The effect of the significant changes made by the TCJA is highly uncertain, and administrative guidance will be required in order to fully evaluate the effect of many provisions on our business and stockholders.

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

Under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, if a corporation undergoes an "ownership change" (generally defined as a greater than 50% change (by value) in its equity ownership over a three-year period), the corporation's ability to use its pre-change net operating loss carryforwards and other pre-change tax attributes to offset its post-change taxable income may be limited. We may experience ownership changes in the future as a result of subsequent shifts in our stock ownership. As of December 31, 2017, we had U.S. federal net operating loss carryforwards and U.S. federal research and development tax credit carryforwards, which could be limited if we experience an "ownership change." The reduction of the corporate tax rate under the TCJA may cause a reduction in the economic benefit of our net operating loss carryforwards and other deferred tax assets available to us. Under the TCJA, net operating losses generated after December 31, 2017 will not be subject to expiration.

Governments outside of the United States tend to impose strict price controls, which may adversely affect our revenues, if any.

In some countries, particularly Canada and 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 may be required to conduct a clinical trial that compares the cost-effectiveness of our drug candidate 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 harmed. In addition, the recent United Kingdom referendum on its membership in the European Union resulted in a majority of United Kingdom voters voting to exit the European Union, often referred to as Brexit. Brexit could lead to legal uncertainty and potentially divergent national laws and regulations, including those related to the pricing of prescription pharmaceuticals, as the United Kingdom determines which EU laws to replicate or replace. If the United Kingdom were to significantly alter its regulations affecting the pricing of

prescription pharmaceuticals, we could face significant new costs. As a result, Brexit could impair our ability to transact business in the European Union and the United Kingdom.

Laws and regulations governing any international operations we may have in the future may preclude us from developing, manufacturing and selling certain drug candidates and products outside of the United States and require us to develop and implement costly compliance programs.

If we expand our operations outside of the United States, we must dedicate additional resources to comply with numerous laws and regulations in each jurisdiction in which we plan to operate. The Foreign Corrupt Practices Act, or FCPA, prohibits any U.S. individual or business from paying, offering, authorizing payment or offering anything of value, directly or indirectly, to any foreign official, political party or candidate for the purpose of influencing any act or decision of such third party in order to assist the individual or business in obtaining or retaining business. The FCPA also obligates companies whose securities are listed in the United States to comply with certain accounting provisions requiring the company to maintain books and records that accurately and fairly reflect all transactions of the company, including international subsidiaries, and to devise and maintain an adequate system of internal accounting controls for international operations.

Compliance with the FCPA is expensive and difficult, particularly in countries in which corruption is a recognized problem. In addition, the FCPA presents particular challenges in the pharmaceutical industry, because, in many countries, hospitals are operated by the government, and doctors and other hospital employees are considered foreign officials. Certain payments to hospitals in connection with clinical trials and other work have been deemed to be improper payments to government officials and have led to FCPA enforcement actions.

Various laws, regulations and executive orders also restrict the use and dissemination outside of the United States, or the sharing with certain non-U.S. nationals, of information classified for national security purposes, as well as certain products and technical data relating to those products. If we expand our presence outside of the United States, it will require us to dedicate additional resources to comply with these laws, and these laws may preclude us from developing, manufacturing or selling certain drug candidates and products outside of the United States, which could limit our growth potential and increase our development costs.

The failure to comply with laws governing international business practices may result in substantial civil and criminal penalties and suspension or debarment from government contracting. The Securities and Exchange Commission also may suspend or bar issuers from trading securities on U.S. exchanges for violations of the FCPA's accounting provisions.

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 harm our business.

We are subject to numerous foreign, federal, state and local 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. Our operations involve the use of hazardous and flammable materials, including chemicals and biological materials. Our operations also produce hazardous waste products. We generally contract with third parties for the disposal of these materials and wastes. We cannot eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting from our use of hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources, including any available insurance.

In addition, our leasing and operation of real property may subject us to liability pursuant to certain of these laws or regulations. Under existing U.S. environmental laws and regulations, current or previous owners or operators of real property and entities that disposed or arranged for the disposal of hazardous substances may be held strictly, jointly and severally liable for the cost of investigating or remediating contamination caused by hazardous substance releases, even if they did not know of and were not responsible for the releases.

We could incur significant costs and liabilities which may adversely affect our financial condition and operating results for failure to comply with such laws and regulations, including, among other things, civil or criminal fines and penalties, property damage and personal injury claims, costs associated with upgrades to our facilities or changes to our operating procedures, or injunctions limiting or altering our operations.

Although we maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials, 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 connection with our storage or disposal of biological, hazardous or radioactive materials.

In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. These current or future laws and regulations, which are becoming increasingly more stringent, may impair our research, development or production efforts. Our failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions.

Risks Related to Employee Matters and Managing Growth

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

We are highly dependent on the research, development and management expertise of Michael D. Taylor, Ph.D., our President and Chief Executive Officer, and the research expertise on kinase switch control inhibition of Daniel L. Flynn, Ph.D., our founder and Chief Scientific Officer, as well as the other principal members of our management, scientific and clinical team. Although we have entered into employment agreements with our executive officers, each of them may terminate their employment with us at any time. We maintain "key person" insurance for Dr. Flynn, but not for any of our other executives or employees.

Recruiting and retaining qualified scientific, clinical, manufacturing and sales and marketing personnel will also be critical to our success. The loss of the services of our executive officers or other key employees could impede the achievement of our research, development and commercialization objectives and seriously harm our ability to successfully implement our business strategy. Furthermore, replacing executive officers and key employees 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 successfully develop, gain marketing approval of and commercialize products. Competition to hire from this limited pool is intense, and we may be unable to hire, train, retain or motivate these 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 pursue our growth strategy will be limited.

We expect to expand our development and regulatory capabilities and potentially implement sales, marketing and distribution capabilities, and as a result, we may encounter difficulties in managing our growth, which could disrupt our operations.

We expect to experience significant growth in the number of our employees and the scope of our operations, particularly in the areas of drug development, manufacturing, regulatory affairs and, if any of our drug candidates receives marketing approval, sales, marketing and distribution. To manage our anticipated future growth, 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 and with developing sales, marketing and distribution infrastructure, we may not be able to effectively manage the expansion of our operations or recruit and train additional qualified personnel. The expansion of our operations may lead to significant costs and may divert our management and business development resources. Any inability to manage growth could delay or prevent the execution of our business plans or disrupt our operations.

We rely significantly on information technology and any failure, inadequacy, interruption or security lapse of that technology, including any cyber security incidents, could harm our ability to operate our business effectively.

Despite the implementation of security measures, our internal computer systems and those of third parties with which we contract are vulnerable to damage from cyber-attacks, computer viruses, worms and other destructive or disruptive software, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. System failures, accidents or security breaches could cause interruptions in our operations, and could result in a material disruption of our clinical and commercialization activities and business operations, in addition to possibly requiring substantial expenditures of resources to remedy. The loss of clinical trial data could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability and our product research, development and commercialization efforts could be delayed. In addition, we may not have adequate insurance coverage to provide compensation for any losses associated with such events.

We could be subject to risks caused by misappropriation, misuse, leakage, falsification or intentional or accidental release or loss of information maintained in the information systems and networks of our company, including personal information of our employees. In addition, outside parties may attempt to penetrate our systems or those of our vendors or fraudulently induce our employees or employees of our vendors to disclose sensitive information in order to gain access to our data. Like other companies, we have on occasion, and will continue to experience, threats to our data and systems, including malicious codes and viruses, and other cyber-attacks. The number and complexity of these threats continue to increase over time. If a material breach of our security or that of our vendors occurs, the market perception of the effectiveness of our security measures could be harmed, we could lose business and our reputation and credibility could be damaged. We could be required to expend significant amounts of money and other resources to repair or replace information systems or networks. Although we develop and maintain systems and controls designed to prevent these events from occurring, and we have a process to identify and mitigate threats, the development and maintenance of these systems, controls and processes is costly and requires ongoing monitoring and updating as technologies change and efforts to overcome security measures become more sophisticated. Moreover, despite our efforts, the possibility of these events occurring cannot be eliminated entirely.

We or the third parties upon whom we depend may be adversely affected by natural disasters and our business continuity and disaster recovery plans may not adequately protect us from a serious disaster.

Natural disasters could severely disrupt our operations, and have a material adverse effect on our business, results of operations, financial condition and prospects. If a natural disaster, power outage or other event occurred that prevented us from using all or a significant portion of our headquarters, that damaged critical infrastructure, such as the manufacturing facilities of our third-party contract manufacturers, or that otherwise disrupted operations, it may be difficult or, in certain cases, impossible for us to continue our business for a substantial period of time. The disaster recovery and business continuity plans we have in place may prove inadequate in the event of a serious disaster or similar event. We may incur substantial expenses as a result of the limited nature of our disaster recovery and business continuity plans, which, could have a material adverse effect on our business.

We may engage in strategic transactions that could impact our liquidity, increase our expenses and present significant distractions to our management.

From time to time, we may consider strategic transactions, such as acquisitions of companies, businesses or assets and out-licensing or in-licensing of products, drug candidates or technologies. Additional potential transactions that we may consider include a variety of different business arrangements, including spin-offs, strategic partnerships, joint ventures, restructurings, divestitures, business combinations and investments. Any such transaction may require us to incur non-recurring or other charges, may increase our near term or long-term expenditures and may pose significant integration challenges or disrupt our management or business, which could adversely affect our operations and financial results. For example, these transactions may entail numerous operational and financial risks, including:

- exposure to unknown liabilities;
- disruption of our business and diversion of our management's time and attention in order to develop acquired products, drug candidates or technologies;
- incurrence of substantial debt or dilutive issuances of equity securities to pay for acquisitions;
- higher than expected acquisition and integration costs;
- write-downs of assets or goodwill or impairment charges;
- increased amortization expenses;
- difficulty and cost in combining the operations, systems and personnel of any acquired businesses with our operations, systems and personnel;
- impairment of relationships with key suppliers or customers of any acquired businesses due to changes in management and ownership; and
- inability to retain key employees of any acquired businesses.

Risks Related to Our Common Stock

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

As of December 31, 2017, our executive officers and directors, combined with our stockholders who own more than 5% of our outstanding capital stock, beneficially own shares, in the aggregate, representing approximately 78% of our capital stock. As a result, if these stockholders were to choose to act together, they would be able to control 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 control 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 and the board of directors; or
- impede a merger, consolidation, takeover or other business combination involving us that other stockholders may desire.

Provisions in our corporate charter documents, under Delaware law and in certain of our contractual agreements could make an acquisition of our company, 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 amended and restated certificate of incorporation and our amended and restated bylaws may discourage, delay or prevent a merger, acquisition or other change in control of our company that

stockholders may consider favorable, including transactions in which you might otherwise receive a premium for your 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 only one of three classes of directors is elected each year;
- 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 our board of directors;
- establish advance notice requirements for stockholder proposals that can be acted on at stockholder meetings and nominations to our board of directors;
- 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 stockholder meetings;
- 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 specified 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.

We are also party to a loan agreement and a security agreement that includes covenants such as limitations on our ability to engage in certain fundamental business transactions, such as mergers or acquisitions of other businesses.

Our amended and restated bylaws designate the Court of Chancery of the State of Delaware as the sole and exclusive forum for certain types of actions and proceedings that may be initiated by our stockholders, which could limit our stockholders’ ability to obtain a favorable judicial forum for disputes with us or our directors, officers or employees.

Our amended and restated bylaws provide that, unless we consent in writing to an alternative forum, the Court of Chancery of the State of Delaware will be the sole and exclusive forum for (i) any derivative action or proceeding brought on our behalf, (ii) any action asserting a claim of breach of a fiduciary duty owed by any of our directors, officers and employees to us or our stockholders, (iii) any action asserting a claim arising pursuant to any provision of the Delaware General Corporation Law, our amended and restated certificate of incorporation or our amended and restated bylaws, or (iv) any action asserting a claim that is governed by the internal affairs doctrine, in each case subject to the Court of Chancery having personal jurisdiction over the indispensable parties named as defendants therein. Any person purchasing or otherwise acquiring any interest in any shares of our capital stock shall be deemed to have notice of and to have consented to this provision of our amended and restated bylaws. This choice of forum provision may limit a stockholder’s ability to bring a claim in a judicial forum that it finds favorable for disputes with us or our directors, officers or employees, which may discourage

such lawsuits against us and our directors, officers and employees even though an action, if successful, might benefit our stockholders. Stockholders who do bring a claim in the Court of Chancery could face additional litigation costs in pursuing any such claim, particularly if they do not reside in or near the State of Delaware. The Court of Chancery may also reach different judgments or results than would other courts, including courts where a stockholder considering an action may be located or would otherwise choose to bring the action, and such judgments or results may be more favorable to us than to our stockholders. Alternatively, if a court were to find this provision of our amended and restated bylaws inapplicable to, or unenforceable in respect of, one or more of the specified types of actions or proceedings, we may incur additional costs, which could have a material adverse effect on our business, financial condition or results of operations.

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

Our shares of common stock began trading on The NASDAQ Global Select Market on September 28, 2017. Given the limited trading history of our common stock, there is a risk that an active trading market for our shares will not be sustained, which could put downward pressure on the market price of our common stock and thereby affect the ability of our stockholders to sell their shares.

Future sales of common stock by stockholders may have an adverse effect on the then prevailing market price of our common stock.

In the event a public market for our common stock is sustained in the future, sales of our common stock may be made by holders of our public float or by holders of restricted securities in compliance with the provisions of Rule 144 of the Securities Act of 1933, or the Securities Act. In general, under Rule 144, a non-affiliated person who has satisfied a six-month holding period in a company registered under the Exchange Act, as amended, may, sell their restricted common stock without volume limitation, so long as the issuer is current with all reports under the Exchange Act in order for there to be adequate common public information. Affiliated persons may also sell their common shares held for at least six months, but affiliated persons will be required to meet certain other requirements, including manner of sale, notice requirements and volume limitations. Non-affiliated persons who hold their common shares for at least one year will be able to sell their common stock without the need for there to be current public information in the hands of the public. Future sales of shares of our public float or by restricted common stock made in compliance with Rule 144 may have an adverse effect on the then prevailing market price, if any, of our common stock.

The market prices for our common stock may be adversely impacted by future events.

Our common stock is currently quoted on The NASDAQ Global Select Market under the symbol “DCPH.” Market prices for our common stock will be influenced by a number of factors, including:

- the issuance of new equity securities pursuant to a future offering, including issuances of preferred stock;
- changes in interest rates;
- significant dilution caused by the anti-dilutive clauses in our financial agreements;
- competitive developments, including announcements by competitors of new products or services or significant contracts, acquisitions, strategic partnerships, joint ventures or capital commitments;
- variations in quarterly operating results;
- change in financial estimates by securities analysts;
- the depth and liquidity of the market for our common stock;
- investor perceptions of our company and the pharmaceutical and biotech industries generally; and
- general economic and other national conditions.

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

Our stock price is likely to be volatile. The stock market in general and the market for smaller biopharmaceutical companies in particular have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. Since our common stock began trading on The NASDAQ Global Select Market on September 28, 2017, our stock has traded at prices as low as $15.15 per share and as high as $29.98 per share through February 28, 2018. The market price for our common stock may be influenced by many factors, including:

- the degree of success of competitive products or technologies;
- results of clinical trials and preclinical studies, of our drug candidates or those of our competitors;
- regulatory or legal developments in the United States and other countries;
- receipt of, or failure to obtain, regulatory approvals;
- developments or disputes concerning patent applications, issued patents or other proprietary rights;
- the recruitment or departure of key personnel;
- the level of expenses related to any of our drug candidates or clinical development programs;
- the results of our efforts to discover, develop, acquire or in-license additional technologies or drug candidates;
- actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;
- variations in our financial results or those of companies that are perceived to be similar to us;
- rumors or announcements regarding transactions involving our company or drug candidates;
- changes in the structure of healthcare payment systems;
- market conditions in the pharmaceutical and biotechnology sectors;
- general economic, industry and market conditions; and
- the other factors described in this “Risk Factors” section.

We could be subject to securities class action litigation.

In the past, securities class action litigation has often been brought against a company following a decline in the market price of its securities. This risk is especially relevant for us because pharmaceutical companies have experienced significant stock price volatility in recent years. If we face such litigation, it could result in substantial costs and a diversion of management’s attention and resources, which could harm our business.

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 Jumpstart Our Business Startups Act of 2012, or the JOBS Act, and may remain an emerging growth company for up to five years. For so long as we remain an emerging growth company, we are permitted and intend to rely on exemptions from certain disclosure requirements that are applicable to other public companies that are not emerging growth companies. These exemptions include:

- being permitted to provide only two years of audited financial statements, in addition to any required unaudited interim financial statements, with correspondingly reduced “Management’s Discussion and Analysis of Financial Condition and Results of Operations” disclosure;

- not being required to comply with the auditor attestation requirements in the assessment of our internal control over financial reporting;
- not being required to comply with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding 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 non-binding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved.

We have taken advantage of some, but not all, of the available exemptions. 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 these accounting standards until they would otherwise apply to private companies. We have irrevocably elected not to avail ourselves of this exemption and, therefore, we will be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies.

