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, 2019
or

☐ **TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**
For transition period from _____ to _____.
Commission File Number: 001-37841

Kadmon Holdings, Inc.

(Exact name of registrant as specified in its charter)

Delaware	**27-3576929**
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification No.)
450 East 29th Street, New York, NY	**10016**
(Address of principal executive offices)	(Zip Code)

(833) 900-5366
(Registrant's telephone number, including area code)

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

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common stock, par value $0.001 per share	KDMN	The New York Stock Exchange

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 15(d) of the Act. YES ☐ NO ☒

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

Indicate by check mark whether the Registrant has submitted electronically every Interactive Data File required to be submitted 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 whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, a smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company" and "emerging growth company" in Rule 12b-2 of the Exchange Act:

Large accelerated filer ☐ Accelerated filer ☒ Non-accelerated filer ☐ Smaller reporting company ☒ Emerging growth company ☒

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

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

As of June 28, 2019, the last business day of the registrant's most recently completed second fiscal quarter, the aggregate market value of its voting and non-voting common equity held by non-affiliates was approximately $139,954,795 based upon the closing price of the registrant's common stock on June 28, 2019.

The number of shares of the registrant's common stock outstanding as of March 2, 2020 was 159,763,100.

DOCUMENTS INCORPORATED BY REFERENCE

Portions of Kadmon Holdings, Inc.'s definitive proxy statement for the 2020 Annual Meeting of Shareholders are incorporated by reference into Part III of this Annual Report on Form 10-K to the extent stated herein.

Kadmon Holdings, Inc.

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FORWARD-LOOKING STATEMENTS

This Annual Report on Form 10-K contains forward-looking statements. All statements other than statements of historical facts contained in this Annual Report on Form 10-K may be forward-looking statements. Statements regarding our future results of operations and financial position, business strategy and plans and objectives of management for future operations, including, among others, statements regarding future capital expenditures and obligations, are forward-looking statements. In some cases, you can identify forward-looking statements by terms such as “may,” “will,” “should,” “expects,” “plans,” “anticipates,” “could,” “intends,” “targets,” “projects,” “contemplates,” “believes,” “estimates,” “predicts,” “potential” or “continue” or the negative of these terms or other similar expressions.

Forward-looking statements involve known and unknown risks, uncertainties and other important factors that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements. We believe that these factors include, but are not limited to, the following:

- the initiation, timing, progress and results of our preclinical studies and clinical trials, and our research and development programs;
- our ability to advance product candidates into, and successfully complete, clinical trials;
- our reliance on the success of our product candidates;
- the timing or likelihood of regulatory filings and approvals;
- our ability to expand our sales and marketing capabilities;
- the commercialization, pricing and reimbursement of our product candidates, if approved;
- the implementation of our business model, strategic plans for our business, product candidates and technology;
- the scope of protection we are able to establish and maintain for intellectual property rights covering our product candidates and technology;
- our ability to operate our business without infringing, misappropriating or otherwise violating the intellectual property rights and proprietary technology of third parties;
- cost associated with defending or enforcing, if any, intellectual property infringement, misappropriation or other intellectual property violation, product liability and other claims;
- regulatory and governmental policy developments in the United States, Europe and other jurisdictions;
- estimates of our expenses, future revenues, capital requirements and our needs for additional financing;
- the potential benefits of strategic collaboration agreements and our ability to enter into strategic arrangements;
- our ability to maintain and establish collaborations;
- the rate and degree of market acceptance, if any, of our product candidates, if approved;
- developments relating to our competitors and our industry, including competing therapies;
- our ability to effectively manage our anticipated growth;
- our ability to attract and retain qualified employees and key personnel;
- our ability to achieve cost savings and benefits from our efforts to streamline our operations and to not harm our business with such efforts;
- our expectations regarding the period during which we qualify as an emerging growth company under the Jumpstart Our Business Startups Act (the “JOBS Act”);
- statements regarding future revenue, hiring plans, expenses, capital expenditures, capital requirements and share performance;
- litigation, including costs associated with prosecuting or defending pending or threatened claims and any adverse outcomes or settlements not covered by insurance;
- our expected use of cash and cash equivalents and other sources of liquidity;
- the future trading price of the shares of our common stock and the impact of securities analysts’ reports on these prices;
- the future trading price of our investments and our potential inability to sell those securities;
- our ability to apply unused federal and state net operating loss carryforwards against future taxable income; and
- other risks and uncertainties, including those listed under the caption “Risk Factors.”

The forward-looking statements in this Annual Report on Form 10-K are only predictions, and we may not actually achieve the plans, intentions or expectations included in our forward-looking statements. We have based these forward-looking statements largely on our current expectations and projections about future events and financial trends that we believe may affect our business, financial condition and results of operations. Because forward-looking statements are inherently subject to risks and uncertainties, some of which cannot be predicted or quantified, you should not rely on these forward-looking statements as predictions of future events. The events and circumstances reflected in our forward-looking statements may not be achieved or occur and actual results could differ materially from those projected in the forward-looking statements.

PART I

Item 1. Business

Overview

We are a clinical-stage biopharmaceutical company that discovers, develops and delivers transformative therapies for unmet medical needs. Our team has a proven track record of successful drug development and commercialization. Our clinical pipeline includes treatments for immune and fibrotic diseases as well as immuno-oncology therapies. We leverage our multi-disciplinary research and clinical development team members to identify and pursue a diverse portfolio of novel product candidates, both through in-licensing products and employing our small molecule and biologics platforms. We expect to continue to progress our clinical candidates and have further clinical trial events throughout 2020. Below is a brief description of our lead product candidates:

- ***Immune and Fibrotic Diseases.*** We are developing oral small molecule inhibitors of Rho-associated coiled-coil kinase ("ROCK") to treat immune and fibrotic diseases. Research by Kadmon and several academic institutions has demonstrated that inhibition of ROCK can regulate aberrant immune responses and fibrotic processes.

  - ***KD025***. KD025, our most advanced product candidate, is an orally administered, selective small molecule inhibitor of Rho-associated coiled-coil kinase 2 ("ROCK2"). A pivotal study of KD025 is ongoing in patients with chronic graft-versus-host disease ("cGVHD"), a complication that can occur following hematopoietic cell transplantation ("HCT") and that results in multi-organ inflammation and fibrosis. The U.S. Food and Drug Administration ("FDA") has granted Breakthrough Therapy Designation to KD025 for the treatment of cGVHD after failure of two or more prior lines of systemic therapy. The FDA has also granted Orphan Drug Designation to KD025 for the treatment of cGVHD.

    In November 2019, we announced positive topline results from the planned interim analysis of ROCKstar (KD025-213), our pivotal trial evaluating KD025 in patients with cGVHD who have received at least two prior lines of systemic therapy. The trial met the primary endpoint of Overall Response Rate ("ORR") at the interim analysis, which was conducted, as scheduled, two months after completion of enrollment. KD025 showed statistically significant ORRs of 64% with KD025 200 mg once daily (95% Confidence Interval ("CI"): 51%, 75%; $p<0.0001$) and 67% with KD025 200 mg twice daily (95% CI: 54%, 78%; $p<0.0001$). In February 2020, we announced expanded results of the interim analysis of KD025-213, showing that ORRs were consistent across key subgroups, including in patients with four or more organs affected by cGVHD (n=69; 64%) and patients who had no response to their last line of treatment (n=74; 68%). Responses were observed in all affected organ systems, including in organs with fibrotic disease. KD025 has been well tolerated and adverse events have been consistent with those expected in the patient population. Additional secondary endpoints, including duration of response, corticosteroid dose reductions, Failure-Free Survival, Overall Survival and Lee Symptom Scale reductions continue to mature and will be available later in 2020.

    Further, in December 2019, we presented two-year follow-up data from our ongoing Phase 2a clinical trial of KD025 in cGVHD (KD025-208). The data showed continued patient benefit, with an ORR of 65% across all three dose cohorts. Responses were observed in all affected organ systems, including organs with fibrotic disease. Kaplan-Meier median duration of response was 35 weeks. KD025 was well tolerated, with no increased risk of infection observed. Twenty-four percent of the patients in the trial had remained on KD025 therapy for more than one-and-a-half years as of June 30, 2019. Subject to FDA feedback, we intend to submit a New Drug Application for KD025 in the second half of 2020.

    We also initiated a double-blind, placebo-controlled Phase 2 clinical trial of KD025 for the treatment of systemic sclerosis, a life-threatening autoimmune disease characterized by chronic inflammation, fibrosis and vascular damage, in 2019.

  - ***KD045.*** KD045 is a potent, selective, oral inhibitor of ROCK for the treatment of fibrotic diseases and is the lead product candidate from our internal effort to identify and develop next-generation ROCK inhibitors. In mouse models of lung, kidney and liver fibrosis, KD045 demonstrated robust activity, highlighting the therapeutic potential of ROCK inhibition and supporting clinical development of this agent. Investigational New Drug ("IND")-enabling activities of KD045 are currently ongoing.

- ***Immuno-oncology.*** We have a biologics research platform focused on the development of immuno-oncology therapeutics, specifically, IL-15-containing fusion proteins for the treatment of cancer.
  - ***KD033.*** KD033 is an anti-PD-L1/IL-15 fusion protein and is the most advanced product candidate from our IL-15 platform. KD033 significantly inhibited tumor growth in many mouse syngeneic models, including PD-L1-expressing models that are resistant to approved immunotherapies. In these models, KD033 has demonstrated long-lasting responses through the induction of immune system memory. We expect to initiate a clinical trial of KD033 in the first half of 2020.

Our Strategy

Our goal is to develop innovative therapies for significant unmet medical needs. Our key strategies to achieve this goal are listed below:

- ***Advance KD025 for the treatment of immune diseases.*** We are conducting a pivotal trial, ROCKstar (KD025-213), of KD025 in patients with cGVHD who have received at least two prior lines of systemic therapy. As further discussed below, we recently announced positive topline results from the planned interim analysis of the trial. We also initiated a Phase 2 clinical trial of KD025 in systemic sclerosis in 2019.
- ***Develop KD033 for the treatment of cancer.*** We plan to initiate clinical trial of KD033 in the first half of 2020.
- ***Develop KD045 for the treatment of fibrotic diseases.*** We expect to complete our ongoing IND-enabling activities of KD045, our ROCK inhibitor.
- ***Leverage our research platforms to develop new product candidates.*** In addition to KD033 and KD045, we intend to use our small molecule and biologics research capabilities to develop new therapies in the areas of immune disorders, fibrotic diseases and immuno-oncology.

Kadmon Pharmaceuticals is our wholly owned, fully integrated commercial operation. We do not currently depend on commercial revenues from Kadmon Pharmaceuticals to support our non-commercial operations. Our commercial infrastructure, including the regulatory, compliance, quality and chemistry, manufacturing and controls (“CMC”) teams of Kadmon Pharmaceuticals, currently supports the development of our clinical-stage product candidates. We plan to leverage our commercial infrastructure to commercialize our product candidates, if approved.