We will 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, and particularly after we are no longer an emerging growth company, we will incur significant legal, accounting and other expenses that we did not incur as a private company. The Sarbanes-Oxley Act of 2002, the Dodd-Frank Wall Street Reform and Consumer Protection Act, the listing requirements of The NASDAQ Global Select 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. Our management and other personnel will need to devote a substantial amount of time to these compliance initiatives. Moreover, these rules and regulations will increase our legal and financial compliance costs and will make some activities more time-consuming and costly. For example, these rules and regulations may make it more difficult and more expensive for us to obtain director and officer liability insurance, which in turn could make it more difficult for us to attract and retain qualified members of our board of directors.

We are evaluating these rules and regulations, and cannot predict or estimate the amount of additional costs we may incur or the timing of such costs. These rules and regulations are often subject to varying 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 Section 404 of the Sarbanes-Oxley Act of 2002, or Section 404, we are required to furnish a report by our management on our internal control over financial reporting. 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. To achieve compliance with Section 404 within the prescribed period, we will be engaged in a process to document and evaluate our internal control over financial reporting, which is both costly and challenging. In this regard, 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 we will not be able to conclude, within the prescribed timeframe or at all, that our internal control over financial reporting is effective as required by 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.

Because we do not anticipate paying any cash dividends on our capital stock in the foreseeable future, capital appreciation, if any, will be your sole source of gain.

We have never declared or paid 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. In addition, the terms of any future debt agreements we may enter into may preclude us from paying dividends. As a result, capital appreciation, if any, of our common stock will be your sole source of gain for the foreseeable future.

ITEM 1B. UNRESOLVED STAFF COMMENTS

None.

ITEM 2. PROPERTIES

We currently lease two facilities. Our headquarters are located in 6,883 rentable square feet of office space in Waltham, Massachusetts that is used primarily for our clinical research, regulatory, business development and administrative functions. We also lease 25,524 square feet of laboratory, office and storage space in Lawrence, Kansas that is used primarily for discovery research, preclinical research, and non-clinical functions. We believe that our existing facilities are adequate for our current needs. We expect to seek additional office space for our Massachusetts operations as we increase our staffing levels as a result of anticipated growth in our clinical and regulatory activities as well as additional financial and administrative resources required to support operations as a public company. We believe that any additional space we may require will be available on commercially reasonable terms.

ITEM 3. LEGAL PROCEEDINGS

We are not currently a party to any material legal proceedings.

ITEM 4. MINE SAFETY DISCLOSURES

Not applicable.

PART II

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

Certain Information Regarding the Trading of Our Common Stock

Our common stock trades under the symbol "DCPH" on the NASDAQ Global Select Market and has been publicly traded since September 28, 2017. Prior to this time, there was no public market for our common stock. The following table sets forth the high and low sales price of our common stock as reported on the NASDAQ Global Select Market for the periods indicated:

	High	Low
Year ended December 31, 2017:		
Third Quarter (beginning September 28, 2017)	$20.53	$16.11
Fourth Quarter	$24.50	$15.15

On March 23, 2018, the last reported sale price for our common stock on the NASDAQ Global Select Market was $21.18 per share.

Holders of Our Common Stock

As of February 28, 2018, there were approximately 14 holders of record of shares of our common stock. This number does not include stockholders for whom shares are held in "nominee" or "street" name.

Dividends

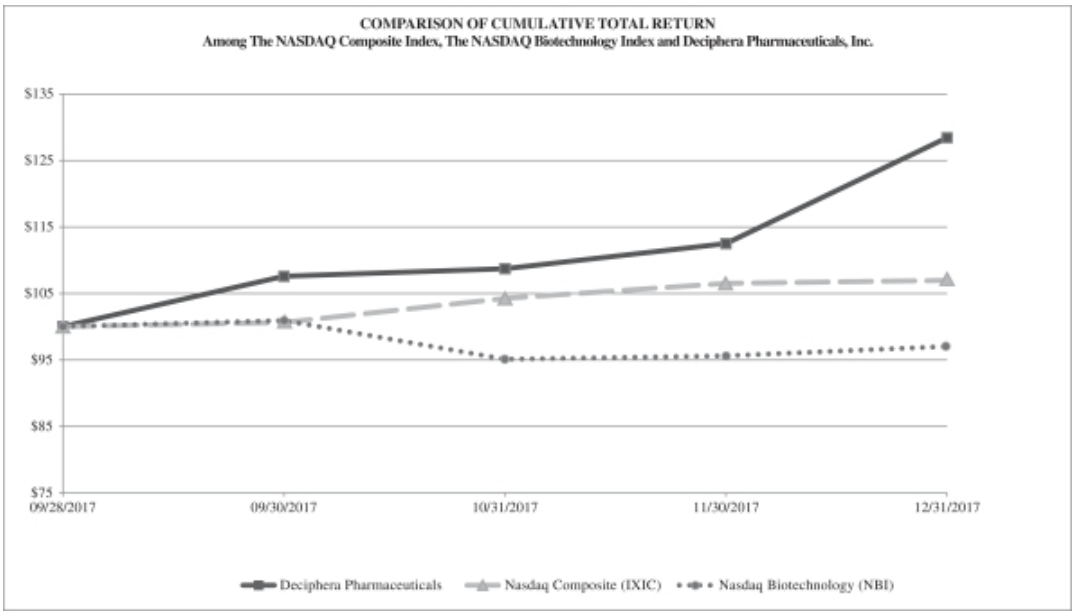
We have never declared or paid any cash dividends on our capital stock. We currently intend to retain all available funds and any future earnings for use in the operation of our business and do not anticipate paying any dividends on our common stock in the foreseeable future. Any future determination to declare dividends will be made at the discretion of our board of directors and will depend on our financial condition, operating results, capital requirements, general business conditions and other factors that our board of directors may deem relevant.

Stock 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 Exchange Act, nor shall such information be incorporated by reference into any future filing under the Exchange Act or 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 September 28, 2017 (the first date that shares of our common stock were publicly traded) through December 31, 2017. The comparison assumes $100 was invested in our common stock and in each of the foregoing indices after the market closed on September 28, 2017, 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.



Securities authorized for issuance under equity compensation plans

Information about our equity compensation plans will be included in our definitive proxy statement to be filed with the SEC with respect to our 2018 Annual Meeting of Stockholders and is incorporated herein by reference.

Unregistered Sales of Equity Securities and Use of Proceeds

Recent Sales of Unregistered Equity Securities

During the period covered by this Annual Report on Form 10-K, we have issued the following securities that were not registered under the Securities Act:

- Between January 1, 2017 and December 31, 2017, we issued to certain of our employees and directors, options to purchase an aggregate of 1,553,639 shares of our common stock at a weighted-average exercise price of $10.02 per share.
- On May 26, 2017, we issued and sold to seven accredited investors an aggregate of 690,333 series C preferred shares at a price per share of $75.76 for aggregate cash consideration of $52.3 million.
- On October 2, 2017, we issued to direct and indirect equity holders of Deciphera Pharmaceuticals, LLC an aggregate of 24,425,190 shares of our common stock in exchange for, directly and indirectly, all of the outstanding preferred shares of Deciphera Pharmaceuticals, LLC (the "Exchange"). The Exchange was part of a series of transactions pursuant to which Deciphera Pharmaceuticals, LLC became our wholly owned subsidiary.

We deemed the issuances in paragraphs (1) and (2) above to be exempt from registration under the Securities Act either in reliance on Rule 701 of the Securities Act as sales and offers under compensatory benefit plans and contracts relating to compensation in compliance with Rule 701, or in reliance on Section 4(a)(2), as

transactions by an issuer not involving a public offering. We deemed the issuances in paragraphs (3) and (4) above to be exempt from registration under the Securities Act, in accordance with Section 4(a)(2) of the Securities Act. Each of the recipients of securities either received adequate information about our company or had access, through employment or other relationships, to such information. No underwriters were involved in the foregoing issuances of securities.

Use of Proceeds from Initial Public Offering

On October 2, 2017, we completed the initial public offering of our common stock pursuant to which we issued and sold 7,500,000 shares of our common stock at a price to the public of $17.00 per share. In addition, on October 4, 2017, we issued and sold an additional 666,496 shares of common stock at the initial public offering price of $17.00 per share as a result of the partial exercise by the underwriters of their option to purchase additional shares of common stock.

The offer and sale of all of the shares in our initial public offering were registered under the Securities Act pursuant to a registration statement on Form S-1 (File No. 333-220299), which was declared effective by the SEC on September 27, 2017, and a registration statement on Form S-1MEF (File No. 333-220681), which was automatically effective upon filing with the SEC on September 27, 2017. Following the sale of the shares in connection with the closing of our initial public offering, the offering terminated. J.P. Morgan Securities LLC and Piper Jaffray & Co. acted as joint book-running managers, JMP Securities LLC as lead manager and Nomura Securities International, Inc. as co-manager of our initial public offering.

We received aggregate gross proceeds from our initial public offering of $138.8 million, or aggregate net proceeds of approximately $124.6 million after deducting underwriting discounts and commissions and offering expenses. None of the underwriting discounts and commissions or offering expenses were incurred or paid, directly or indirectly, to directors or officers of ours or their associates or to persons owning 10 percent or more of our common stock or to any of our affiliates, and we have not used any of the net proceeds from the offering to make payments, directly or indirectly, to any such persons. We have invested the net proceeds from the offering in money market accounts. There has been no material change in our planned use of the net proceeds from the offering as described in our final prospectus filed pursuant to Rule 424(b)(4) under the Securities Act with the SEC on September 28, 2017.

As of December 31, 2017, we have not used the net proceeds from our initial public offering.

Purchases of Equity Securities by the Issuer and Affiliated Purchasers

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

ITEM 6. SELECTED FINANCIAL DATA

You should read the following selected financial data together with our consolidated financial statements and the related notes appearing at the end of this Annual Report on Form 10-K and the "Management's Discussion and Analysis of Financial Condition and Results of Operations" section. We have derived the statement of operations data for the years ended December 31, 2017, 2016 and 2015 and the balance sheet data as of December 31, 2017 and 2016 from our audited consolidated financial statements appearing at the end of this Annual Report on Form 10-K. The balance sheet data as of December 31, 2015 is derived from our audited consolidated financial statements not included in this Annual Report on Form 10-K. Our historical results are not necessarily indicative of the results that may be expected in any future period.

	Year Ended December 31,		
	2017	2016	2015
	(in thousands, except share and per share data)		
Statement of Operations Data:			
Revenue	$ —	$ —	$ —
Operating expenses:			
Research and development(1)	39,514	20,163	12,475
General and administrative(1)	11,421	5,675	5,135
Total operating expenses	50,935	25,838	17,610
Loss from operations	(50,935)	(25,838)	(17,610)
Other income (expense):			
Interest expense	(95)	(106)	(2,209)
Interest and other income, net	746	4	3
Total other income (expense), net	651	(102)	(2,206)
Net loss and comprehensive loss	$ (50,284)	$ (25,940)	$ (19,816)
Net loss per share —basic and diluted	$ (2.99)	$ (2.23)	$ (4.67)
Weighted average common shares outstanding—basic and diluted(2)	16,792,179	11,626,287	4,245,698

(1) Amounts include stock-based compensation expense, as follows:

	Year Ended December 31,		
	2017	2016	2015
	(in thousands)		
Research and development	$1,320	$ 541	$1,382
General and administrative	3,546	946	1,175
	$4,866	$1,487	$2,557

(2) We did not have any common shares outstanding during the years ended December 31, 2015 and 2016 or for the period from January 1, 2017 through the closing of our initial public offering on October 2, 2017. To determine the weighted average shares outstanding for purposes of calculating net loss per share during those periods, we used the weighted average number of Series A convertible preferred shares outstanding because such shares represented the most subordinated share class outstanding during those periods. Share amounts for periods prior to the IPO have been retrospectively adjusted to give effect to the exchange of Series A convertible preferred shares into shares of common stock upon the Conversion (see Note 1 to our consolidated financial statements and the related notes appearing at the end of this Annual Report on Form 10-K).

	As of December 31,		
	2017	2016	2015
		(in thousands)	
Balance Sheet Data:			
Cash and cash equivalents	$196,754	$ 57,461	$ 25,777
Working capital	184,367	53,695	23,333
Total assets	199,095	58,945	26,790
Notes payable to related party, including current portion	1,481	1,668	1,866
Convertible preferred shares	—	192,667	137,368
Total stockholders' equity/members' deficit	183,973	(139,760)	(115,307)

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

The following discussion and analysis of our financial condition and results of operations should be read in conjunction with our consolidated financial statements and related notes appearing at the end of this Annual Report on Form 10-K. Some of the information contained in this discussion and analysis or set forth elsewhere in this Annual Report on Form 10-K, including information with respect to our plans and strategy for our business, includes forward looking statements that involve risks and uncertainties. As a result of many factors, including those factors set forth in the "Risk Factors" section of this Annual Report on Form 10-K, our actual results could 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 developing new drugs to improve the lives of cancer patients by addressing key mechanisms of drug resistance that limit the rate and durability of response of many cancer therapies. Our targeted, small molecule drug candidates, designed using our proprietary kinase switch control inhibitor platform, inhibit the activation of kinases, an important family of enzymes, that, when mutated or over expressed, are known to be directly involved in the growth and spread of many cancers. We have built a diverse pipeline of wholly owned, orally administered drug candidates that include three clinical-stage and two research-stage programs.

Since our inception in 2003, we have focused substantially all of our efforts and financial resources on organizing and staffing our company, business planning, raising capital, developing product and technology rights, and conducting research and development activities for our drug candidates. We do not have any products approved for sale and have not generated any revenue from product sales.

On October 2, 2017, we completed an initial public offering, or IPO, of our common stock, pursuant to which we issued and sold 7,500,000 shares of common stock at a public offering price of $17.00 per share, resulting in net proceeds of $114.1 million after deducting underwriting discounts and commissions and other offering expenses. On October 4, 2017, we issued and sold an additional 666,496 shares of our common stock at the IPO price of $17.00 per share, pursuant to the underwriters' partial exercise of their option to purchase additional shares of common stock, resulting in additional net proceeds of $10.5 million after deducting discounts and commissions. Prior to our IPO, we had funded our operations primarily with proceeds from the sales of preferred shares, proceeds from the issuance of convertible notes, payments received under a concluded collaboration agreement, borrowings under a construction loan and research and development grants from the Kansas Bioscience Authority, or KBA.

Since our inception, we have incurred significant operating losses. Our ability to generate product revenue sufficient to achieve profitability will depend heavily on the successful development and eventual commercialization of one or more of our drug candidates. Our net loss was $50.3 million for the year ended December 31, 2017, $25.9 million for the year ended December 31, 2016 and $19.8 million for the year ended December 31, 2015. As of December 31, 2017, we had an accumulated deficit of $195.9 million. We expect to continue to incur significant expenses and increasing operating losses for at least the next several years. We expect that our expenses and capital requirements will increase substantially in connection with our ongoing activities, particularly as we:

- continue enrollment in and proceed with the expansion cohorts of our Phase 1 clinical trial for DCC-2618;
- continue with our ongoing and planned pivotal Phase 3 clinical trials of DCC-2618;
- advance our planned clinical programs for DCC-3014 and rebastinib;

- develop any other future drug candidates we may choose to pursue;
- continue research and development and drug discovery and initiate additional clinical trials;
- seek marketing approval for any of our drug candidates that successfully complete clinical development;
- develop and scale up our capabilities to support our ongoing preclinical activities and clinical trials for our drug candidates and commercialization of any of our drug candidates for which we obtain marketing approval;
- maintain, expand, protect and enforce our intellectual property portfolio;
- develop and expand our sales, marketing and distribution capabilities for our drug candidates for which we obtain marketing approval, if any; and
- expand our operational, financial and management systems and increase personnel, including to support our clinical development and commercialization efforts and our operations as a public company.

We will not generate revenue from product sales unless and until we successfully complete clinical development and obtain regulatory approval for our drug candidates. If we obtain regulatory approval for any of our drug candidates and do not enter into a commercialization partnership, we expect to incur significant expenses related to developing our internal commercialization capability to support product sales, marketing and distribution. Further, we expect to incur additional costs associated with operating as a public company.

As a result, we will need substantial additional funding to support our continuing operations and pursue our growth strategy. Until such time as we can generate significant revenue from product sales, if ever, we expect to finance our operations through a combination of equity offerings, debt financings, collaborations, strategic alliances and marketing, distribution or licensing arrangements. We may be unable to raise additional funds or enter into such other agreements or arrangements when needed on favorable terms, or at all. If we fail to raise capital or enter into such agreements as, and when, needed, we may have to significantly delay, scale back or discontinue the development and commercialization of one or more of our drug candidates.

Because of the numerous risks and uncertainties associated with pharmaceutical product development, we are unable to accurately predict the timing or amount of increased expenses or when or if we will be able to achieve or maintain profitability. Even if we are able to generate product sales, we may not become profitable. If we fail to become profitable or are unable to sustain profitability on a continuing basis, then we may be unable to continue our operations at planned levels and be forced to reduce or terminate our operations.

As of December 31, 2017, we had cash and cash equivalents of $196.8 million. We believe that our existing cash and cash equivalents, will enable us to fund our operating expenses, capital expenditure requirements and debt service payments into the second half of 2019. See “Liquidity and Capital Resources.”