Our Clinical-Stage Pipeline

Product	Indication	Status
KD025 (ROCK2 inhibitor)	Chronic Graft-Versus-Host Disease (cGVHD)	• Pivotal trial ongoing; study met primary endpoint at interim analysis • 2020: NDA submission planned, subject to FDA feedback
	Systemic Sclerosis	• Phase 2 clinical trial ongoing
KD033 (anti-PD-L1/IL-15 fusion protein)	Immuno-oncology	• 1H 2020: Initiate clinical trial
KD045 (pan-ROCK inhibitor)	Fibrotic Diseases	• IND-enabling activities ongoing

ROCK Inhibitors for Immune and Fibrotic Diseases

ROCK is an “on” switch in cells that regulates cell movement, shape, differentiation and function. Two isoforms exist: ROCK1 and ROCK2, and dysregulation of ROCK is implicated in many chronic diseases. Kadmon’s research has demonstrated that inhibition of ROCK can regulate aberrant immune responses and fibrotic processes. Kadmon’s ROCK portfolio includes a ROCK2-selective inhibitor, KD025, for the treatment of immune diseases, and KD045, a ROCK inhibitor for the treatment of fibrotic diseases.

Kadmon's research has helped define the role of ROCK in the pathogenesis of many diseases, including immune and fibrotic disorders. Specifically, our research has demonstrated that KD025 helps to resolve immune dysregulation by down-regulating pro-inflammatory Th17 cells and increasing regulatory T ("Treg") cells.

ROCK is downstream of major pro-fibrotic mediators and regulates multiple fibrotic processes, including stress fiber formation, myofibroblast activation and pro-fibrotic gene transcription. KD025 has down-regulated key fibrotic processes in preclinical models, including with profibrotic gene transcription, stress fiber formation, myofibroblast activation and collagen deposition.

KD025 Clinical Program

To date, more than 550 subjects have been dosed with KD025 for immune or fibrotic diseases or as healthy volunteers. KD025 has been well tolerated and demonstrated clinical activity.

KD025 for the Treatment of Chronic Graft-Versus-Host Disease

Medical Need: Chronic Graft-Versus-Host Disease

cGVHD is a common complication that can occur following HCT. In cGVHD, transplanted immune cells (graft) attack the patient's cells (host), leading to inflammation and fibrosis in multiple tissues. Approximately 14,000 patients in the United States are living with cGVHD, and approximately 5,000 new patients are diagnosed with cGVHD per year.

KD025 in Chronic Graft-Versus-Host Disease

KD025 has demonstrated clinical activity and tolerability in an ongoing pivotal trial in patients with cGVHD who have received two or more prior lines of systemic therapy (KD025-213, or ROCKstar) as well as in an ongoing Phase 2 clinical trial in patients with steroid-dependent or steroid-refractory cGVHD with one to three prior lines of treatment for the disease (KD025-208).

Ongoing Pivotal Trial of KD025 in Chronic Graft-Versus-Host Disease (ROCKstar (KD025-213))

KD025-213 is an ongoing, open-label pivotal trial of KD025 in adults and adolescents with cGVHD who have received at least two prior lines of systemic therapy. Patients were randomized to receive KD025 200 mg QD or 200 mg BID (63 patients per arm). Either KD025 dose may be considered by the FDA for registration. The primary endpoint is the ORR, defined as the percentage of patients who meet the 2014 National Institutes of Health ("NIH") Consensus Conference overall response criteria of Complete Response (CR) or Partial Response (PR). During a Type C meeting in March 2018, the FDA provided guidance to Kadmon on the design of a pivotal study of KD025 in cGVHD. The FDA granted Breakthrough Therapy Designation to KD025 in cGVHD in October 2018. We plan to continue our dialogue with regulatory authorities throughout 2020 to obtain further guidance on the regulatory pathway to approval for KD025 in cGVHD.

In November 2019, we announced positive topline results from the planned interim analysis of ROCKstar. The trial met the primary endpoint at the interim analysis, which was conducted as scheduled two months after completion of enrollment. KD025 showed statistically significant ORRs of 64% with KD025 200 mg QD (95% CI: 51%, 75%; $p<0.0001$) and 67% with KD025 200 mg BID (95% CI: 54%, 78%; $p<0.0001$). Statistical significance is achieved if the lower bound of the 95% CI of ORR exceeds 30%, which was achieved in both arms of the trial at the interim analysis. While the ORR endpoint was met at the interim analysis, the primary analysis of the KD025-213 study will occur six months after completion of enrollment. Topline data from the primary analysis of KD025-213 are expected in the second quarter of 2020.

In February 2020, we announced expanded results of the interim analysis of KD025-213, showing that ORRs were consistent across key subgroups, including in patients with four or more organs affected by cGVHD (n=69; 64%) and patients who had no response to their last line of treatment (n=74; 68%). Responses were observed in all affected organ systems, including in organs with fibrotic disease. KD025 has been well tolerated and adverse events have been consistent with those expected in the patient population. Additional secondary endpoints, including duration of response, corticosteroid dose reductions, Failure-Free Survival, Overall Survival and Lee Symptom Scale reductions continue to mature and will be available later in 2020.

Ongoing Phase 2a Clinical Trial of KD025 in cGVHD (KD025-208)

KD025-208 is an ongoing Phase 2 clinical trial in patients with steroid-dependent or steroid-refractory cGVHD with one to three prior lines of treatment for the disease. Three cohorts of patients, (KD025 200 mg QD (n=17), KD025 200 mg BID (n=16) and KD025 400 mg QD (n=21)), were enrolled sequentially following a safety assessment of the previous cohort. KD025 achieved an ORR of approximately 65% across all three cohorts. Responses were observed across all affected organ systems, including in organs with fibrotic disease. Responses were durable, with a median duration of response of 35 weeks.

and patients reported improvements in quality of life and were also able to reduce and/or completely discontinue doses of corticosteroids and other immunosuppressants. Pharmacodynamics data showed a decrease in Th17 cells and an increase in Treg cells during KD025 treatment, consistent with KD025 mechanism of action. KD025 was well tolerated, with no treatment-related serious adverse events and no apparent increased risk of infection. Twenty-four percent of the patients in the trial had remained on KD025 therapy for more than one-and-a-half years as of June 30, 2019.