The Conversion

On October 2, 2017, immediately prior to the completion of our IPO, we engaged in a series of transactions whereby Deciphera Pharmaceuticals, LLC became a wholly owned subsidiary of Deciphera Pharmaceuticals, Inc., a newly formed Delaware corporation. As part of the transactions, shareholders of Deciphera Pharmaceuticals, LLC exchanged their shares of Deciphera Pharmaceuticals, LLC for shares of Deciphera Pharmaceuticals, Inc. on a one-for-5.65 basis and exchanged their outstanding equity incentive awards of Deciphera Pharmaceuticals, LLC for options to purchase the same number of shares of common stock of Deciphera Pharmaceuticals, Inc. multiplied by 5.65, with a corresponding adjustment to divide the exercise price by 5.65. We refer to these transactions as the Conversion.

Components of Our Results of Operations

Revenue

To date, we have not generated any revenue from product sales and do not expect to generate any revenue from the sale of products in the foreseeable future. If our development efforts for our drug candidates are successful and result in regulatory approval, we may generate revenue in the future from product sales. If we enter into license or collaboration agreements for any of our drug candidates or intellectual property, we may generate revenue in the future from payments as a result of such license or collaboration agreements. We cannot predict if, when, or to what extent we will generate revenue from the commercialization and sale of our drug candidates. We may never succeed in obtaining regulatory approval for any of our drug candidates.

Operating Expenses

Research and Development Expenses

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

- employee-related expenses, including salaries, related benefits, travel and stock-based compensation expense for employees engaged in research and development functions;
- expenses incurred in connection with the preclinical and clinical development of our drug candidates, including under agreements with contract research organizations, or CROs;
- the cost of consultants and contract manufacturing organizations, or CMOs, that manufacture drug products for use in our preclinical studies and clinical trials; and
- facilities, depreciation and other expenses, which include direct and allocated expenses for rent and maintenance of facilities, insurance and supplies.

We expense research and development costs to operations as incurred. Advance payments for goods or services to be received in the future for use in research and development activities are recorded as prepaid expenses. The prepaid amounts are expensed as the related goods are delivered or the services are performed.

Our direct research and development expenses are tracked on a program-by-program basis and consist primarily of external costs, such as fees paid to consultants, central laboratories, contractors, CMOs and CROs in connection with our preclinical and clinical development activities. We do not allocate employee costs, costs associated with our proprietary kinase switch control inhibitor platform technology or facility expenses, including depreciation or other indirect costs, to specific product development programs because these costs are deployed across multiple product development programs and, as such, are not separately classified.

The table below summarizes our research and development expenses incurred by development program:

	Year Ended December 31,		
	2017	2016	2015
	(in thousands)		
DCC-2618	$22,241	$ 6,791	$ 3,309
DCC-3014	2,550	2,766	1,043
Rebastinib	357	1,019	571
Discontinued program	775	3,584	3,307
Unallocated expenses	13,591	6,003	4,245
Total research and development expenses	$39,514	$20,163	$12,475

Drug candidates in later stages of clinical development generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of later-stage clinical

trials. We expect that our research and development expenses will increase substantially in connection with our planned clinical and preclinical development activities in the near term and in the future. At this time, we cannot reasonably estimate or know the nature, timing and costs of the efforts that will be necessary to complete the preclinical and clinical development of any of our drug candidates.

The successful development and commercialization of our drug candidates is highly uncertain. This is due to the numerous risks and uncertainties, including the following:

- successful completion of preclinical studies and clinical trials;
- receipt and related terms of marketing approvals from applicable regulatory authorities;
- raising additional funds necessary to complete clinical development of and commercialize our drug candidates;
- obtaining and maintaining patent, trade secret and other intellectual property protection and regulatory exclusivity for our drug candidates;
- making arrangements with third-party manufacturers, or establishing manufacturing capabilities, for both clinical and commercial supplies of our drug candidates;
- developing and implementing marketing and reimbursement strategies;
- establishing sales, marketing and distribution capabilities and launching commercial sales of our products, if and when approved, whether alone or in collaboration with others;
- acceptance of our products, if and when approved, by patients, the medical community and third-party payors;
- effectively competing with other therapies;
- the ability to obtain clearance or approval of companion diagnostic tests, if required, on a timely basis, or at all;
- obtaining and maintaining third-party coverage and adequate reimbursement;
- protecting and enforcing our rights in our intellectual property portfolio; and
- maintaining a continued acceptable safety profile of the products following approval.

A change in the outcome of any of these variables with respect to the development of any of our drug candidates would significantly change the costs and timing associated with the development of that drug candidate. We may never succeed in obtaining regulatory approval for any of our drug candidates.

Research and development activities are central to our business model. Drug candidates in later stages of clinical development generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of later-stage clinical trials. We expect research and development costs to increase significantly for the foreseeable future as our drug candidate development programs progress. However, we do not believe that it is possible at this time to accurately project total program-specific expenses through commercialization. There are numerous factors associated with the successful commercialization of any of our drug 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, including stock-based compensation, for personnel in executive, finance and administrative functions. General and administrative expenses also include direct and allocated facility-related costs as well as professional fees for legal, patent, consulting, accounting and audit services.

We anticipate that our general and administrative expenses will increase in the future as we increase our headcount to support our continued research activities and development of our drug candidates. We also anticipate that we will incur increased accounting, audit, legal, regulatory, compliance, and director and officer insurance costs as well as investor and public relations expenses associated with operating as a public company.

Other Income (Expense)

Interest Expense

Interest expense consists of interest expense associated with an outstanding construction loan from a related party. See "Liquidity and Capital Resources—Construction Loan."

Interest and Other Income, net

Interest income consists of interest earned on our cash and cash equivalent balances. In the past our interest income has not been significant due to nominal cash and cash equivalent balances and low interest earned on those balances. We anticipate that our interest income will increase in the future due to higher cash and cash equivalent balances now existing as a result of our IPO in October 2017. Other income, net, consists of insignificant amounts of miscellaneous income and expenses unrelated to our core operations.

Income Taxes

Prior to the Conversion, we have been treated as a partnership for tax purposes and have not been subject to U.S. federal or state income taxation. As a result, since our inception, we have not recorded any U.S. federal or state income tax benefits for the net losses we have incurred in each year or for our earned research and orphan drug credits.

Upon the Conversion, we became subject to typical corporate U.S. federal and state income taxation; however, we do not have net operating loss carryforwards from periods prior to October 2, 2017 available to offset taxable income earned in future periods in which we will be treated as a corporation. To the extent we incur operating losses in future periods in which we are treated as a corporation for tax purposes, net operating loss carryforwards may generally be used by us to offset cash taxes on future taxable income, subject to applicable tax laws.

Critical Accounting Policies and Significant Judgments and Estimates

Our consolidated financial statements are prepared in accordance with generally accepted accounting principles in the United States, or GAAP. The preparation of our consolidated financial statements and related disclosures requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, revenue, costs and expenses, and the disclosure of contingent assets and liabilities in our consolidated financial statements. We base our estimates on historical experience, known trends and events and various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. We evaluate our estimates and assumptions on an ongoing basis. Our actual results may differ from these estimates under different assumptions or conditions.

While our significant accounting policies are described in more detail in Note 2 to our consolidated financial statements, we believe that the following accounting policies are those most critical to the judgments and estimates used in the preparation of our consolidated financial statements.

Accrued Research and Development Expenses

As part of the process of preparing our consolidated financial statements, we are required to estimate our accrued research and development expenses. This process involves reviewing open contracts and purchase

orders, communicating with our applicable 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 actual costs. The majority of our service providers invoice us in arrears for services performed, on a pre-determined schedule or when contractual milestones are met; however, some require advance payments. We make estimates of our accrued expenses as of each balance sheet date in the consolidated financial statements based on facts and circumstances known to us at that time. We periodically confirm the accuracy of the estimates with the service providers and make adjustments if necessary. Examples of estimated accrued research and development expenses include fees paid to:

- vendors in connection with the preclinical development activities;
- CMOs in connection with the production of preclinical and clinical trial materials;
- CROs in connection with preclinical and clinical studies; and
- investigative sites in connection with clinical trials.

We base our expenses related to preclinical studies and clinical trials on our estimates of the services received and efforts expended pursuant to quotes and contracts with multiple research institutions and CROs that conduct and manage preclinical studies and clinical trials on our behalf. 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 expense. 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 the estimate, we adjust the accrual or prepaid expense accordingly. Although we do not expect our estimates to be materially different from amounts actually incurred, our understanding of the status and timing of services performed relative to the actual status and timing of services performed may vary and may result in reporting amounts that are too high or too low in any particular period. To date, there have not been any material adjustments to our prior estimates of accrued research and development expenses.

Stock-Based Compensation

We measure all stock options and other stock-based awards granted to employees and directors based on the fair value on the date of the grant and recognize compensation expense of those awards over the requisite service period, which is generally the vesting period of the respective award. The straight-line method of expense recognition is applied to all awards with service-only conditions while the graded-vesting method is applied to all awards with both service and performance conditions.

We estimate the fair value of each stock-based award using the Black-Scholes option-pricing model, which uses as inputs the fair value of our common stock and assumptions we make for the volatility of our common stock, the expected term of our stock-based awards, the risk-free interest rate for a period that approximates the expected term of our stock-based awards and our expected dividend yield. Prior to October 2017, we were a privately-held company and lacked company-specific historical and implied volatility information. Therefore, we estimated our expected volatility based on the historical volatility of a set of our publicly traded peer companies and expect to continue to do so until such time as we have adequate historical data regarding the volatility of our own traded stock price. We estimated the expected term of our options using the “simplified” method for awards that qualify as “plain-vanilla” options. For options that do not qualify as “plain-vanilla”, we estimated the expected term using the average of vesting date and expiration date as we believe there is no better estimate of expected term. The risk-free interest rate is determined by reference to the U.S. Treasury yield curve in effect at the time of grant of the award for time periods approximately equal to the expected term of the award. Expected dividend yield is based on the fact that we have never paid cash dividends on common stock and do not expect to pay any cash dividends in the foreseeable future.

The assumptions used in determining the fair value of stock-based awards represent our best estimates, but the estimates involve inherent uncertainties and the application of our judgment. As a result, if factors change and we use significantly different assumptions or estimates, our stock-based compensation expense could be materially different in the future.

JOBS Act

We are an "emerging growth company," as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act, and may remain an emerging growth company for up to five years from the date of our initial public offering. The JOBS Act contains provisions that, among other things, reduce certain reporting requirements for an "emerging growth company." As an "emerging growth company," we elected not to take advantage of the extended transition period afforded by the JOBS Act for the implementation of new or revised accounting standards, and as a result, we will comply with new or revised accounting standards on the relevant dates on which adoption of such standards is required for non-emerging growth companies. Section 107 of the JOBS Act provides that our decision not to take advantage of the extended transition period is irrevocable. Subject to certain conditions set forth in the JOBS Act, as an "emerging growth company", we are not required to, among other things, (i) provide an auditor's attestation report on our system of internal controls over financial reporting pursuant to Section 404, (ii) provide all of the compensation disclosure that may be required of non-emerging growth public companies under the Dodd-Frank Wall Street Reform and Consumer Protection Act, (iii) 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 (auditor discussion and analysis), and (iv) disclose certain executive compensation-related items such as the correlation between executive compensation and performance and comparisons of the Chief Executive Officer's compensation to median employee compensation.

Results of Operations

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:

	Year Ended December 31,		
	2017	2016	Change
	(in thousands)		
Revenue	$ —	$ —	$ —
Operating expenses:			
Research and development	39,514	20,163	19,351
General and administrative	11,421	5,675	5,746
Total operating expenses	50,935	25,838	25,097
Loss from operations	(50,935)	(25,838)	(25,097)
Other income (expense):			
Interest expense	(95)	(106)	11
Interest and other income, net	746	4	742
Total other income (expense), net	651	(102)	753
Net loss	$(50,284)	$(25,940)	$(24,344)

Research and Development Expenses

	Year Ended December 31,		
	2017	2016	Change
		(in thousands)	
Direct research and development expenses by program:			
DCC-2618	$22,241	$ 6,791	$15,450
DCC-3014	2,550	2,766	(216)
Rebastinib	357	1,019	(662)
Discontinued program	775	3,584	(2,809)
Unallocated expenses:			
Personnel related (including stock-based compensation)	8,579	4,444	4,135
Facility related and other	5,012	1,559	3,453
Total research and development expenses	$39,514	$20,163	$19,351

Expenses related to our DCC-2618 program increased primarily as a result of increases in clinical trial costs of $10.1 million and manufacturing costs of $4.7 million. The increase in clinical trial costs was due to increased costs related to the expansion cohorts of our Phase 1 clinical trial of DCC-2618 which began enrollment in May 2017. In addition, clinical trial costs increased due to start-up activities related to the pivotal Phase 3 trial in fourth-line plus GIST, which was initiated in January 2018 and increased patient enrollment in the dose escalation stage of our Phase 1 clinical trial of DCC-2618, which was initiated in the fourth quarter of 2015. Manufacturing costs increased in preparation for our current and planned clinical trials.

Expenses related to our DCC-3014 program decreased primarily as a result of a decrease in preclinical costs of $1.4 million, partially offset by an increase in clinical trial costs of $1.3 million. The decrease in preclinical costs was primarily due to costs incurred in the prior year to support our investigational new drug application, or IND, submitted to FDA in the fourth quarter of 2016. The increase in clinical trial costs was primarily due to the initiation of the dose escalation stage of our Phase 1 clinical trial of DCC-3014 in the first quarter of 2017.

Expenses related to our rebastinib program decreased primarily as a result of costs incurred in prior year for research to help assess potential indications for use of rebastinib in human studies and an increase in manufacturing costs to support future clinical studies.

The increase in personnel-related costs included in unallocated expenses was due to an increase in headcount in our research and development function. Personnel-related costs for the years ended December 31, 2017 and 2016 included stock-based compensation expense of $1.3 million and $0.5 million, respectively. The increase in stock-based compensation expense was primarily related to additional employee stock options and a higher value of our common stock. The increase in facility-related and other costs included in unallocated expenses was primarily due to increased costs of $2.2 million incurred in connection with our early-stage drug discovery programs and increased consultant fees of $0.6 million.

General and Administrative Expenses

	Year Ended December 31,		
	2017	2016	Change
		(in thousands)	
Personnel related (including stock-based compensation)	$ 6,568	$2,816	$3,752
Professional and consultant fees	3,564	2,103	1,461
Facility related and other	1,289	756	533
Total general and administrative expenses	$11,421	$5,675	$5,746

The increase in personnel-related costs was primarily a result of an increase in stock-based compensation expense and an increase in headcount. Personnel-related costs for the years ended December 31, 2017 and 2016 included stock-based compensation expense of $3.5 million and $0.9 million, respectively. The increase in stock-based compensation expense was primarily related to additional employee stock options and a higher value of our common stock. The increase in professional and consultant fees was primarily due to an increase in various advisory fees, including those related to business development, accounting and legal fees associated with ongoing business activities and our preparations to operate as a public company.

Interest and Other Income, Net

The increase in interest and other income, net, was primarily due to an increase in interest income resulting from net proceeds from our IPO in October 2017 of $124.6 million, after deducting underwriting discounts and commissions and other offering expenses.

Comparison of the Years Ended December 31, 2016 and 2015

The following table summarizes our results of operations for the years ended December 31, 2016 and 2015:

	Year Ended December 31,		
	2016	2015	Change
	(in thousands)		
Revenue	$ —	$ —	$ —
Operating expenses:			
Research and development	20,163	12,475	7,688
General and administrative	5,675	5,135	540
Total operating expenses	25,838	17,610	8,228
Loss from operations	(25,838)	(17,610)	(8,228)
Other income (expense):			
Interest expense	(106)	(2,209)	2,103
Other income (expense), net	4	3	1
Total other income (expense), net	(102)	(2,206)	2,104
Net loss	$(25,940)	$(19,816)	$(6,124)

Research and Development Expenses

	Year Ended December 31,		
	2016	2015	Change
	(in thousands)		
Direct research and development expenses by program:			
DCC-2618	$ 6,791	$ 3,309	$3,482
DCC-3014	2,766	1,043	1,723
Rebastinib	1,019	571	448
Discontinued program	3,584	3,307	277
Unallocated expenses:			
Personnel related (including stock-based compensation)	4,444	3,677	767
Facility related and other	1,559	568	991
Total research and development expenses	$20,163	$12,475	$7,688

Expenses related to our DCC-2618 program increased primarily as a result of increases in clinical trial costs of $1.9 million and manufacturing costs of $2.1 million, both partially offset by a decrease in preclinical costs of $0.5 million. The increase in clinical trial costs and manufacturing costs was primarily due to costs associated with the dose escalation stage of our Phase 1 clinical trial of DCC-2618, which was initiated in the fourth quarter of 2015. Manufacturing costs also increased in preparation for the planned expansion cohorts of our Phase 1 clinical trial of DCC-2618, which began enrollment in May 2017. These increases were partially offset by a decrease in preclinical activities related to the completion of our toxicology studies in 2015.

Expenses related to our DCC-3014 program increased primarily as a result of an increase in manufacturing costs of $1.0 million and an increase in preclinical activities of $0.6 million. The increase in manufacturing costs primarily resulted from an increase in our supply of clinical trial material in preparation for the initiation of the dose escalation stage of our Phase 1 clinical trial of DCC-3014, following our IND going into effect in the fourth quarter of 2016. The increase in preclinical costs was primarily due to activities supporting our IND filing with FDA.

Expenses related to our rebastinib program increased for the year ended December 31, 2016 compared to the year ended December 31, 2015 primarily due to an increase in research expenses of $0.4 million to help assess potential indications for use of rebastinib in human studies and an increase in manufacturing costs of $0.2 million to support future clinical studies.

The increase in personnel-related costs included in unallocated expenses was due to an increase in headcount in our research and development function, partially offset by a decrease in stock-based compensation. Personnel-related costs for the years ended December 31, 2016 and 2015 included stock-based compensation expense of $0.5 million and $1.4 million, respectively. The increase in facility-related and other costs included in unallocated expenses was primarily due to increased costs of $0.5 million incurred in connection with our early-stage drug discovery programs. Facility-related and other costs also increased by $0.4 million due to new lease agreements for our Lawrence, Kansas and Waltham, Massachusetts facilities, which we entered into in 2015 and 2016, respectively, and increased utilization of our space by our research and development personnel.

General and Administrative Expenses

	Year Ended December 31,		
	2016	2015	Change
		(in thousands)	
Personnel related (including stock-based compensation)	$2,816	$2,476	$ 340
Professional and consultant fees	2,103	2,141	(38)
Facility related and other	756	518	238
Total general and administrative expenses	$5,675	$5,135	$ 540

General and administrative expenses increased primarily due to an increase in personnel-related costs as a result of an increase in headcount, partially offset by a decrease in stock-based compensation. Personnel-related costs for the years ended December 31, 2016 and 2015 included stock-based compensation expense of $0.9 million and $1.2 million, respectively.

Interest Expense

Interest expense was $0.1 million for the year ended December 31, 2016, compared to $2.2 million for the year ended December 31, 2015. The decrease of $2.1 million in interest expense was due to the conversion of outstanding debt from our investors into preferred shares in September 2015.

Liquidity and Capital Resources

Since our inception, we have incurred significant operating losses. We have generated limited revenue to date from a concluded collaboration agreement and research and development grants from the Kansas Bioscience Authority, or KBA. We have not yet commercialized any of our drug candidates and we do not expect to generate revenue from sales of any drug candidates for several years, if at all.

On October 2, 2017, we completed an IPO of our common stock, pursuant to which we issued and sold 7,500,000 shares of common stock at a public offering price of $17.00 per share, resulting in net proceeds of $114.1 million after deducting underwriting discounts and commissions and other offering expenses. On October 4, 2017, we issued and sold an additional 666,496 shares of our common stock at the IPO price of $17.00 per share, pursuant to the partial exercise of the underwriters' option to purchase additional shares of common stock, resulting in additional net proceeds of $10.5 million after deducting underwriting discounts and commissions. Prior to our IPO, we had funded our operations with proceeds from the sales of preferred shares, proceeds from the issuance of convertible notes, payments received under a concluded collaboration agreement, borrowings under a construction loan and research and development grants from the KBA. As of December 31, 2017, we had cash and cash equivalents of $196.8 million.

Cash Flows

As of December 31, 2017, our principal sources of liquidity were cash and cash equivalents of $196.8 million.

The following table summarizes our sources and uses of cash and cash equivalents for each of the periods presented:

Operating Activities

	Year Ended December 31,		
	2017	2016	2015
		(in thousands)	
Cash used in operating activities	$ (36,702)	$(23,090)	$(13,269)
Cash used in investing activities	(406)	(223)	(142)
Cash provided by financing activities	176,401	54,997	38,418
Net increase in cash and cash equivalents	$139,293	$ 31,684	$ 25,007

During the year ended December 31, 2017, operating activities used $36.7 million of cash, primarily resulting from our net loss of $50.3 million, offset by non-cash charges of $5.0 million and cash provided by changes in our operating assets and liabilities of $8.6 million. Net cash provided by changes in our operating assets and liabilities for the year ended December 31, 2017 consisted primarily of a $9.2 million increase in accounts payable and accrued expenses, partially offset by an increase in prepaid expenses and other current assets of $0.6 million.

During the year ended December 31, 2016, operating activities used $23.1 million of cash, primarily resulting from our net loss of $25.9 million, partially offset by non-cash charges of $1.6 million and cash provided by changes in our operating assets and liabilities of $1.3 million. Net cash provided by changes in our operating assets and liabilities for the year ended December 31, 2016 consisted primarily of a $1.5 million increase in accounts payable and accrued expenses, partially offset by an increase in prepaid expenses and other current assets of $0.2 million.

During the year ended December 31, 2015, operating activities used $13.3 million of cash, primarily resulting from our net loss of $19.8 million, partially offset by non-cash charges of $4.7 million and cash provided by changes in our operating assets and liabilities of $1.8 million. Net cash provided by changes in our

operating assets and liabilities for the year ended December 31, 2015 consisted primarily of a $2.3 million increase in accounts payable and accrued expenses, partially offset by an increase in prepaid expenses and other current assets of $0.5 million.

Changes in accounts payable, accrued expenses and prepaid expenses in all periods were generally due to growth in our business and the timing of vendor invoicing and payments.

Investing Activities

During the years ended December 31, 2017, 2016 and 2015 we used $0.4 million, $0.2 million and $0.1 million, respectively, to purchase property and equipment.

Financing Activities

During the year ended December 31, 2017, net cash provided by financing activities was $176.4 million, consisting primarily of proceeds from our IPO, net of underwriting discounts and commissions, of $129.1 million and gross proceeds of $52.3 million from the sale of series C preferred shares, both partially offset by $4.4 million of payments of IPO costs, $0.4 million of payments of series C preferred shares issuance costs and $0.2 million of repayments of notes payable to a related party.

During the year ended December 31, 2016, net cash provided by financing activities was $55.0 million, consisting primarily of net proceeds of $55.3 million from the sale of series B-2 preferred shares, partially offset by $0.2 million of repayments of notes payable to a related party.

During the year ended December 31, 2015, net cash provided by financing activities was $38.4 million, consisting primarily of net proceeds of $31.1 million from the sale of series B-1 preferred shares and proceeds of $7.6 million from the issuance of convertible notes to related parties, partially offset by $0.2 million of repayments of notes payable to a related party.

Construction Loan

We are party to a loan agreement and a security agreement, each dated as of June 11, 2010, with Clinical Reference Laboratory, Inc., or CRL, a related party. The loan was assigned to CHC, Inc., a related party, in December 2016. As of the year ended December 31, 2017 and 2016, there was $1.5 million and $1.7 million, respectively, in principal outstanding under the loan.

Funding Requirements

We expect our expenses to increase substantially in connection with our ongoing activities, particularly as we advance the preclinical activities and clinical trials for our drug candidates in development. In addition, we expect to incur additional costs associated with operating as a public company. The timing and amount of our operating expenditures will depend largely on:

- the timing and progress of preclinical and clinical development activities;
- successful enrollment in and completion of clinical trials;
- the timing and outcome of regulatory review of our drug candidates;
- the cost to develop companion diagnostics as needed for each of our drug candidates;
- our ability to establish agreements with third-party manufacturers for clinical supply for our clinical trials and, if any of our drug candidates are approved, commercial manufacturing;
- addition and retention of key research and development personnel;

- our efforts to enhance operational, financial and information management systems, and hire additional personnel, including personnel to support development of our drug candidates;
- the costs and timing of future commercialization activities, including product manufacturing, marketing, sales and distribution, for any of our drug candidates for which we obtain marketing approval;
- the legal patent costs involved in prosecuting patent applications and enforcing patent claims and other intellectual property claims; and
- the terms and timing of any collaboration, license or other arrangement, including the terms and timing of any milestone payments thereunder.

As of December 31, 2017, we had cash and cash equivalents of $196.8 million. We believe that the net proceeds from the IPO, together with our existing cash and cash equivalents, will enable us to fund our operating expenses, capital expenditure requirements and debt service payments into the second half of 2019. We have based these estimates on assumptions that may prove to be wrong, and we could utilize our available capital resources sooner than we expect.

Until such time, if ever, as we can generate substantial product revenue, we expect to finance our operations through a combination of equity offerings, debt financings, collaborations, strategic alliances and marketing, distribution or licensing arrangements. We do not currently have any committed external source of funds. To the extent that we raise additional capital through the sale of equity or convertible debt securities, your ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect your rights as a common stockholder. Debt financing and preferred equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making acquisitions or capital expenditures or declaring dividends. If we raise additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or drug candidates or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings or other arrangements when needed, we may be required to delay, limit, reduce or terminate our research, product development or future commercialization efforts or grant rights to develop and market drug candidates that we would otherwise prefer to develop and market ourselves.

Contractual Obligations and Commitments

The following table summarizes our contractual obligations as of December 31, 2017 and the effects that such obligations are expected to have on our liquidity and cash flows in future periods:

	Payments Due By Period				
	Total	Less Than 1 Year	1 to 3 Years	4 to 5 Years	More Than 5 Years
			(in thousands)		
Operating lease commitments(1)	$1,627	$ 675	$ 952	$ —	$ —
Notes payable to related party(2)	1,837	271	508	463	595
Total	$3,464	$ 946	$ 1,460	$ 463	$ 595

(1) Reflects payments due for our lease of office and laboratory space in Lawrence, Kansas under operating lease agreements that expire at various dates through 2020 and for our sublease agreement for corporate office space in Waltham, Massachusetts that expires in 2019.

(2) Reflects the contractually required principal and interest payments payable pursuant to our outstanding construction loan. See “Certain Relationships and Related Person Transactions.”

We enter into contracts in the normal course of business with various third parties for clinical trials, preclinical research studies and testing, manufacturing and other services and products for operating purposes.

These contracts provide for termination upon notice. Payments due upon cancellation consist only of payments for services provided or expenses incurred, including non-cancellable obligations of our service providers, up to the date of cancellation. These payments are not included in the table of contractual obligations above.

Pursuant to the terms of our research and development grants from the KBA, we may be required to repay some or all of the financial assistance received thereunder if certain conditions are met. As we do not consider repayment related to the KBA grants to be probable, we have not included a related obligation in the table above.

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.

Recently Issued and Adopted Accounting Pronouncements

A description of recently issued accounting pronouncements that may potentially impact our financial position and results of operations is disclosed in Note 2 to our consolidated financial statements appearing at the end of this prospectus.

ITEM 7A. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK

Our cash and cash equivalents as of December 31, 2017 consisted of cash and money market accounts. Our primary exposure to market risk is interest income sensitivity, which is affected by changes in the general level of interest rates. Because of the short-term nature of the instruments in our portfolio, a sudden change in market interest rates would not be expected to have a material impact on our financial position or results of operations. As of December 31, 2017, our outstanding indebtedness accrued interest at a fixed interest rate. As a result, a change in market interest rates would not have had any impact on our financial position or results of operations.

ITEM 8. CONSOLIDATED FINANCIAL STATEMENTS AND SUPPLEMENTARY DATA

DECIPHERA PHARMACEUTICALS, INC.

Index to Consolidated Financial Statements

Report of Independent Registered Public Accounting Firm

To the Board of Directors and Stockholders of Deciphera Pharmaceuticals, Inc.

Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheets of Deciphera Pharmaceuticals, Inc. and its subsidiaries as of December 31, 2017 and 2016, and the related consolidated statements of operations and comprehensive loss**,** convertible preferred shares and stockholders' equity/members' deficit, and cash flow for each of the three years in the period ended December 31, 2017, including the related notes (collectively referred to as the "consolidated financial statements"). In our opinion, the consolidated financial statements present fairly, in all material respects, the financial position of the Company as of December 31, 2017 and 2016, and the results of their operations and their cash flows for each of the three years in the period ended December 31, 2017 in conformity with accounting principles generally accepted in the United States of America.

Basis for Opinion

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

We conducted our audits of these consolidated financial statements in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the consolidated financial statements are free of material misstatement, whether due to error or fraud.

Our audits included performing procedures to assess the risks of material misstatement of the consolidated financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the consolidated financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the consolidated financial statements. We believe that our audits provide a reasonable basis for our opinion.

Emphasis of Matter

As discussed in Note 1 to the consolidated financial statements, the Company will require additional financing to fund future operations. Management's plans in regard to this matter are described in Note 1.

/s/ PricewaterhouseCoopers LLP

Boston, Massachusetts
March 28, 2018

We have served as the Company's auditor since 2009.

DECIPHERA PHARMACEUTICALS, INC.

CONSOLIDATED BALANCE SHEETS

(In thousands, except share and per share amounts)

	December 31, 2017	December 31, 2016
Assets		
Current assets:		
Cash and cash equivalents	$ 196,754	$ 57,461
Prepaid expenses and other current assets	1,428	791
Total current assets	198,182	58,252
Property and equipment, net	838	514
Deferred offering costs	—	104
Other assets	75	75
Total assets	$ 199,095	$ 58,945
Liabilities, Convertible Preferred Shares and Stockholders' Equity/Members' Deficit		
Current liabilities:		
Accounts payable	$ 4,395	$ 1,413
Accrued expenses	9,233	2,957
Notes payable to related party	187	187
Total current liabilities	13,815	4,557
Notes payable to related party, net of current portion	1,294	1,481
Other long-term liabilities	13	—
Total liabilities	15,122	6,038
Commitments and contingencies (Note 11)		
Convertible preferred shares (Series A, B-1, B-2, C), no par value; no shares and 3,632,711 authorized as of December 31, 2017 and 2016, respectively; no shares and 3,632,711 shares issued and outstanding as of December 31, 2017 and 2016	—	192,667
Stockholders' Equity/Members' Deficit:		
Common shares, no par value; no shares and 4,366,052 shares authorized as of December 31, 2017 and 2016, respectively; no shares issued or outstanding as of December 31, 2017 and 2016	—	—
Preferred stock, $0.01 par value per share; 5,000,000 shares and no shares authorized as of December 31, 2017 and 2016, respectively; no shares issued or outstanding as of December 31, 2017 and 2016, respectively;	—	—
Common stock, $0.01 par value per share; 125,000,000 shares and no shares authorized, as of December 31, 2017 and 2016, respectively; 32,591,686 shares and no shares issued and outstanding as of December 31, 2017 and 2016, respectively	326	—
Additional paid-in capital	379,516	5,825
Accumulated deficit	(195,869)	(145,585)
Total stockholders' equity/members' deficit	183,973	(139,760)
Total liabilities, convertible preferred shares and stockholders' equity/members' deficit	$ 199,095	$ 58,945

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

DECIPHERA PHARMACEUTICALS, INC.

CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS

(In thousands, except share and per share amounts)

	Year Ended December 31,		
	2017	2016	2015
Revenue	$ —	$ —	$ —
Operating expenses:			
Research and development	39,514	20,163	12,475
General and administrative	11,421	5,675	5,135
Total operating expenses	50,935	25,838	17,610
Loss from operations	(50,935)	(25,838)	(17,610)
Other income (expense):			
Interest expense	(95)	(106)	(2,209)
Interest and other income, net	746	4	3
Total other income (expense), net	651	(102)	(2,206)
Net loss and comprehensive loss	$ (50,284)	$ (25,940)	$ (19,816)
Net loss per share—basic and diluted	$ (2.99)	$ (2.23)	$ (4.67)
Weighted average common shares outstanding—basic and diluted(1)	16,792,179	11,626,287	4,245,698

(1) The Company did not have any common shares outstanding during the years ended December 31, 2015 and 2016 or for the period from January 1, 2017 through the closing of its initial public offering on October 2, 2017. To determine the weighted average shares outstanding for purposes of calculating net loss per share during those periods, the Company used the weighted average number of Series A convertible preferred shares outstanding because such shares represented the most subordinated share class outstanding during those periods. Share amounts for periods prior to the initial public offering have been retrospectively adjusted to give effect to the exchange of Series A convertible preferred shares into shares of common stock upon the Conversion (see Note 1).

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

DECIPHERA PHARMACEUTICALS, INC.