KD025 for the Treatment of Systemic Sclerosis

Medical Need: Systemic Sclerosis

Systemic sclerosis ("SSc") is a life-threatening autoimmune disease characterized by chronic tissue inflammation, fibrosis and vascular damage. SSc affects 75,000 to 100,000 people in the United States. Currently, there are no FDA approved drugs for the treatment of SSc.

KD025 in SSc

In 2019, we initiated a double-blind, placebo-controlled Phase 2 clinical trial of KD025 in SSc, a disease in which KD025 has demonstrated potential in preclinical models. Sixty patients are being randomized to receive KD025 200 mg QD, KD025 200 mg BID or placebo (20 patients per arm) blinded for 28 weeks and open-label for an additional 24 weeks (total 52 weeks). The primary endpoint is the change in Combined Response Index for Systemic Sclerosis (CRISS) score, a measure of improvement in systemic sclerosis, at 24 weeks.

KD045

Kadmon is developing KD045, a next-generation ROCK inhibitor for the treatment of fibrotic diseases. A key challenge in the development of ROCK inhibitors is to develop potent, selective oral therapies. Earlier-generation ROCK inhibitors target the majority of the AGC kinase family and lack specificity or potency to effectively target ROCK. Using innovative medicinal chemistry, computational and structure-based design approaches, we have identified and developed proprietary, next-generation inhibitors with enhanced potency and AGC-kinase selectivity to specifically target ROCK. We have selected our lead candidate from this effort, KD045, for clinical development.

KD045 inhibited key fibrotic processes in multiple *in vivo* pharmacology models, including in models of bleomycin-induced lung fibrosis, renal fibrosis and liver fibrosis. KD045 has been shown to selectively target ROCK, exhibiting a favorable safety profile compared to earlier-generation ROCK inhibitors. IND-enabling studies are ongoing for KD045.

KD033

We have an in-house novel phage display library able to generate fully human monoclonal antibodies against many protein targets. This platform is run by an experienced group of scientists with an outstanding antibody development track record. Prior to joining Kadmon, this team was involved in the development of multiple commercially successful antibodies including Erbitux (cetuximab) and Cyramza (ramucirumab). Our scientists are developing monoclonal antibodies as well as fusion proteins and bispecific antibodies that we believe represent the next generation of cancer immunotherapies.

Our most advanced candidate from the biologics platform, KD033, is a novel anti-PD-L1/IL-15 fusion protein designed to stimulate an immune response directed to the tumor microenvironment. Recombinant IL-15 alone, which stimulates cancer-fighting immune effector cells. We have developed KD033 as a novel approach to overcome this challenge by fusing IL-15 to an anti-PD-L1 antibody to direct IL-15 activity specifically to the tumor microenvironment, which is designed to promote efficacy and induce durable responses while potentially decreasing safety concerns.

Preclinical data demonstrated that a single dose of KD033 inhibited tumor growth across multiple *in vivo* syngeneic tumor models. KD033 induced a strong immune response with a single treatment, resulting in mice that remained tumor-free following several tumor re-challenges. Furthermore, KD033 in combination with anti-PD-1 therapy demonstrated synergistic activity, providing clinical rationale for administering KD033 in combination with other immune checkpoint inhibitors. KD033 has demonstrated significant tumor inhibition in murine models that are resistant to approved immunotherapies (PD-L1, PD-1 or CTLA-4 antibodies), suggesting that KD033 may deliver promising clinical outcomes in cancer patients resistant or refractory to immuno-oncology monotherapy. We have presented encouraging preclinical data on KD033 at scientific conferences. We plan to initiate a clinical trial of KD033 in the first half of 2020.

Other Clinical Programs

KD025 for the Treatment of Idiopathic Pulmonary Fibrosis

Independent research from academic institutions has demonstrated that ROCK signaling is increased in idiopathic pulmonary fibrosis ("IPF") in humans as well as in murine lung samples. In our preclinical research, KD025 reduced fibrosis in multiple preclinical models, including lung fibrosis in a bleomycin mouse model system. These data suggest that ROCK inhibition has therapeutic potential in IPF by blocking key fibrotic processes mediated by ROCK.

Ongoing Phase 2 Clinical Trial of KD025 in Idiopathic Pulmonary Fibrosis (KD025-207)

We are conducting a randomized, open-label, Phase 2 clinical trial to examine the safety, tolerability and activity of KD025 in IPF patients who have received or been offered pirfenidone and/or nintedanib. The study enrolled 39 patients who were randomized 2:1 to receive KD025 at 400 mg QD or best supportive care ("BSC"). The primary efficacy endpoint is a measure of lung function, the forced vital capacity ("FVC"), at 24 weeks. In initial data, KD025 demonstrated clinical benefit in IPF patients, with a median decline in FVC of 48 mL at week 24, compared to a median decline of 175 mL in patients treated with BSC. KD025 was well tolerated, with no drug-related SAEs.

We have expanded the KD025-207 study to enroll approximately 35 additional patients. We expect these data to support the development of KD045, Kadmon's ROCK inhibitor, for the treatment of fibrotic diseases, including IPF.

Tesevatinib for the Treatment of Polycystic Kidney Disease ("PKD")

Tesevatinib is an oral tyrosine kinase inhibitor in development for the treatment of autosomal dominant polycystic kidney disease ("ADPKD"), a genetic kidney disorder. To date, more than 300 subjects have received tesevatinib for the treatment of PKD or cancer, or as healthy volunteers.

Our randomized, placebo-controlled Phase 2 clinical trial of tesevatinib in ADPKD is ongoing and has completed enrollment. We have de-prioritized the tesevatinib program, and we do not currently expect to fund additional studies once the Phase 2 trial is concluded.

Our Drug Discovery Platforms

We have two drug discovery platforms that support our pipeline of clinical-stage product candidates: small molecules and biologics. We are developing novel therapies in the areas of immune and fibrotic diseases as well as immuno-oncology.