CONSOLIDATED STATEMENTS OF CONVERTIBLE PREFERRED SHARES AND STOCKHOLDERS' EQUITY/MEMBERS' DEFICIT

(In thousands, except share amounts)

	Member Units		Series A, B-1, B-2 and C Convertible Preferred Shares		Common Stock		Additional Paid-in Capital	Accumulated Deficit	Total Stockholders' Equity/ Members' Deficit
	Shares	Amount	Shares	Amount	Shares	Amount			
Balances at December 31, 2014	202,500	$ 8,491	—	$ —	—	$ —	$ 294	$ (99,829)	$ (99,535)
Conversion of member units into Series A convertible preferred shares	(202,500)	(8,491)	202,500	8,491	—	—	—	—	—
Gain on extinguishment of notes payable to related party	—	—	—	—	—	—	1,487	—	1,487
Conversion of notes payable and accrued interest into Series A convertible preferred shares, net of issuance costs of $30	—	—	1,855,250	94,065	—	—	—	—	—
Conversion of notes payable and accrued interest into Series B-1 convertible preferred shares	—	—	73,811	3,728	—	—	—	—	—
Issuance of Series B-1 convertible preferred shares, net of issuance costs of $468	—	—	624,784	31,084	—	—	—	—	—
Share-based compensation expense	—	—	—	—	—	—	2,557	—	2,557
Net loss	—	—	—	—	—	—	—	(19,816)	(19,816)
Balances at December 31, 2015	—	—	2,756,345	137,368	—	—	4,338	(119,645)	(115,307)
Issuance of Series B-2 convertible preferred shares, net of issuance costs of $25	—	—	876,366	55,299	—	—	—	—	—
Share-based compensation expense	—	—	—	—	—	—	1,487	—	1,487
Net loss	—	—	—	—	—	—	—	(25,940)	(25,940)
Balances at December 31, 2016	—	—	3,632,711	192,667	—	—	5,825	(145,585)	(139,760)
Issuance of Series C convertible preferred shares, net of issuance costs of $429	—	—	690,333	51,871	—	—	—	—	—
Effect of Conversion (Note 1)	—	—	(4,323,044)	(244,538)	24,425,190	244	244,294	—	244,538
Issuance of common stock sold in initial public offering, net of underwriting discounts, commissions and offering costs	—	—	—	—	8,166,496	82	124,531	—	124,613
Share-based compensation expense	—	—	—	—	—	—	4,866	—	4,866
Net loss	—	—	—	—	—	—	—	(50,284)	(50,284)
Balances at December 31, 2017	—	$ —	—	$ —	32,591,686	$ 326	$ 379,516	$ (195,869)	$ 183,973

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

DECIPHERA PHARMACEUTICALS, INC.

CONSOLIDATED STATEMENTS OF CASH FLOWS

(In thousands)

	Year ended December 31,		
	2017	2016	2015
Cash flows from operating activities:			
Net loss	$ (50,284)	$(25,940)	$(19,816)
Adjustments to reconcile net loss to net cash used in operating activities:			
Share-based compensation expense	4,866	1,487	2,557
Depreciation and amortization expense	150	90	73
Non-cash interest expense	—	—	2,091
Loss on disposal of property and equipment	10	6	—
Changes in operating assets and liabilities:			
Prepaid expenses and other current assets	(637)	(185)	(516)
Accounts payable	2,904	(187)	1,176
Accrued expenses	6,276	1,694	1,160
Other assets	—	(55)	6
Other long-term liabilities	13	—	—
Net cash used in operating activities	(36,702)	(23,090)	(13,269)
Cash flows from investing activities:			
Purchases of property and equipment	(406)	(223)	(142)
Net cash used in investing activities	(406)	(223)	(142)
Cash flows from financing activities:			
Proceeds from initial public offering, net of underwriting discounts and commissions	129,112	—	—
Payments of initial public offering costs	(4,395)	(104)	—
Proceeds from issuance of convertible preferred shares	52,300	55,324	31,552
Proceeds from issuance of convertible notes payable to related parties	—	—	7,550
Repayment of notes payable to related party	(187)	(198)	(186)
Payments of convertible preferred share issuance costs	(429)	(25)	(498)
Net cash provided by financing activities	176,401	54,997	38,418
Net increase in cash and cash equivalents	139,293	31,684	25,007
Cash and cash equivalents at beginning of period	57,461	25,777	770
Cash and cash equivalents at end of period	$196,754	$ 57,461	$ 25,777
Supplemental disclosure of cash flow information:			
Cash paid for interest	$ 95	$ 106	$ 117
Supplemental disclosure of non-cash financing activities:			
Conversion of convertible preferred shares into common stock	$244,538	$ —	$ —
Conversion of notes payable and accrued interest into convertible preferred shares	$ —	$ —	$ 97,793
Conversion of member units into Series A convertible preferred shares	$ —	$ —	$ 8,491
Gain on extinguishment of notes payable to related party	$ —	$ —	$ 1,487
Purchases of property and equipment included in accounts payable or accrued expenses	$ 78	$ —	$ —

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

DECIPHERA PHARMACEUTICALS, INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

1. Nature of the Business and Basis of Presentation

Deciphera Pharmaceuticals, Inc. (the "Company") is a clinical-stage biopharmaceutical company developing new drugs to improve the lives of cancer patients by addressing key mechanisms of drug resistance that limit the rate and durability of response of many cancer therapies. The Company's targeted, small molecule drug candidates, designed using its proprietary kinase switch control inhibitor platform, inhibit the activation of kinases, an important family of enzymes, that, when mutated or over expressed, are known to be directly involved in the growth and spread of many cancers.

On October 2, 2017, immediately prior to the completion of its initial public offering ("IPO"), the Company engaged in a series of transactions whereby Deciphera Pharmaceuticals, LLC became a wholly owned subsidiary of Deciphera Pharmaceuticals, Inc., a Delaware corporation. As part of the transactions, shareholders of Deciphera Pharmaceuticals, LLC exchanged their shares of Deciphera Pharmaceuticals, LLC for shares of Deciphera Pharmaceuticals, Inc. on a one-for-5.65 basis, (the "Conversion").

On October 2, 2017, Deciphera Pharmaceuticals, Inc., completed the IPO, pursuant to which it issued and sold 7,500,000 shares of common stock at the IPO price of $17.00 per share, resulting in net proceeds of $114.1 million after deducting underwriting discounts and commissions and other offering expenses. On October 4, 2017, the Company issued and sold an additional 666,496 shares of common stock at the IPO price of $17.00 per share pursuant to the underwriters' partial exercise of their option to purchase additional shares of common stock, resulting in additional net proceeds of $10.5 million after deducting underwriting discounts and commissions. Upon the closing of the IPO, the Company's outstanding convertible preferred shares automatically converted into shares of common stock.

The Company is subject to risks and uncertainties common to early-stage companies in the biotechnology industry, including, but not limited to, development by competitors of new technological innovations, dependence on key personnel, protection of proprietary technology, compliance with government regulations and the ability to secure additional capital to fund operations. Drug candidates currently under development will require significant additional research and development efforts, including extensive preclinical and clinical testing and regulatory approval prior to commercialization. These efforts require significant amounts of additional capital, adequate personnel and infrastructure and extensive compliance-reporting capabilities. Even if the Company's drug development efforts are successful, it is uncertain when, if ever, the Company will realize significant revenue from product sales.

The accompanying consolidated financial statements have been prepared on the basis of continuity of operations, realization of assets and the satisfaction of liabilities and commitments in the ordinary course of business. Since inception, the Company has incurred recurring losses including net losses of $50.3 million and $25.9 million for the years ended December 31, 2017 and 2016, respectively. As of December 31, 2017 the Company had an accumulated deficit of $195.9 million. The Company expects to continue to generate operating losses in the foreseeable future. As of the issuance date of the consolidated financial statements, the Company expects that its cash and cash equivalents of $196.8 million as of December 31, 2017 would be sufficient to fund its operating expenses, capital expenditure requirements and debt service payments through at least 12 months from the issuance date of these consolidated financial statements. The future viability of the Company is dependent on its ability to raise additional capital to fund its operations.

The Company expects its expenses to increase substantially in connection with ongoing activities, particularly as the Company advances its preclinical activities and clinical trials for its drug candidates in development. Accordingly, the Company will need to obtain substantial additional funding in connection with continuing operations. If the Company is unable to raise capital when needed, or on attractive terms, it could be

forced to delay, reduce or eliminate its research or drug development programs or any future commercialization efforts. Although management continues to pursue these plans, there is no assurance that the Company will be successful in obtaining sufficient funding on terms acceptable to the Company to fund continuing operations, if at all.

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

The consolidated financial statements have been prepared in accordance with accounting principles generally accepted in the United States of America ("GAAP").

2. Summary of Significant Accounting Policies

Use of Estimates

The preparation of financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities, the disclosure of contingent assets and liabilities at the date of the financial statements and the reported amounts of revenue and expenses during the reporting periods. Significant estimates and assumptions reflected in these consolidated financial statements include, but are not limited to, the accrual for research and development expenses, prior to the Company's IPO, the valuation of common stock, and the valuation of stock-based awards. Estimates are periodically reviewed in light of changes in circumstances, facts and experience. Changes in estimates are recorded in the period in which they become known. Actual results could differ from those estimates.

Cash Equivalents

The Company considers all highly liquid investments with original maturities of three months or less at the date of purchase to be cash equivalents.

Concentrations of Credit Risk and of Significant Suppliers

Financial instruments that potentially expose the Company to concentrations of credit risk consist primarily of cash and cash equivalents. The Company maintains all cash and cash equivalents at one accredited financial institution. The Company does not believe that it is subject to unusual credit risk beyond the normal credit risk associated with commercial banking relationships.

The Company is dependent on third-party manufacturers to supply products for research and development activities in its programs. In particular, the Company relies and expects to continue to rely on a small number of manufacturers to supply it with its requirements for the active pharmaceutical ingredients and formulated drugs related to these programs. These programs could be adversely affected by a significant interruption in the supply of active pharmaceutical ingredients and formulated drugs.

Deferred Offering Costs

The Company capitalizes certain legal, professional accounting and other third-party fees that are directly associated with in-process equity financings as deferred offering costs until such financings are consummated. After consummation of the equity financing, these costs are recorded in stockholders' equity/members' deficit as a reduction of additional paid-in capital generated as a result of the offering. Should the planned equity financing be abandoned, the deferred offering costs will be expensed immediately as a charge to operating expenses in the statement of operations and comprehensive loss. As of December 31, 2016, the Company recorded $0.1 million of deferred offering costs in contemplation of the Company's IPO, which closed in October 2017, and such costs were transferred to additional paid-in capital upon completion of the IPO. The Company has no deferred offering costs as of December 31, 2017.

Property and Equipment

Property and equipment are stated at cost less accumulated depreciation and amortization. Depreciation and amortization expense is recognized using the straight-line method over the estimated useful life of each asset as follows:

	Estimated Useful Life
Lab equipment	5 to 7 years
Computer equipment	3 to 5 years
Furniture and fixtures	7 years
Leasehold improvements	Shorter of life of lease or 15 years

Upon retirement or sale, the cost of assets disposed of and the related accumulated depreciation and amortization are removed from the accounts and any resulting gain or loss is included in loss from operations. Expenditures for repairs and maintenance are charged to expense as incurred.

Impairment of Long-Lived Assets

Long-lived assets consist of property and equipment. Long-lived assets to be held and used are tested for recoverability whenever events or changes in business circumstances indicate that the carrying amount of the assets may not be fully recoverable. Factors that the Company considers in deciding when to perform an impairment review include significant underperformance of the business in relation to expectations, significant negative industry or economic trends and significant changes or planned changes in the use of the assets. If an impairment review is performed to evaluate a long-lived asset group for recoverability, the Company compares forecasts of undiscounted cash flows expected to result from the use and eventual disposition of the long-lived asset group to its carrying value. An impairment loss would be recognized when estimated undiscounted future cash flows expected to result from the use of an asset group are less than its carrying amount. The impairment loss would be based on the excess of the carrying value of the impaired asset group over its fair value, determined based on discounted cash flows. The Company did not record any impairment losses on long-lived assets during the years ended December 31, 2017, 2016 or 2015.

Fair Value Measurements

Certain assets and liabilities are carried at fair value under GAAP. Fair value is defined as the exchange price that would be received for an asset or paid to transfer a liability (an exit price) in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants on the measurement date. Valuation techniques used to measure fair value must maximize the use of observable inputs and minimize the use of unobservable inputs. Financial assets and liabilities carried at fair value are to be classified and disclosed in one of the following three levels of the fair value hierarchy, of which the first two are considered observable and the last is considered unobservable:

- Level 1—Quoted prices in active markets for identical assets or liabilities.
- Level 2—Observable inputs (other than Level 1 quoted prices), such as quoted prices in active markets for similar assets or liabilities, quoted prices in markets that are not active for identical or similar assets or liabilities, or other inputs that are observable or can be corroborated by observable market data.
- Level 3—Unobservable inputs that are supported by little or no market activity and that are significant to determining the fair value of the assets or liabilities, including pricing models, discounted cash flow methodologies and similar techniques.

The Company’s cash equivalents are carried at fair value, determined according to the fair value hierarchy described above (see Note 3). The carrying values of the Company’s accounts payable and accrued expenses

approximate their fair values due to the short-term nature of these liabilities. The fair value of the Company's outstanding notes payable to related party (see Note 6) as of December 31, 2017 and 2016 approximated $1.2 million and $1.3 million, respectively. The fair value of the outstanding debt was estimated using a discounted cash flow analysis based on current market interest rates for debt issuances with similar remaining years to maturity, adjusted for credit risk, which represents a Level 3 measurement.

Segment Information

The Company manages its operations as a single segment for the purposes of assessing performance and making operating decisions. The Company's singular focus is designing, optimizing and introducing small molecule switch control inhibitors of protein kinases for human clinical trials and the global pharmaceutical marketplace through the use of its proprietary drug discovery technology platform. All of the Company's tangible assets are held in the United States.

Research and Development Costs

Research and development costs are expensed as incurred. Research and development expenses are comprised of costs incurred in performing research and development activities, including salaries, stock-based compensation and benefits, facilities costs, depreciation, manufacturing expenses and external costs of outside vendors engaged to conduct preclinical development activities and trials.

Advance payments for goods or services to be received in the future for use in research and development activities are recorded as prepaid expenses. The prepaid amounts are expensed as the related goods are delivered or the services are performed.

Research Contract Costs and Accruals

The Company has entered into various research and development contracts with research institutions and other companies both inside and outside of the United States. These agreements are generally cancelable, and related payments are recorded as research and development expenses as incurred. The Company records accruals for estimated ongoing research costs. When evaluating the adequacy of the accrued liabilities, the Company analyzes progress of the studies or trials, including the phase or completion of events, invoices received and contracted costs. Significant judgments and estimates are made in determining the accrued balances at the end of any reporting period. Actual results could differ from the Company's estimates. The Company's historical accrual estimates have not been materially different from the actual costs.

Patent Costs

All patent-related costs incurred in connection with filing and prosecuting patent applications are expensed as incurred due to the uncertainty about the recovery of the expenditure. Amounts incurred are classified as general and administrative expenses.

Stock-Based Compensation

The Company measures all stock options and other stock-based awards granted to employees and directors based on the fair value on the date of the grant and recognizes compensation expense of those awards over the requisite service period, which is generally the vesting period of the respective award. The straight-line method of expense recognition is applied to all awards with service-only conditions while the graded-vesting method is applied to all awards with both service and performance conditions. The Company accounts for forfeitures as they occur.

The Company classifies stock-based compensation expense in its statements of operations and comprehensive loss in the same manner in which the award recipient's payroll costs are classified or in which the award recipient's service payments are classified.

Income Taxes

Upon consummation of the Conversion on October 2, 2017, the Company became subject to corporate U.S. federal and state income taxes. Prior to the Conversion, the Company was treated as a partnership for income tax purposes and was not subject to U.S. federal or state income taxation. As a result, the Company had not recorded any U.S. federal or state income tax benefits prior to October 2, 2017 for the net losses incurred in each reporting period or for any earned research and orphan drug credits as the operating losses incurred by the Company had been passed through to its members.

The Company accounts for income taxes using the asset and liability method, which requires the recognition of deferred tax assets and liabilities for the expected future tax consequences of events that have been recognized in the consolidated financial statements or in the Company's tax returns. Deferred taxes are determined based on the difference between the financial statement and tax basis of assets and liabilities using enacted tax rates in effect in the years in which the differences are expected to reverse. Changes in deferred tax assets and liabilities are recorded in the provision for income taxes. The Company assesses the likelihood that its deferred tax assets will be recovered from future taxable income and, to the extent it believes, based upon the weight of available evidence, that it is more likely than not that all or a portion of deferred tax assets will not be realized, a valuation allowance is established through a charge to income tax expense. Potential for recovery of deferred tax assets is evaluated by estimating the future taxable profits expected and considering prudent and feasible tax planning strategies.

The Company accounts for uncertainty in income taxes recognized in its consolidated financial statements by applying a two-step process to determine the amount of tax benefit to be recognized. First, the tax position must be evaluated to determine the likelihood that it will be sustained upon external examination by the taxing authorities. If the tax position is deemed more-likely-than- not to be sustained, the tax position is then assessed to determine the amount of benefit to recognize in the consolidated financial statements. The amount of the benefit that may be recognized is the largest amount that has a greater than 50% likelihood of being realized upon ultimate settlement. The provision for income taxes includes the effects of any resulting tax reserves, or unrecognized tax benefits, that are considered appropriate as well as the related net interest and penalties.

Comprehensive Loss

Comprehensive loss includes net loss as well as other changes in stockholders' equity/members' deficit that result from transactions and economic events other than those with shareholders. There was no difference between net loss and comprehensive loss for each of the periods presented in the accompanying consolidated financial statements.

Net Loss per Share

Basic net income (loss) per share is computed by dividing the net income (loss) by the weighted average number of common shares outstanding for the year ended December 31, 2017. Diluted net income (loss) per is computed by dividing the diluted net income (loss) by the weighted average number of common shares, including potential dilutive common shares assuming the dilutive effect of outstanding stock options and unvested restricted common shares, as determined using the treasury stock method. For periods in which the Company has reported net losses, diluted net loss per common share is the same as basic net loss per common share, since dilutive common shares are not assumed to have been issued if their effect is antidilutive.