Research and Development

Research and development expenses consist primarily of costs incurred for the development of our product candidates. For the years ended December 31, 2019 and 2018, we recognized $56.5 million and $49.0 million, respectively, in research and development expenses. For further detail about our research and development activities, refer to the research and development sections in "Management's Discussion and Analysis of Financial Condition and Results of Operations" in this Annual Report on Form 10-K.

Sales and Marketing

Kadmon Pharmaceuticals is our wholly owned, fully integrated commercial operation. We currently market CLOVIQUE™ (Trientine Hydrochloride Capsules, USP), a room-temperature stable innovative product developed in-house at Kadmon and generic Trientine Hydrochloride Capsules USP, 250 mg (collectively, "CLOVIQUE"). Trientine hydrochloride is used for the treatment of Wilson's disease in patients who are intolerant of penicillamine. CLOVIQUE™ is the first FDA-approved trientine product in a portable blister pack that offers room temperature stability for up to 30 days, potentially providing patients more convenience than existing treatment options.

We do not currently place significant value on our commercial operations from a revenue-generation standpoint, as revenues from such operations do not currently support our research and development efforts. We leverage our commercial infrastructure to support the development of our clinical-stage product candidates by providing quality assurance, compliance, regulatory and pharmacovigilance among other capabilities. We believe our commercial infrastructure will be most advantageous to us in the future, in connection with the anticipated commercialization of our pipeline product candidates, if approved.

Kadmon Pharmaceuticals is a commercial organization encompassing managed care and specialty pharmacy account directors, experienced regulatory, quality, compliance and CMC teams, marketing experts and sales specialists.

Kadmon Pharmaceuticals has long-standing relationships with specialty pharmacies. The specialty pharmacies through which we distribute our products are fully independent of Kadmon. We do not have any ownership interest in or affiliations with any specialty pharmacy, nor do we consolidate the financial results of any specialty pharmacies with our own.

Investment in MeiraGTx

On June 12, 2018, MeiraGTx Holdings plc ("MeiraGTx") completed its initial public offering (the "MeiraGTx IPO") whereby it sold 5,000,000 ordinary shares at $15.00 per share. Upon completion of the MeiraGTx IPO, we owned approximately 13.0% of MeiraGTx's issued and outstanding ordinary shares and we no longer had the ability to exert significant influence over MeiraGTx's operations. In October 2019, we entered into a transaction pursuant to which we sold approximately 1.4 million ordinary shares of MeiraGTx for gross proceeds of $22.0 million. As of December 31, 2019, we owned approximately 2.1 million, or 5.7%, of the issued and outstanding ordinary shares of MeiraGTx, with a fair value of $42.0 million.

Strategic Collaborations and License Agreements

Nano Terra, Inc.

In April 2011, in connection with the acquisition of Surface Logix, Inc. ("SLx") by Nano Terra, Inc. ("Nano Terra"), our subsidiary Kadmon Corporation entered into a joint venture with SLx through the formation of NT Life Sciences, LLC ("NT Life"), whereby Kadmon Corporation contributed $0.9 million at the date of formation in exchange for a 50.0% interest in NT Life. Contemporaneously with our entry into the joint venture, we entered into an exclusive sub-license agreement with NT Life and SLx, under which NT Life granted us rights to certain patents and know-how it licensed from SLx relating to KD025 (formerly SLx-2119). Under this agreement, NT Life granted to us an exclusive, worldwide, royalty-bearing, sublicensable license (under the patents and know how it licensed from SLx) to make, have made, use, sell, offer for sale, import and export certain products, including KD025. NT Life also granted to us a worldwide, non-exclusive, non-transferable, sublicensable license under certain SLx platform technology to make, have made, use, sell, offer for sale, import and export the products. The initial purpose of the joint venture with SLx was to develop assets licensed to us from SLx and to define the royalty obligations with respect to certain products not exclusively licensed to us. The joint venture is, however, currently inactive. We expect that the joint venture will become active and develop certain intellectual property in the future.

We are the sublicensee of granted patents in the United States for KD025, as well as applications in Australia, Canada, Europe, Japan and the United States, which claim KD025 as a composition-of-matter, and use of KD025 to treat certain diseases. The last-to-expire U.S. patent in this family has a term that ends in October 2029 based on a calculated PTA and without regard to any potential PTE, which could further extend the term by an additional five years.

In consideration for the rights granted to us by NT Life, we agreed to assume certain of Nano Terra's payment obligations, which are limited to the royalty percentages discussed in this paragraph, under the Agreement and Plan of Merger dated April 8, 2011, by and among Nano Terra, NT Acquisition, Inc., SLx, and Dion Madsen, as the Stockholder Representative of SLx (the "Nano Terra Merger Agreement"). Pursuant to these obligations, we are required to pay to the Stockholder Representative and NT Life royalties on net sales in the amount of 5% and 10% respectively. As we own 50% of NT Life, the cumulative result of these obligations is that we will owe aggregate royalty payments totaling 9.75% on net sales of licensed products. Pursuant to the assumption of Nano Terra's payment obligations, if we further assign or sublicense our rights to any licensed product to certain third parties, we are also required to pay to the Stockholder Representative a portion of any sublicensing revenue relating to such licensed product ranging from the low twenty percents to the low forty percents, subject to specified deductions and adjustments. We are also required to pay to NT Life any remaining sublicensing revenue after giving effect to the foregoing sublicense revenue payment to the Stockholder Representative.