The Company did not have any common shares outstanding during the years ended December 31, 2015 and 2016 or for the period from January 1, 2017 through the closing of its initial public offering on October 2, 2017. To determine the weighted average shares outstanding for purpose of calculating net loss per share during those periods, the Company used the weighted average number of Series A convertible preferred shares outstanding because such shares represented the most subordinate share class outstanding during those periods, and such

shares were retrospectively adjusted to give effect to the exchange of Series A convertible preferred shares into shares of common stock upon the Conversion (see Note 1).

Recently Issued Accounting Pronouncements

In May 2014, the FASB issued ASU No. 2014-09, *Revenue from Contracts with Customers (Topic 606)* ("ASU 2014-09"), which supersedes existing revenue recognition guidance under GAAP. The standard's core principle is that a company will recognize revenue when it transfers promised goods or services to customers in an amount that reflects the consideration to which the company expects to be entitled in exchange for those goods or services. The standard defines a five-step process to achieve this principle, and will require companies to use more judgment and make more estimates than under the current guidance. The Company expects that these judgments and estimates will include identifying performance obligations in the customer contract, estimating the amount of variable consideration to include in the transaction price and allocating the transaction price to each separate performance obligation. ASU 2014-09 also requires additional disclosure about the nature, amount, timing and uncertainty of revenue and cash flows arising from customer contracts. In August 2015, the FASB issued ASU 2015-14, *Revenue from Contracts with Customers (Topic 606): Deferral of the Effective Date*, which delays the effective date of ASU 2014-09 such that the standard is effective for public entities for annual periods beginning after December 15, 2017 and for interim periods within those fiscal years. In March 2016, the FASB issued ASU No. 2016-08, *Revenue from Contracts with Customers (Topic 606): Principal versus Agent Considerations* ("ASU 2016-08"), which further clarifies the implementation guidance on principal versus agent considerations in ASU 2014-09. In April 2016, the FASB issued ASU No. 2016-10, *Revenue from Contracts with Customers (Topic 606): Identifying Performance Obligations and Licensing*, clarifying the implementation guidance on identifying performance obligations and licensing. Specifically, the amendments in this update reduce the cost and complexity of identifying promised goods or services and improve the guidance for determining whether promises are separately identifiable. The amendments in this update also provide implementation guidance on determining whether an entity's promise to grant a license provides a customer with either a right to use the entity's intellectual property (which is satisfied at a point in time) or a right to access the entity's intellectual property (which is satisfied over time). In May 2016, the FASB issued ASU No. 2016-12, *Revenue from Contracts with Customers (Topic 606): Narrow-Scope Improvements and Practical Expedients* ("ASU 2016-12"), which clarifies the objective of the collectability criterion, presentation of taxes collected from customers, non-cash consideration, contract modifications at transition, completed contracts at transition and how guidance in ASU 2014-09 is retrospectively applied. ASU 2016-08, ASU 2016-10 and ASU 2016-12 have the same effective dates and transition requirements as ASU 2014-09. The adoption of these standards is not expected to have an impact on the Company's financial position, results of operations or cash flows as the Company does not currently have any revenue-generating arrangements.

In February 2016, the FASB issued ASU No. 2016-02, *Leases* ("ASU 2016-02"). ASU 2016-02 will require lessees to recognize most leases on their balance sheet as a right-of-use asset and a lease liability. Leases will be classified as either operating or finance, and classification will be based on criteria similar to current lease accounting, but without explicit bright lines. The guidance is effective for annual reporting periods beginning after December 15, 2018 and interim periods within those fiscal years, and early adoption is permitted. The Company is currently evaluating the impact that the adoption of ASU 2016-02 will have on its financial position, results of operations or cash flows.

In May 2017, the FASB issued ASU No. 2017-09, *Compensation—Stock Compensation (Topic 718): Scope of Modification Accounting* ("ASU 2017-09"), which clarifies when to account for a change to the terms or conditions of a share-based payment award as a modification. Under the new guidance, modification accounting is required only if the fair value, the vesting conditions, or the classification of the award (as equity or liability) changes as a result of the change in terms or conditions. The standard is effective for annual periods beginning after December 15, 2017, including interim periods within those fiscal years. Early adoption is permitted. The Company is currently evaluating the impact that the adoption of ASU 2017-09 will have on its consolidated financial statements. The Company does not expect that the adoption of ASU 2017-09 will have a material impact on its financial position, results of operations or cash flows.

3. Fair Value of Financial Assets

The following tables present information about the Company's financial assets measured at fair value on a recurring basis and indicate the level of the fair value hierarchy utilized to determine such fair values (in thousands):

	Fair Value Measurements at December 31, 2017 Using:			
	Level 1	Level 2	Level 3	Total
Cash equivalents:				
Money market funds	$ —	$ 191,950	$ —	$ 191,950
Total	$ —	$ 191,950	$ —	$ 191,950

	Fair Value Measurements at December 31, 2016 Using:			
	Level 1	Level 2	Level 3	Total
Cash equivalents:				
Money market funds	$ —	$ 53,180	$ —	$ 53,180
Total	$ —	$ 53,180	$ —	$ 53,180

During the years ended December 31, 2017 and 2016, there were no transfers between Level 1, Level 2 and Level 3.

4. Property and Equipment, Net

Property and equipment, net consisted of the following (in thousands):

	December 31,	
	2017	2016
Laboratory equipment	$ 981	$ 602
Leasehold improvements	344	349
Computer equipment	300	225
Furniture and fixtures	128	104
	1,753	1,280
Less: Accumulated depreciation and amortization	(915)	(766)
	$ 838	$ 514

Depreciation and amortization expense was $0.2 million for the year ended December 31, 2017 and $0.1 million for each of the years ended December 31, 2016 and 2015, respectively.

5. Accrued Expenses

Accrued expenses consisted of the following (in thousands):

	December 31,	
	2017	2016
Accrued external research and development expenses	$6,625	$1,433
Accrued payroll and related expenses	2,233	1,267
Accrued professional fees	353	240
Accrued other	22	17
	$9,233	$2,957

6. Notes Payable to Related Parties

2004 Convertible Loan Agreement

In 2004, the Company entered into a convertible loan agreement (the "2004 Loan") with Brightstar Associates, LLC ("Brightstar"), a related party (see Note 14). In May 2015, the 2004 Loan was amended to (i) consolidate the then-current accrued interest of $5.0 million and outstanding principal for a new principal balance of $12.6 million, (ii) extend the maturity date to be due on demand after December 31, 2019 and (iii) allow Brightstar, at its option, to convert the outstanding principal and accrued interest into member units of the Company at a price of $51.52 per member unit, subject to appropriate adjustment in the event of the issuance of any member units or securities convertible into member units at a lower price per member unit than $51.52. The Company determined that such amendments should be accounted for as an extinguishment of the 2004 Loan for accounting purposes. As a result, the carrying value of the 2004 Loan of $12.6 million at the time of the extinguishment was removed from the balance sheet and was recorded at its then-current fair value of $11.1 million. The resulting gain on extinguishment of $1.5 million was recognized as additional paid-in capital, a component of members' deficit, due to the related party nature of the 2004 Loan. At the date of extinguishment, there were no unamortized debt discounts or debt issuance costs.

The Company assessed the embedded conversion and repayment features of the 2004 Loan and determined that there were no features that were required to be separated and accounted for as derivatives.

2006 through 2013 Convertible Loan Agreements

From September 2006 through June 2013, the Company entered into five convertible loan agreements (the "Pre-2015 Convertible Loans") with Brightstar and Biochenomix, L.L.C. ("Biochenomix"), which provided for aggregate borrowings of up to $70.1 million. Through December 31, 2014, the Company drew down $66.2 million under the Pre-2015 Convertible Loans, and in 2015, the remaining $3.9 million was drawn down. Interest under the Pre-2015 Convertible Loans accrued monthly at the prime rate.

The Company assessed the embedded conversion and repayment features of the Pre-2015 Convertible Loans and determined that there were no features that were required to be separated and accounted for as derivatives.

Conversion of 2004 Loan and Pre-2015 Convertible Loans

In September 2015, all outstanding principal and accrued interest of $95.6 million, with a carrying value of $94.1 million, due under the 2004 Loan and the Pre-2015 Convertible Loans was converted into 1,855,250 Series A convertible preferred shares (see Note 7).

2015 Convertible Loan Agreement

In May 2015, the Company entered into a seventh convertible loan agreement (the "2015 Loan") with Brightstar and Biochenomix, which provided for aggregate borrowings of up to $15.0 million. Interest under the 2015 Loan accrued monthly at the prime rate. In May 2015 and July 2015, the Company drew down $2.0 million and $1.7 million, respectively, under the 2015 Loan.

The Company assessed the embedded conversion and repayment features of the 2015 Loan and determined that there were no features that were required to be separated and accounted for as derivatives.

In September 2015, all outstanding principal and accrued interest of $3.7 million due under the 2015 Loan was converted into 73,811 Series B-1 convertible preferred shares (see Note 7).

2010 Construction Loan Agreement

In June 2010, the Company entered into a loan agreement and a security agreement (together, the "CRL Construction Loan") with Clinical Reference Laboratory, Inc. ("CRL"), a related party (see Note 14), which

provided for aggregate borrowings of up to $2.8 million to finance construction of the Company's biology and chemistry laboratories. Borrowings under the CRL Construction Loan bear interest at a fixed rate equal to 6.0% per annum and interest accrues monthly. The CRL Construction Loan requires monthly payments of principal and interest commencing on January 1, 2011 and through the maturity date of January 1, 2026, based on a 15-year straight-line amortization schedule.

The CRL Construction Loan is collateralized by a security interest in all of the equipment and fixtures at the Company's laboratories in Lawrence, Kansas. Under the loan and security agreements, the Company has agreed to affirmative, negative and financial covenants to which it will remain subject until the loan has been paid off in full. These covenants include limitations on the Company's ability to incur additional indebtedness and engage in certain fundamental business transactions, such as mergers or acquisitions of other businesses, as well as requirements that the Company comply with a maximum liabilities-to-assets ratio, a minimum working capital threshold and a maximum debt-to-equity ratio. Events of default under the loan agreement include failure to make payments when due, insolvency events, failure to comply with covenants and material adverse effects with respect to the Company. The lender's remedies upon an event of default include the ability to accelerate all amounts that are due under the CRL Construction Loan to become immediately due and payable. As of December 31, 2017 and 2016, the Company was in compliance with the financial covenants of the CRL Construction Loan.

Notes payable to related party as of December 31, 2017 and 2016 consisted only of outstanding borrowings under the CRL Construction Loan, as follows (in thousands):

	December 31,	
	2017	2016
Notes payable to related party	$1,481	$1,668
Less: Current portion	(187)	(187)
Notes payable to related party, net of current portion	$1,294	$1,481

As of December 31, 2017, scheduled payments of principal and interest for the CRL Construction Loan are as follows (in thousands):

Year ending December 31,	Principal	Interest	Total
2018	$ 187	$ 84	$ 271
2019	187	73	260
2020	187	61	248
2021	187	50	237
2022	187	39	226
Thereafter	546	49	595
	$ 1,481	$ 356	$1,837

Total interest expense for each of the years ended December 31, 2017, 2016 and 2015 was $0.1 million, $0.1 million and $2.2 million, respectively.

7. Convertible Preferred Shares

2015 Recapitalization

During 2015, the Company amended its operating agreement to affect a recapitalization of its members' interests into three classes of shares: common shares, Series A Shares and Series B Shares. As a result of this recapitalization, the previously outstanding member units became 202,500 Series A Shares.

Series B Preferred Shares Purchase Agreement

In September 2015, the Company entered into a Series B preferred shares purchase agreement, which provided for total gross cash proceeds of up to $90.6 million, comprised of two tranches: a Series B-1 Share financing for total gross proceeds of $31.6 million (the "Series B-1 Share Financing") and a Series B-2 Share financing for total gross cash proceeds of $55.3 million (the "Series B-2 Share Financing").

In connection with the Series B preferred shares purchase agreement, in September 2015, the Company amended its operating agreement such that each member of the LLC would convert debt held by such member issued prior to January 1, 2015 into Series A Shares at their applicable conversion prices and that each member of the LLC would convert debt held by such member issued after January 1, 2015 into Series B-1 Shares at their applicable conversion prices. At that time, an aggregate of $95.6 million of outstanding principal and interest under the Company's 2004 Loan and the Pre-2015 Convertible Loans, with a carrying value of $94.1 million, was converted into 1,855,250 Series A Shares. Additionally, $3.7 million of outstanding principal and accrued interest under the Company's 2015 Loan was converted into 73,811 Series B-1 Shares.

In connection with the Series B-1 Share Financing, in September 2015 and December 2015, the Company sold an aggregate of 624,784 Series B-1 Shares at a price of $50.50 per share for gross proceeds of $31.6 million. The Company recorded issuance costs of $0.5 million in connection with the sale and issuance of the Series B-1 Shares. Purchasers of Series B-1 Shares also agreed to purchase an aggregate of 876,366 Series B-2 Shares at a price of $63.13 per share upon the achievement by the Company of one of two clinical development milestones and the certification by the Company's board of directors that the milestone(s) had occurred. In addition, the purchasers of Series B-1 Shares were granted the right to purchase their respective allocation of Series B-2 Shares at any time more than 45 days from the initial closing and before the Company completed its next financing of more than $5.0 million. In July 2016, upon notice from the Company of the achievement of one of the required clinical milestones, the Company issued and sold 876,366 Series B-2 Shares at a price of $63.13 per share for gross proceeds of $55.3 million. Issuance costs associated with the issuance of Series B-2 Shares were less than $0.1 million. The Company determined that the future tranche obligation of the Series B preferred shares purchase agreement did not meet the definition of a freestanding financial instrument because, while separately exercisable, it was not legally detachable. Further, the Company determined that the embedded future tranche obligation did not require bifurcation for accounting purposes as it is clearly and closely related to the economic characteristics and risks of the initial preferred shares and would not meet the definition of a derivative on a standalone basis.

Series C Preferred Shares Purchase Agreement

In May 2017, the Company entered into a Series C preferred shares purchase agreement, pursuant to which the Company sold 690,333 Series C Shares at a price of $75.76 per share for proceeds of $51.9 million, net of issuance costs of $0.4 million.

Upon issuance of each class of Preferred Shares, the Company assessed the embedded conversion and liquidation features of the securities and determined that such features did not require the Company to separately account for these features. The Company also concluded that no beneficial conversion feature existed upon the issuance date of each class of Preferred Shares.

As of December 31, 2016, the Preferred Shares consisted of the following (in thousands, except share amounts):

	Preferred Shares Authorized	Preferred Shares Issued and Outstanding	Carrying Value	Liquidation Preference	Common Shares Issuable Upon Conversion(1)
Series A Shares	2,057,750	2,057,750	$102,556	$ 103,484	11,626,287
Series B-1 Shares	698,595	698,595	34,812	35,279	3,947,060
Series B-2 Shares	876,366	876,366	55,299	55,325	4,951,467
	3,632,711	3,632,711	$192,667	$ 194,088	20,524,814

(1) Amounts reflect the exchange of convertible preferred shares into shares of common stock on a one-for 5.65 basis upon the Conversion described in Note 1.

Upon closing of the IPO in October 2017, all of the outstanding Preferred Shares including the Series C issued in May 2017 were converted into 24,425,190 shares of common stock.

Conversion

On October 2, 2017, immediately prior to the completion of the IPO, the Company engaged in a series of transactions whereby Deciphera Pharmaceuticals, LLC became a wholly owned subsidiary of Deciphera Pharmaceuticals, Inc. As part of the transactions, shareholders of Deciphera Pharmaceuticals, LLC exchanged their shares of Deciphera Pharmaceuticals, LLC for shares of Deciphera Pharmaceuticals, Inc. on a one-for-5.65 basis and exchanged their outstanding equity incentive awards of Deciphera Pharmaceuticals, LLC for options to purchase the same number of shares of common stock of Deciphera Pharmaceuticals, Inc. multiplied by 5.65, with a corresponding adjustment to divide the exercise price by 5.65 (the "Conversion"). The Conversion included the exchange of all outstanding series A, series B and series C preferred shares of Deciphera Pharmaceuticals, LLC for an aggregate of 24,425,190 shares of common stock of Deciphera Pharmaceuticals, Inc. and the exchange of all outstanding options and share appreciations rights of Deciphera Pharmaceuticals, LLC for 4,092,710 options to purchase common stock of Deciphera Pharmaceuticals, Inc. with a weighted average exercise price of $3.37 per share.

Prior to the Conversion, the holders of the Preferred Shares had the following rights and preferences:

Voting Rights

The holders of Preferred Shares were entitled to vote, together with the holders of common shares, on all matters submitted to shareholders for a vote. Each preferred shareholder was entitled to the number of votes equal to the number of common shares into which each Preferred Share was convertible at the time of such vote.

Dividends and Distributions

There were no stated dividends on the Preferred Shares, however, holders of Preferred Shares were entitled to receive distributions when, as and if approved by the board of directors of the Company and together with holders of common shares in proportion to the number of common shares into which each Preferred Share was convertible at the time of such distribution. Additionally, to the extent the Company had sufficient cash available, without incurring any borrowings, the Company should make a tax distribution in an amount of cash equal to the excess, if any, of (i) the product of the net taxable income or gain of the Company for the year allocated to each member and the highest combined marginal federal, state and local income tax rate applicable to residents in Kansas over (ii) the aggregate amount of distributions previously made to such member during such taxable year.

Through December 31, 2017, no distributions had been approved or paid.

Liquidation

In the event of any liquidation, dissolution or winding-up of the Company or Liquidating Event (as described below), the holders of Preferred Shares were entitled to be paid out of the assets of the Company available for distribution to shareholders in the order and preference as specified in the Company's certificate of incorporation, as amended and restated.

Unless the holders of a majority of the outstanding Preferred Shares, voting together as a single class, elect otherwise, a Liquidating Event should include a merger or consolidation (other than one in which shareholders of the Company own a majority of the voting power of the outstanding shares of the surviving or acquiring corporation) or a sale, lease, transfer, exclusive license or other disposition of all or substantially all of the assets of the Company.

Conversion

Each Preferred Share was convertible at the option of the shareholder at any time after the date of issuance, and automatically converted into shares of common stock upon the closing of the Company's IPO in October 2017 on a one-for-5.65 basis.

8. Common Stock

On October 2, 2017, the Company's certificate of incorporation, as amended and restated, authorized the Company to issue up to 125,000,000 shares of common stock and 5,000,000 shares of preferred stock, each with a par value of $0.01 per share.

As of December 31, 2016, the Company's operating agreement, as amended and restated, authorized the Company to issue 4,366,052 shares of no par value common shares. Common shares shall be issued in one or more series as determined by the board of directors of the Company at the time of issuance. Each common share entitled the holder to one vote on all matters submitted to a vote of the Company's shareholders.

9. Stock-Based Compensation

2017 Equity Incentive Plan

On September 21, 2017, the Company adopted the 2017 Stock Option and Incentive Plan (the "2017 Plan") which became effective on September 26, 2017. The 2017 Plan provides for the grant of equity-based incentive awards. The Company initially reserved 2,655,831 shares of common stock for the issuance of awards under the 2017 Plan. The 2017 Plan provides that the number of shares reserved and available for issuance under the plan will automatically increase each January 1, beginning on January 1, 2018, by 4% of the outstanding number of shares of our common stock on the immediately preceding December 31 or such lesser number of shares as determined by the Compensation Committee of the Company's Board of Directors. As of December 31, 2017, 2,150,189 remained available for issuance under the 2017 Plan. The number of shares reserved for issuance under the 2017 Plan was increased by 1,303,667 shares effective January 1, 2018.

2017 Employee Stock Purchase Plan

On September 21, 2017, the Company adopted the 2017 Employee Stock Purchase Plan, (the "ESPP") which became effective on September 26, 2017. The ESPP initially reserves and authorizes the issuance of up to a total of 306,750 shares of common stock to participating employees. The ESPP provides that the number of shares reserved and available for issuance will automatically increase each January 1, beginning on January 1, 2018 and each January 1 thereafter through January 1, 2027, by the least of (i) 1% of the outstanding number of shares of common stock on the immediately preceding December 31; (ii) 400,000 shares or (iii) such number of

shares as determined by the ESPP administrator. The number of shares reserved for issuance under the ESPP was increased by 325,916 shares effective January 1, 2018.

As of December 31, 2017, no offering periods have commenced under the ESPP.

2015 Equity Incentive Plan

Under the Company's 2015 Equity Incentive Plan (the "2015 Plan") the Company was authorized to sell or issue common shares or restricted common shares, or to grant options for the purchase of common shares, share appreciation rights (SARs) and other awards, to employees, members of the board of directors, consultants and advisors of the Company. Upon effectiveness of the 2017 Plan no further awards were available to be issued under the 2015 Plan.

Both the 2017 and 2015 Plans provide that they be administered by the board of directors or, at the discretion of the board of directors, by a committee of the board of directors. The exercise prices for stock options may not be less than 100% of the fair market value of the common stock on the date of grant and the term of awards may not be greater than ten years. The Company bases fair value of common stock on the quoted market price. Prior to the IPO, the Company valued its common shares by taking into consideration its most recently available third-party valuations of common shares performed under the direction of the board of directors as well as additional factors which may have changed since the date of the most recent contemporaneous valuation through the date of grant. Vesting periods are determined at the discretion of the board of directors. Awards granted to employees and directors typically vest over four years.

Conversion

In connection with the Conversion all outstanding options and share appreciation rights of Deciphera Pharmaceuticals, LLC were exchanged for options to purchase common stock of Deciphera Pharmaceuticals, Inc. Option and share appreciation amounts for periods prior to the Company's IPO have been retrospectively adjusted to give effect to this exchange.

Stock Option Valuation

The fair value of each stock-based award is estimated on the date of grant using the Black-Scholes option-pricing model, which uses as inputs the fair value of the Company's common stock and assumptions for the volatility of its common stock, the expected term of stock-based awards, the risk-free interest rate for a period that approximates the expected term of stock-based awards and the expected dividend yield. Prior to October 2017, the Company was privately-held and lacked company-specific historical and implied volatility information. Therefore, it estimates its expected share volatility based on the historical volatility of a set of publicly traded peer companies and expects to continue to do so until such time as it has adequate historical data regarding the volatility of its own traded stock price. The Company estimated the expected term of its options using the "simplified" method for awards that qualify as "plain-vanilla" options. For options that do not qualify as "plain-vanilla", the Company estimated the expected term using the average of vesting date and expiration date as it believes there is no better estimate of expected term. The risk-free interest rate is determined by reference to the U.S. Treasury yield curve in effect at the time of grant of the award for time periods approximately equal to the expected term of the award. Expected dividend yield is based on the fact that the Company has never paid cash dividends on common shares and does not expect to pay any cash dividends in the foreseeable future.

The assumptions that the Company used to determine the fair value of options granted to employees and directors were as follows, presented on a weighted average basis:

	Year Ended December 31,		
	2017	2016	2015
Risk-free interest rate	1.9%	1.3%	2.0%
Expected term (in years)	6.0	6.1	7.4
Expected volatility	78.3%	76.1%	86.6%
Expected dividend yield	0%	0%	0%

The following table summarizes the Company's option activity from January 1, 2017 to December 31, 2017:

	Number of Shares	Weighted Average Exercise Price	Weighted Average Remaining Contractual Term (in years)	Aggregate Intrinsic Value (in thousands)
Outstanding as of December 31, 2016	3,214,452	$ 2.43	9.2	$ 4,890
Granted	1,553,639	$ 10.02		
Exercised	—	—		
Forfeited	(169,739)	$ 2.58		
Outstanding as of December 31, 2017	4,598,352	$ 4.99	8.6	$ 81,311
Options vested and expected to vest as of December 31, 2017	4,598,352	$ 4.99	8.6	$ 81,311
Options exercisable as of December 31, 2017	2,436,866	$ 3.21	8.2	$ 47,431

The aggregate intrinsic value of options is calculated as the difference between the exercise price of the options and the fair value of the Company's common shares for those options that had exercise prices lower than the fair value of the Company's common shares.

The weighted average grant-date fair value per share of options granted during the years ended December 31, 2017, 2016 and 2015 was $7.63, $2.43 and $1.45, respectively. The total fair value of options vested during the years ended December 31, 2017, 2016 and 2015 was $3.6 million, $0.8 million, and $1.9 million, respectively.

Stock-Based Compensation

Stock-based compensation expense was classified in the statements of operations and comprehensive loss as follows (in thousands):

	Year Ended December 31,		
	2017	2016	2015
Research and development expenses	$1,320	$ 541	$1,382
General and administrative expenses	3,546	946	1,175
	$4,866	$1,487	$2,557

As of December 31, 2017, total unrecognized compensation cost related to the unvested share-based awards was $8.8 million, which is expected to be recognized over a weighted average of 1.7 years.

During the year ended December 31, 2017, the Company granted 208,000 options, of which 115,918 options were immediately vested and the remaining 92,082 options vest over a weighted average period of 1.8 years.

10. Net Loss per Share

On October 2, 2017, in connection with the closing of the IPO, all outstanding convertible preferred shares of Deciphera Pharmaceuticals, LLC were exchanged for shares of common stock of Deciphera Pharmaceuticals, Inc. upon the Conversion (see Note 1). In addition, because the Company had a net loss in each of the periods presented, diluted net loss per share attributable to common shareholders has not been presented as the effect of including common share equivalents in the calculations would have had an anti-dilutive impact.

The Company did not have any common shares outstanding during the years ended December 31, 2015 and 2016 or during the period from January 1, 2017 through the closing of its initial public offering on October 2, 2017. Because the Series A convertible preferred shares represented the most subordinate share class outstanding during those periods, in determining weighted average common shares outstanding for purposes of calculating net loss per share for periods prior to the IPO, the Company utilized Series A convertible preferred shares. Such shares have also been retrospectively adjusted to give effect to the exchange of Series A convertible preferred shares into shares of common stock upon the Conversion.

Basic and diluted net loss per share was calculated as follows (in thousands, except share and per share amounts):

	Year Ended December 31,		
	2017	2016	2015
Numerator:			
Net loss	$ (50,284)	$ (25,940)	$ (19,816)
Denominator:			
Weighted average common shares outstanding—basic and diluted	16,792,179	11,626,287	4,245,698
Net loss per share —basic and diluted	$ (2.99)	$ (2.23)	$ (4.67)

Common Stock Equivalents

The following potential dilutive securities, presented based on amounts outstanding at the end of each reporting period, have been excluded from the calculation of diluted net loss per share because including them would have had an anti-dilutive impact:

	December 31,		
	2017	2016	2015
Series B Shares (as converted to common shares)(1)	—	8,898,527	3,947,060
Options to purchase common stock(1)	4,598,352	3,214,452	2,241,679
	4,598,352	12,112,979	6,188,739

(1) Adjusted for the Conversion as described in Note 1

11. Commitments and Contingencies

Leases

The Company has a three-year sublease agreement for office space in Waltham, Massachusetts that began in September 2016 and expires in September 2019. Prior to this lease, the Company had a lease agreement for office space in Waltham, Massachusetts that expired in September 2016.

The Company has two five-year lease agreements for office and laboratory space in Lawrence, Kansas that began on January 1, 2016 and expire on December 31, 2020. During 2017, the Company entered into two leases for additional office space in Lawrence, Kansas, that will expire in December 2020, with annual payments due of $0.1 million.

Payment escalations specified in the lease agreements are accrued, and rent expense is recognized on a straight-line basis over the terms of occupancy.

The Company recorded rent expense of $0.6 million, 0.4 million and $0.2 million during the years ended December 31, 2017, 2016 and 2015, respectively.

The following table summarizes the future minimum lease payments due under the operating leases as of December 31, 2017 (in thousands):

Year Ending December 31,	
2018	$ 675
2019	578
2020	374
	$1,627

KBA Grants

Prior to 2014, the Company received funding from two research and development grants from the KBA totaling $2.0 million. As of December 31, 2013, no further amounts will be received under these grants. Pursuant to Kansas law, the Company may be required to repay some or all of the financial assistance received from the KBA, subject to the discretion of the KBA, if the Company relocates the operations in which the KBA invested outside of the State of Kansas, if the Company initiates procedures to dissolve and wind up or cease operations within ten years after receiving such financial assistance, or upon certain significant changes to ownership of the Company. The Company will only account for the repayment of the grants if it becomes probable that the Company will be required to repay any funds previously received.

Legal Proceedings

The Company is not currently a party to any material legal proceedings. At each reporting date, the Company evaluates whether or not a potential loss amount or a potential range of loss is probable and reasonably estimable under the provisions of the authoritative guidance that addresses accounting for contingencies. The Company expenses the costs related to its legal proceedings as they are incurred.

Indemnification Agreements

In the ordinary course of business, the Company may provide indemnification of varying scope and terms to vendors, lessors, business partners and other parties with respect to certain matters including, but not limited to, losses arising out of breach of such agreements or from intellectual property infringement claims made by third parties. In addition, the Company has entered into indemnification agreements with members of its board of directors and senior management that will require the Company, among other things, to indemnify them against certain liabilities that may arise by reason of their status or service as directors or officers. The maximum potential amount of future payments the Company could be required to make under these indemnification agreements is, in many cases, unlimited. To date, the Company has not incurred any material costs as a result of such indemnifications. The Company is not aware of any claims under indemnification arrangements, and it has not accrued any liabilities related to such obligations in its consolidated financial statements as of December 31, 2017 or 2016.

12. Income Taxes

Prior to the Conversion, the Company has been treated as a partnership for tax purposes and has not been subject to U.S. federal or state income taxation. As a result, since its inception, the Company has not recorded any U.S. federal or state income tax benefits for the net losses incurred in each year or for earned research and orphan drug credits. Upon the Conversion in October 2017, the Company became subject to Corporate U.S. federal and state income taxes.

During the period from October 2, 2017 to December 31, 2017, the Company recorded no income tax benefits for the net operating losses, due to its uncertainty of realizing a benefit from those items.

A reconciliation of the U.S. federal statutory income tax rate to the Company's effective income tax rate is as follows:

	Year Ended December 31, 2017
Federal statutory income tax rate	34.0%
State taxes, net of federal benefit	1.6
Federal research and orphan drug credit	7.7
Federal research and orphan drug credit addback	(6.6)
Impact of change in tax status	(8.7)
Effect of federal tax law change	(6.1)
Permanent adjustments	(0.3)
Increase in the valuation allowance	(21.6)
Effective income tax rate	— %

Net deferred tax assets as of December 31, 2017 consisted of the following (in thousands):

	December 31, 2017
Deferred tax assets:	
Net operating loss carryforwards	$ 4,030
Research and orphan drug credit carryforwards	3,888
Stock-based compensation	2,366
Accrued expenses	672
Other	(121)
Total gross deferred tax assets	10,835
Valuation allowance	(10,835)
Net deferred tax assets	$ —

On December 22, 2017, the Tax Cuts and Jobs Act (the "TCJA") was signed into United States law. The TCJA includes a number of changes to existing tax law, including, among other things, a permanent reduction in the federal corporate income tax rate from a top marginal rate of 35% to a flat rate of 21%, effective as of January 1, 2018, as well as limitation of the deduction for net operating losses to 80% of annual taxable income and elimination of net operating loss carrybacks, in each case, for losses arising in taxable years beginning after December 31, 2017 (though any such net operating losses may be carried forward indefinitely).

The Company remeasured certain deferred tax assets and liabilities based on the rates at which they are expected to reverse in the future. The provisional amount related to the re-measurement of the Company's deferred tax balance was a reduction of $3.1 million. Due to the corresponding valuation allowance fully offsetting deferred taxes, there was no impact to the statement of operations and comprehensive loss.

In December 2017, the SEC staff issued Staff Accounting Bulletin No. 118, Income Tax Accounting Implications of the Tax Cuts and Jobs Act (SAB 118) which allows the Company to record provisional amounts during a measurement period not to extend beyond one year of the enactment date. Since the Act was passed late in the fourth quarter of 2017, and ongoing guidance and accounting interpretation are expected over the next 12 months, the Company considers the accounting for the effect of the TCJA to be provisional in accordance with SAB 118.

Upon the Conversion in October 2017, the Company became subject to U.S. federal and state income taxes. The change in the valuation allowance was as follows (in thousands):

	Year Ended December 31, 2017
Valuation allowance as of beginning of year	$ —
Decreases recorded as benefit to income tax provision	—
Increases recorded to income tax provision	(10,835)
Valuation allowance as of end of year	$ (10,835)

As of December 31, 2017, the Company had net operating loss carryforwards for federal and state income tax purposes of $14.9 million and $15.2 million, respectively, which begin to expire in 2037 and 2027, respectively. As of December 31, 2017, the Company also had available research and orphan drug credit carryforwards for federal and state income tax purposes of $3.8 million and less than $0.1 million, respectively, which begin to expire in 2037 and 2032, respectively. Utilization of the net operating loss carryforwards and research and orphan drug credit carryforwards may be subject to a substantial annual limitation under Section 382 of the Internal Revenue Code of 1986, as amended, (the “Code”), and similar state law due to ownership changes that have occurred previously or that could occur in the future. These ownership changes may limit the amount of carryforwards that can be utilized annually to offset future taxable income. The Company has not conducted a formal study to assess whether a change of control has occurred or whether there have been multiple changes of control since the IPO due to the significant complexity and cost associated with such a study. If the Company has experienced a change of control, as defined by Section 382 of the Code and similar state law, at any time since the IPO, utilization of the net operating loss carryforwards or research and orphan drug credit carryforwards may be subject to an annual limitation under Section 382 of the Code, which is determined by first multiplying the value of the Company’s stock at the time of the ownership change by the applicable long-term tax-exempt rate, and then could be subject to additional adjustments, as required. Any limitation may result in expiration of a portion of the net operating loss carryforwards or research and orphan drug credit carryforwards before utilization.

The Company has evaluated the positive and negative evidence bearing upon its ability to realize the deferred tax assets. Management has considered the Company’s history of cumulative net losses incurred since inception and its lack of commercialization of any products since inception and has concluded that it is more likely than not that the Company will not realize the benefits of the deferred tax assets. Accordingly, a full valuation allowance has been established against the deferred tax assets as of December 31, 2017. Management reevaluates the positive and negative evidence at each reporting period.

The Company has not recorded any amounts for unrecognized tax benefits as of December 31, 2017.