Unless earlier terminated, our agreement with NT Life will, with respect to a licensed product, end on a country-by-country and licensed product-by-licensed product basis upon the latest of (a) the expiration or invalidation of the last valid claim of a licensed patent right covering such licensed product in such country and (b) the expiration or termination of payment obligations with respect to such licensed product in such country under the Nano Terra Merger Agreement. The agreement will, with respect to the licensed SLx platform technology, end on a country-by-country basis upon the expiration or invalidation of the last valid claim of a licensed patent right covering such SLx platform technology. We may terminate the agreement at any time upon six months' written notice to NT Life and if we provide such notice, NT Life may accelerate such termination upon thirty days' prior written notice. Either party may terminate the agreement for any material breach by the other party that is not cured within a specified time period. NT Life may terminate the agreement if we challenge the licensed patents. Either party may terminate the agreement upon the bankruptcy or insolvency of the other party. The agreement shall terminate in the event we are dissolved.

In addition, the agreement shall terminate on a licensed product-by-licensed product basis in the event such licensed product reverts to the Stockholder Representative because of a failure to satisfy the diligence requirements as set forth in the Nano Terra Merger Agreement. More specifically, pursuant to our sub-license agreement with NT Life and SLx, we agreed

to assume certain of Nano Terra's diligence obligations under the Nano Terra Merger Agreement such that we are obligated to use commercially reasonable efforts to develop the licensed products, including KD025. With respect to KD025, our diligence obligations do not expire until the completion of a certain specified Phase 2 clinical trial of KD025 in oncology. If, prior to the expiration of our diligence obligations, we fail to comply with such diligence obligations for any licensed product, including KD025, the Stockholder Representative may require Nano Terra to assign all assets of SLx, including intellectual property, relating to such licensed product to an entity designated by such Stockholder Representative, subject to Nano Terra's and our rights to contest such assignment. If such an assignment takes place, our sublicense rights to such intellectual property for such licensed product will terminate.

If the agreement is terminated, among other things, we will be required to cease all development and commercialization of the licensed products, including KD025, all licenses granted to us will terminate and we are obligated to grant NT Life a perpetual, irrevocable, worldwide, exclusive license under certain intellectual property owned or controlled by us that relate to the licensed products to develop and commercialize such licensed products.

Dyax Corp. (acquired by Shire Plc in January 2016 and acquired by Takeda Pharmaceutical Co. Ltd in 2018)

In July 2011, we entered into a license agreement with Dyax Corp. ("Dyax") for the rights to use the Dyax Antibody Libraries, Dyax Materials and Dyax Know-How (collectively "Dyax Property"). The agreement terminated on September 22, 2015, but we had a right to a commercial license of any research target within two years of expiration of the agreement. We exercised this right to a commercial license of two targets in September 2017, one of which is KD033, resulting in a license fee payable to Shire Plc of $1.5 million, which was recorded as research and development expense in the third quarter of 2017. Under the terms of the agreement, we also recorded $1.5 million as research and development expense during the fourth quarter of 2019, related to development milestones we met during that quarter.

Our Intellectual Property

The proprietary nature of, and protection for, our product candidates, their methods of use, and our technologies are an important part of our strategy to discover and develop small molecules and biologics that address areas of significant unmet medical needs in inflammatory and fibrotic diseases, genetic diseases, and in the area of immuno-oncology. We are the owner or exclusive licensee of patents and applications relating to certain of our product candidates, and are pursuing additional patent protection for them and for our other product candidates and technologies. 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. Additionally, we maintain copyrights and trademarks, both registered and unregistered.

Our success will depend significantly on our ability to obtain and maintain patent and other proprietary protection for commercially important products, product candidates, technologies, inventions and know-how related to our business and our ability to defend and enforce our patents, preserve the confidentiality of our trade secrets and operate without infringing the valid and enforceable patents and proprietary rights of third parties. We also rely on know-how, continuing technological innovation and in-licensing opportunities to develop, strengthen and maintain the proprietary position of our development programs. We actively seek to protect our proprietary information, including our trade secrets and proprietary know-how, by requiring our employees, consultants, advisors and partners to enter into confidentiality agreements and other arrangements upon the commencement of their employment or engagement. The chart below identifies which of our product candidates are covered by patents and patent applications that we own or license, the relevant expiration periods and the major jurisdictions. Additional patent applications have been filed to extend the patent life on some of these products, but there can be no assurance that these will issue as filed.

Product Candidate	Description/ Indications	US Patent Numbers	Patent Expiration	Patent Type	Major Jurisdictions	Claim Type
KD025	ROCK2 Inhibitor/cGVHD	15,303,420 (pending)	2035*	Utility	US	Method of Use
KD025	ROCK2 Inhibitor/immune diseases (incl. GVHD)	US2013/063752	2033+	Utility	US, JP	Method of Use
KD025	Inhibitor/immune diseases (incl. GVHD)	(pending)	2033*		CA, CN, EA, EP	Method of Use
KD025	ROCK2 Inhibitor/Fibrosis	8,357,693	2029+	Utility	US	Composition of Matter/ Method of Use
KD025	ROCK2 Inhibitor/Fibrosis	8,916,576	2026+	Utility	CA, CN, EA, EP, JP,	Composition of Matter/ Method of Use
Tesevatinib	Multi-kinase Inhibitor/Oncology	7,576,074 8,658,654	2026+	Utility	AU, CA, EP, JP, US	Composition of Matter/ Method of Use
Tesevatinib	Multi-kinase Inhibitor/Polycystic Kidney Disease	9,364,479	2031+	Utility	CA, CN, EA, EP, TW, US	Method of Use
KD033	Monoclonal Antibody, Immunoconjugate/Oncology	15,111,102	2035+	Utility	US (pending in CN, JP, EU)	Composition of Matter/ Method of Use
KD045	Inhibitors of Rho-associated Coiled-Coil Kinase	US 62/553,619 (pending)	2037*	Utility	US	Composition of Matter/ Method of Use
CLOVIQUE	Chelating Agent/Wilson's Disease	Pending	2036*	Provisional	US	Formulation
KD035	VEGFR2 Monoclonal Antibody/Oncology, Angiogenesis	2015/0284464 A1	2033+	Utility	CN, EA, EP, JP, US	Composition of Matter/ Method of Use
Ribavirin	Nucleoside Inhibitor/Hepatitis	6,720,000 7,538,094 7,723,310	2028+	Utility	US	Composition of Matter
Metabolic Inhibitors	Metabolic Inhibitors/Viral Infection	9,029,413	2028*	Utility	CA, EP, JP, US	Method of Use
GLUT Inhibitors	Glucose Uptake Inhibitors/Immunological and Infectious Diseases	Pending	2036*	Provisional	US	Composition of Matter
PD-L1/VEGFR Antibody	Bispecific Antibody/Oncology	Pending	2037*	Provisional	US, TBD	Composition of Matter/ Method of Use