The Company files tax returns as prescribed by the tax laws of the jurisdictions in which it operates. In the normal course of business, the Company is subject to examination by federal and state jurisdictions, where applicable. There are currently no pending income tax examinations. The Company’s tax years that are open

under statute are from October 2, 2017 to the present. The Company's policy is to record interest and penalties related to income taxes as part of its income tax provision.

13. 401(k) Savings Plan

The Company has a defined contribution plan under Section 401(k) of the Internal Revenue Code that is managed by CRL, a related party (the "2015 401(k) Plan"). Under the 2015 401(k) Plan, the Company provides matching contributions up to 50% of actual dollars contributed, not to exceed a maximum of 4% of gross wages, subject to certain time-based vesting requirements. Total employer matching contributions related to the 2015 401(k) Plan were $0.1 million the year ended December 31, 2017 and less than $0.1 million for each of the years ended December 31, 2016 and 2015. Effective January 1, 2017, the matching contribution limit was increased to up to 50% of actual dollars contributed, not to exceed a maximum of 6% of gross wages.

14. Related Parties

Clinical Reference Laboratory, Inc.

One of the members of the Company's board of directors is the Chief Executive Officer of CRL. CRL is the owner of approximately 31% of Brightstar, a holder of more than 5% of the Company's common stock.

The Company is a party to a loan agreement and a security agreement, each dated as of June 11, 2010, with CRL. The Company borrowed an aggregate of $2.8 million under the loan agreement to finance improvements to the Company's biology and chemistry laboratories in Lawrence, Kansas. In December 2016, the loan was assigned to CHC, Inc., a related party, which owns 100% of CRL. Borrowings under the loan bear interest at a fixed rate equal to 6.0% per annum and the Company is required to make monthly payments of principal and interest, based on a 15-year straight-line amortization schedule. For each of the years ended December 31, 2017, 2016 and 2015, the Company recorded $0.1 million of interest expense related to this loan. For each of the years ended December 31, 2017, 2016 and 2015, the Company made $0.3 million in principal and interest payments under the loan. As of December 31, 2017 and 2016, principal amounts owed under the loan agreement totaled $1.5 million and $1.7 million, respectively (see Note 6).

The Company is party to a master services agreement, effective as of May 20, 2013, with CRL under which the Company purchased and expects to continue to purchase laboratory services. Under the agreement, the Company has agreed to use CRL on an exclusive basis for laboratory testing needs. For the years ended December 31, 2017, 2016 and 2015, the Company recorded $0.4 million, $0.2 million and $0.1 million, respectively, of research and development expense incurred under this agreement, of which $0.4 million, $0.1 million and less than $0.1 million, respectively, were paid to CRL during those same periods. As of December 31, 2017 and 2016, total amounts owed to CRL for laboratory services were $0.1 million and less than $0.1 million, respectively, which amounts were included in accounts payable and accrued expenses. The Company is not committed to purchase any minimum amounts under the agreement.

In 2015, the Company entered into an agreement with CRL under which the Company became a participating employer in CRL's 401(k) plan. For each of the years ended December 31, 2017, 2016 and 2015, the total amount of contributions made by employees of the Company under the plan was $0.6 million, $0.2 million and $0.2 million, respectively.

15. Selected Quarterly Financial Data (Unaudited)

The following information has been derived from unaudited consolidated financial statements that, in the opinion of management, include all recurring adjustments necessary for a fair statement of such information. (in thousands) except per share data:

	Three Months Ended							
	Dec. 31, 2017	Sept. 30, 2017	June 30, 2017	March 31, 2017	Dec. 31, 2016	Sept. 30, 2016	June 30, 2016	March 31, 2016
Statements of Operations Data:								
Revenue	$ —	$ —	$ —	$ —	$ —	$ —	$ —	$ —
Loss from operations	(20,338)	(12,181)	(10,691)	(7,725)	(8,534)	(6,067)	(5,913)	(5,324)
Net loss	(19,912)	(12,038)	(10,626)	(7,708)	(8,557)	(6,099)	(5,932)	(5,352)
Net loss per share—basic and diluted(1)	(0.62)	(1.04)	(0.91)	(0.66)	(0.74)	(0.52)	(0.51)	(0.46)

(1) The Company did not have any common shares outstanding for all periods through September 30, 2017. For purposes of calculating net loss per share, amounts for periods prior to the Company's IPO have been retrospectively adjusted to give effect to the exchange of Series A convertible preferred shares into shares of common stock upon the Conversion described in Note 1 because series A preferred shares represent the most subordinated share class outstanding during those periods.

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

Our management, with the participation of our Chief Executive Officer and Chief Financial Officer (our principal executive officer and principal financial officer, respectively), evaluated the effectiveness of our disclosure controls and procedures as of December 31, 2017. The term "disclosure controls and procedures," as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act means controls and other procedures of a company 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 SEC'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.

Management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Based on the evaluation of our disclosure controls and procedures as of December 31, 2017, our Chief Executive Officer and Chief Financial Officer concluded that, as of such date, our disclosure controls and procedures were effective at the reasonable assurance level.

Internal Control Over Financial Reporting

Management's Report on Internal Control Over Financial Reporting

This Annual Report on Form 10-K does not include a report of management's assessment regarding internal control over financial reporting or an attestation report of our independent registered public accounting firm due to a transition period established by rules of the SEC for newly public companies.

Changes in Internal Control Over Financial Reporting

No change in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) occurred during the three months ended December 31, 2017 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 this Item 10 will be included in our definitive proxy statement to be filed with the SEC with respect to our 2018 Annual Meeting of Stockholders and is incorporated herein by reference.

ITEM 11. EXECUTIVE COMPENSATION

The information required by this Item 11 will be included in our definitive proxy statement to be filed with the SEC with respect to our 2018 Annual Meeting of Stockholders and is incorporated herein by reference.

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

The information required by this Item 12 will be included in our definitive proxy statement to be filed with the SEC with respect to our 2018 Annual Meeting of Stockholders and is incorporated herein by reference.

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

The information required by this Item 13 will be included in our definitive proxy statement to be filed with the SEC with respect to our 2018 Annual Meeting of Stockholders and is incorporated herein by reference.

ITEM 14. PRINCIPAL ACCOUNTING FEES AND SERVICES

The information required by this Item 14 will be included in our definitive proxy statement to be filed with the SEC with respect to our 2018 Annual Meeting of Stockholders and is incorporated herein by reference.

PART IV

ITEM 15. EXHIBITS, FINANCIAL STATEMENT SCHEDULES

(a) 1. *Financial Statements*

For a list of the financial statements included herein, see Index to the Consolidated Financial Statements on page 120 of this Annual Report on Form 10-K, incorporated into this Item by reference.

2. *Financial Statement Schedules*

Financial statement schedules have been omitted because they are either not required or not applicable or the information is included in the consolidated financial statements or the notes thereto.

3. *Exhibits*

See the Exhibit Index in Item 15(b) below.

(b) **Exhibit Index.**

Exhibit Number	Description
2.1*	Reorganization Agreement and Plan of Merger by and among the Registrant, Deciphera Pharmaceuticals, LLC and the other parties named therein, dated October 2, 2017 (Incorporated by reference to Exhibit 2.1 to the Registrant's Quarterly Report on Form 10-Q filed on November 14, 2017)(1)
3.1*	Amended and Restated Certificate of Incorporation of the Registrant (Incorporated by reference to Exhibit 3.1 to the Registrant's Current Report on Form 8-K filed on October 5, 2017).
3.2*	Amended and Restated Bylaws of the Registrant (Incorporated by reference to Exhibit 3.2 to the Registrant's Current Report on Form 8-K filed on October 5, 2017).
4.1*	Specimen Common Stock Certificate (Incorporated by reference to Exhibit 4.1 to Amendment No. 3 to the Registrant's Registration Statement on Form S-1 filed on September 22, 2017).
4.2*	Second Amended and Restated Investors' Rights Agreement among Deciphera Pharmaceuticals, LLC and certain of its shareholders, dated May 26, 2017 (Incorporated by reference to Exhibit 4.2 to Amendment No. 1 to the Registrant's Registration Statement on Form S-1 filed on September 11, 2017).
4.3*	Registration Rights Agreement by and among the Registrant and certain of its stockholders, dated October 2, 2017 (Incorporated by reference to Exhibit 4.1 to the Registrant's Quarterly Report on Form 10-Q filed on November 14, 2017).
10.1#*	2015 Equity Incentive Plan, as amended, and form of award agreements thereunder (Incorporated by reference to Exhibit 10.1 to Amendment No. 2 to the Registrant's Registration Statement on Form S-1 filed on September 18, 2017).
10.2#*	2017 Stock Option and Incentive Plan and form of award agreements thereunder (Incorporated by reference to Exhibit 10.2 to Amendment No. 3 to the Registrant's Registration Statement on Form S-1 filed on September 22, 2017).
10.3#*	2017 Employee Stock Purchase Plan (Incorporated by reference to Exhibit 10.3 to Amendment No. 3 to the Registrant's Registration Statement on Form S-1 filed on September 22, 2017).

Exhibit Number	Description
10.4#*	Form of Indemnification Agreement between Deciphera Pharmaceuticals, Inc. and each of its directors (Incorporated by reference to Exhibit 10.4 to Amendment No. 3 to the Registrant's Registration Statement on Form S-1 filed on September 22, 2017).
10.5#*	Form of Indemnification Agreement between Deciphera Pharmaceuticals, Inc. and each of its executive officers (Incorporated by reference to Exhibit 10.5 to Amendment No. 3 to the Registrant's Registration Statement on Form S-1 filed on September 22, 2017).
10.6#*	Employment Agreement, between Deciphera Pharmaceuticals, LLC and Michael D. Taylor, Ph.D. (Incorporated by reference to Exhibit 10.6 to Amendment No. 4 to the Registrant's Registration Statement on Form S-1 filed on September 25, 2017).
10.7#*	Employment Agreement, between Deciphera Pharmaceuticals, LLC and Christopher J. Morl (Incorporated by reference to Exhibit 10.7 to Amendment No. 4 to the Registrant's Registration Statement on Form S-1 filed on September 25, 2017).
10.8#*	Employment Agreement, between Deciphera Pharmaceuticals, LLC and Oliver Rosen, M.D. (Incorporated by reference to Exhibit 10.8 to Amendment No. 4 to the Registrant's Registration Statement on Form S-1 filed on September 25, 2017).
10.9#*	Deciphera Pharmaceuticals, Inc. Non-Employee Director Compensation Policy (Incorporated by reference to Exhibit 10.10 to Amendment No. 3 to the Registrant's Registration Statement on Form S-1 filed on September 22, 2017).
10.10#*	Deciphera Pharmaceuticals, Inc. Senior Executive Cash Incentive Bonus Plan (Incorporated by reference to Exhibit 10.11 to Amendment No. 3 to the Registrant's Registration Statement on Form S-1 filed on September 22, 2017).
21.1	List of Subsidiaries of Registrant.
23.1	Consent of PricewaterhouseCoopers LLP, independent registered public accounting firm.
24.1	Power of Attorney (included on signature page to this Annual Report on Form 10-K).
31.1	Certification of Chief Executive Officer of the Registrant Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
31.2	Certification of Chief Financial Officer of the Registrant Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
32.1†	Certification of Chief Executive Officer of the Registrant Pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
32.2†	Certification of Chief Financial Officer of the Registrant Pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
101INS	XBRL Instance Document.
101SCH	XBRL Taxonomy Extension Schema Document.
101CAL	XBRL Taxonomy Extension Calculation Linkbase Document.
101LAB	XBRL Taxonomy Extension Labels Linkbase Document.
101PRE	XBRL Taxonomy Extension Presentation Linkbase Document.
101DEF	XBRL Taxonomy Extension Definition Linkbase Document.

* Previously filed.

\# Indicates management contract or compensation plan.

(1) Schedules and exhibits have been omitted from this filing pursuant to Item 601(b)(2) of Regulation S-K. The registrant agrees to furnish supplementally a copy of any omitted schedule or exhibit to the Securities and Exchange Commission upon its request; provided, however, that the registrant may request confidential

treatment pursuant to Rule 24b-2 of the Securities Exchange Act of 1934, as amended, for any schedule or exhibit so furnished.

† The certifications attached as Exhibits 32.1 and 32.2 that accompany this Annual Report on Form 10-K, are not deemed filed with the Securities and Exchange Commission and are not to be incorporated by reference into any filing of Deciphera Pharmaceuticals, Inc. under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended, whether made before or after the date of this Annual Report on Form 10-K, irrespective of any general incorporation language contained in such filing.

ITEM 16. FORM 10-K SUMMARY

None.

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.

Dated: March 28, 2018

DECIPHERA PHARMACEUTICALS, INC.

By: /s/ Michael D. Taylor, Ph.D.
Michael D. Taylor, Ph.D.
President and Chief Executive Officer

POWER OF ATTORNEY

Each person whose individual signature appears below hereby authorizes and appoints Michael D. Taylor and Thomas P. Kelly, and each of them, with full power of substitution and resubstitution and full power to act without the other, as his or her true and lawful attorney-in-fact and agent to act in his or her name, place and stead and to execute in the name and on behalf of each person, individually and in each capacity stated below, and to file any and all amendments to this annual report on Form 10-K and to file the same, with all exhibits thereto, and other documents in connection therewith, with the Securities and Exchange Commission, granting unto said attorneys-in-fact and agents, and each of them, full power and authority to do and perform each and every act and thing, ratifying and confirming all that said attorneys-in-fact and agents or any of them or their or his substitute or substitutes may lawfully do or cause to be done by virtue thereof.

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/ Michael D. Taylor, Ph.D. Michael D. Taylor, Ph.D.	President, Chief Executive Officer and Director (Principal Executive Officer)	March 28, 2018
/s/ Thomas P. Kelly Thomas P. Kelly	Chief Financial Officer (Principal Financial and Accounting Officer)	March 28, 2018
/s/ Patricia L. Allen Patricia L. Allen	Director	March 28, 2018
/s/ James A. Bristol, Ph.D. James A. Bristol, Ph.D.	Director	March 28, 2018
/s/ Edward J. Benz, Jr., M.D. Edward J. Benz, Jr., M.D.	Director	March 28, 2018
/s/ John R. Martin John R. Martin	Director	March 28, 2018
/s/ Liam Ratcliffe, M.D., Ph.D. Liam Ratcliffe, M.D., Ph.D.	Director	March 28, 2018
/s/ Michael Ross, Ph.D. Michael Ross, Ph.D.	Director	March 28, 2018
/s/ Dennis L. Walsh Dennis L. Walsh	Director	March 28, 2018

Exhibit 21.1

Legal Name	State of Organization
Deciphera Pharmaceuticals, LLC	Delaware
DRAGSA 20 LLC	Delaware
NLV-3 Deciphera, Inc.	Delaware
NLV-G Deciphera, Inc.	Delaware
SVLS-Deciphera, Inc.	Delaware
Redmile Deciphera Holdings, Inc.	Delaware
Deciphera Pharmaceuticals Securities Corporation	Massachusetts

Exhibit 23.1

Consent of Independent Registered Public Accounting Firm

We hereby consent to the incorporation by reference in the Registration Statement on Form S-8 (No. 333- 220866) of Deciphera Pharmaceuticals, Inc. of our report dated March 28, 2018 relating to the financial statements, which appears in this Form 10-K.

/s/ PricewaterhouseCoopers LLP

Boston, Massachusetts
March 28, 2018

Exhibit 31.1

CERTIFICATIONS

I, Michael D. Taylor, certify that:

1. I have reviewed this Annual Report on Form 10-K of Deciphera Pharmaceuticals, Inc.;

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

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

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

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

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

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

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

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

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

Date: March 28, 2018

By: /s/ Michael D. Taylor
Michael D. Taylor
President and Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

CERTIFICATIONS

I, Thomas P. Kelly, certify that:

1. I have reviewed this Annual Report on Form 10-K of Deciphera Pharmaceuticals, Inc.;

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

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

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

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

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

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

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

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

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

Date: March 28, 2018

By: /s/ Thomas P. Kelly
Thomas P. Kelly
Chief Financial Officer
(Principal Financial and Accounting Officer)

Exhibit 32.1

CERTIFICATION PURSUANT TO 18 U.S.C. SECTION 1350,

AS ADOPTED PURSUANT TO

SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Annual Report on Form 10-K of Deciphera Pharmaceuticals, Inc. (the "Company") for the fiscal year ended December 31, 2017 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Michael D. Taylor, President and Chief Executive Officer of the Company, hereby certifies, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that to the best of his knowledge:

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

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

Date: March 28, 2018

By: /s/ Michael D. Taylor
Michael D. Taylor
President and Chief Executive Officer
(Principal Executive Officer)

Exhibit 32.2

CERTIFICATION PURSUANT TO 18 U.S.C. SECTION 1350,

AS ADOPTED PURSUANT TO

SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Annual Report on Form 10-K of Deciphera Pharmaceuticals, Inc. (the "Company") for the fiscal year ended December 31, 2017 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Thomas P. Kelly, Chief Financial Officer of the Company, hereby certifies, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that to the best of his knowledge:

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

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

Date: March 28, 2018

By: /s/ Thomas P. Kelly
Thomas P. Kelly
Chief Financial Officer
(Principal Financial and Accounting Officer)