\+ Indicates the expiration date of a granted patent for which a Patent Term Adjustment (PTA) has been fixed by the United States Patent and Trademark Office. The date may be lengthened by a Patent Term Extension (PTE) upon regulatory approval.

* Indicates the calculated expiration date of a pending patent application based solely on a twenty-year term from the international filing date, without regard to the outcome of patent prosecution or obtaining a PTA and/or PTE.

Manufacturing and Supply

We currently do not own or operate manufacturing facilities for the production of our product candidates. We currently outsource to a limited number of external service providers the production of all active pharmaceutical ingredients (API), drug substances and drug products, and we expect to continue to do so to meet the preclinical and clinical requirements of our product candidates. We do not have long-term agreements with these third parties. We have framework agreements with most of our external service providers, under which they generally provide services to us on a short-term, project-by-project basis. We have long-term relationships with our manufacturing and supply chain partners for our commercial products.

Currently, our drug substance or API raw materials for our product candidates can be supplied by multiple source suppliers. Manufacturing is subject to extensive regulations that impose various procedural and documentation requirements, which govern record keeping, manufacturing processes and controls, personnel, quality control and quality assurance, among others. The contract manufacturing organizations that we use to manufacture our product are obligated to operate under current Good Manufacturing Practice regulations (cGMP) conditions.

Competition

We compete directly with companies that focus on cGVHD, systemic sclerosis and IPF, and companies dedicating their resources to novel forms of therapies for these indications. We also face competition from academic research institutions, governmental agencies and other various public and private research institutions. With the proliferation of new drugs and therapies in these areas, we expect to face increasingly intense competition as new technologies become available.

Any product candidates that we successfully develop and commercialize will compete with existing therapies and new therapies that may become available in the future.

Many of our competitors have significantly greater financial, manufacturing, marketing, drug development, technical and human resources than we do. Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These competitors also compete with us in recruiting and retaining top 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.

The key competitive factors affecting the success of all of our product candidates, if approved, are likely to be their efficacy, safety, dosing convenience, price, the effectiveness of companion diagnostics in guiding the use of related therapeutics, the level of generic competition and the availability of reimbursement from government and other third-party payors.

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, less expensive, more convenient or easier to administer, or have fewer or less severe effects than any products that we may develop. Our competitors also may obtain FDA, European Medicines Agency ("EMA") or other regulatory approval for their products more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market. Even if our product candidates achieve marketing approval, they may be priced at a significant premium over competitive products if any have been approved by then.

There are a number of currently marketed therapies and products in late-stage clinical development to treat cGVHD, systemic sclerosis and IPF, including:

cGVHD	Systemic Sclerosis	IPF
Imbruvica (ibrutinib)	Lenabasum	Esbriet (pirfenidone)
Jakafi (ruxolitinib)	Ofev (nintedanib)	Ofev (nintedanib)
Corticosteroids	Mycophenolate mofetil	
Calcineurin inhibitors	Cyclosporine	

Certain products in development may provide efficacy, safety, dosing convenience and other benefits that are not provided by currently marketed therapies. As a result, they may provide significant competition for any of our product candidates for which we obtain marketing approval.

Government Regulation

Government Regulation and Product Approval

Government authorities in the United States and in other countries extensively regulate, among other things, the research, development, testing, manufacture (including manufacturing changes), quality control, approval, labeling, packaging, storage, record-keeping, promotion, advertising, distribution, marketing, export and import of products such as those we are developing. The processes for obtaining regulatory approvals in the United States and in foreign countries, along with subsequent compliance with applicable statutes and regulations, require the expenditure of substantial resources.

U.S. Drug Development Process

In the United States, the FDA regulates drugs under the Federal Food, Drug and Cosmetic Act ("FDCA"), and in the case of biologics, also the Public Health Service Act, and various implementing regulations. Most biological products meet the FDCA's definition of "drug" and are subject to FDA drug requirements, supplemented by biologics requirements.

Failure to comply with the applicable U.S. requirements at any time during the product development process, approval process, or after approval, may subject an applicant to administrative or judicial sanctions. These sanctions could include the FDA's refusal to approve pending applications, withdrawal of an approval, a clinical hold, untitled or warning letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, refusals of government contracts, restitution, disgorgement or civil or criminal penalties. The process required by the FDA before a drug or biologic may be marketed in the United States generally involves the following:

- completion of preclinical laboratory tests, animal studies and formulation studies according to Good Laboratory Practices regulations;
- submission to the FDA of an IND, which must become effective before human clinical studies may begin;
- approval by an independent institutional review board ("IRB"), at each clinical site before each trial may be initiated;
- performance of adequate and well-controlled human clinical studies according to "good clinical practices" ("GCP") regulations, to establish the safety and efficacy of the proposed drug or biologic for its intended use;
- preparation and submission to the FDA of a New Drug Application ("NDA") or Biologics License Application ("BLA");
- satisfactory completion of an FDA inspection of the manufacturing facility or facilities at which the product, or components thereof, are produced to assess compliance with cGMP to assure that the facilities, methods, and controls are adequate to preserve the drug's identity, strength, quality, and purity; and
- FDA review and approval of the NDA or BLA.

The testing and approval process requires substantial time, effort and financial resources, and we cannot be certain that any approvals for our product candidates will be granted on a timely basis, if at all.

Once a pharmaceutical or biological product candidate is identified for development, it enters the preclinical testing stage. Preclinical tests include laboratory evaluations of product chemistry, toxicity, formulation and stability, as well as animal studies. When a sponsor wants to proceed to test the product candidate in humans, it must submit an IND in order to conduct clinical trials.

An IND sponsor must submit the results of the preclinical tests, together with manufacturing information, analytical data and any available clinical data or literature, to the FDA as part of the IND. The sponsor must also include a protocol detailing, among other things, the objectives of the initial clinical study, the parameters to be used in monitoring safety and the effectiveness criteria to be evaluated if the initial clinical study lends itself to an efficacy evaluation. Some preclinical testing may continue even after the IND is submitted. The IND automatically becomes effective 30 days after receipt by the FDA, unless the FDA raises concerns or questions related to a proposed clinical study and places the study on a clinical hold within that 30-day time period. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical study can begin. Clinical holds also may be imposed by the FDA at any time before or during clinical studies due to safety concerns or non-compliance, and may be imposed on all product candidates within a certain pharmaceutical class. The FDA also can impose partial clinical holds, for example, prohibiting the initiation of clinical studies of a certain duration or for a certain dose.

All clinical studies must be conducted under the supervision of one or more qualified investigators in accordance with GCP regulations. These regulations include the requirement that all research subjects provide informed consent in writing before their participation in any clinical study. Further, an IRB must review and approve the plan for any clinical study before it commences at any institution, and the IRB must conduct continuing review and reapprove the study at least annually. An IRB considers, among other things, whether the risks to individuals participating in the clinical study are minimized and are reasonable in relation to anticipated benefits. The IRB also approves the information regarding the clinical study and the consent form that must be provided to each clinical study subject or his or her legal representative and must monitor the clinical study until completed.

Each new clinical protocol and any amendments to the protocol must be submitted for FDA review, and to the IRBs for approval. Protocols detail, among other things, the objectives of the clinical study, dosing procedures, subject selection and exclusion criteria, and the parameters to be used to monitor subject safety.

Information about certain clinical trials must be submitted within specific timeframes to the NIH, for public dissemination on their ClinicalTrials.gov website.

Human clinical studies are typically conducted in three sequential phases that may overlap or be combined:

- *Phase 1.* The product is initially introduced into a small number of healthy human subjects or patients and tested for safety, dosage tolerance, absorption, metabolism, distribution and excretion and, if possible, to gain early evidence on effectiveness. In the case of some products for severe or life-threatening diseases, especially when the product is suspected or known to be unavoidably toxic, the initial human testing may be conducted in patients.
- *Phase 2.* Involves clinical studies in a limited patient population to identify possible adverse effects and safety risks, to preliminarily evaluate the efficacy of the product for specific targeted diseases and to determine dosage tolerance and optimal dosage and schedule.
- *Phase 3.* Clinical studies are undertaken to further evaluate dosage, clinical efficacy and safety in an expanded patient population at geographically dispersed clinical study sites. These clinical studies are intended to establish the overall risk/benefit relationship of the product and provide an adequate basis for product labeling.

Progress reports detailing the results of the clinical studies must be submitted at least annually to the FDA and safety reports must be submitted to the FDA and the investigators for serious and unexpected suspected adverse events. Phase 1, Phase 2 and Phase 3 testing may not be completed successfully within any specified period, if at all. The FDA or the sponsor may suspend or terminate a clinical study 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 study at its institution if the clinical study is not being conducted in accordance with the IRB's requirements or if the drug has been associated with unexpected serious harm to patients.

Concurrent with clinical studies, companies usually complete additional animal studies and must also develop additional information about the chemistry and physical characteristics of the product and finalize a process for manufacturing the product in commercial quantities in accordance with cGMP requirements. The manufacturing process must be capable of consistently producing quality batches of the product candidate and, among other things, the manufacturer must develop methods for testing the identity, strength, quality and purity of the final product. Additionally, appropriate packaging must be selected and tested and stability studies must be conducted to demonstrate that the product candidate does not undergo unacceptable deterioration over its shelf life.

United States Review and Approval Processes

Assuming successful completion of the required clinical testing, the results of product development, preclinical studies and clinical studies, along with descriptions of the manufacturing process, analytical tests conducted on the drug, proposed labeling and other relevant information, are submitted to the FDA as part of an NDA for a new drug, or a BLA for a biological drug product, requesting approval to market the product.

The submission of an NDA or BLA is subject to the payment of a substantial application user fee although a waiver of such fee may be obtained under certain limited circumstances. For example, the agency will waive the application fee for the first human drug application that a small business or its affiliate submits for review. The sponsor of an approved NDA or BLA is also subject to annual product and establishment user fees. For FDA fiscal year 2019 the application fee for an application with clinical data was $2,942,965. Sponsors are also subject to a prescription drug program fee. For fiscal 2019, the prescription drug program fee was $325,424.

In addition, under the Pediatric Research Equity Act of 2003, an NDA or BLA applications (or supplements to applications) for a new active ingredient, new indication, new dosage form, new dosing regimen, or new route of administration must contain data that are adequate to assess the safety and effectiveness 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 product is safe and effective, unless the applicant has obtained a waiver or deferral.

In 2012, the Food and Drug Administration Safety and Innovation Act ("FDASIA") amended the FDCA to require that a sponsor who is planning to submit a marketing application for a drug or biological product that includes a new active ingredient, new indication, new dosage form, new dosing regimen or new route of administration submit an initial Pediatric Study Plan (PSP), within sixty days of an End-of-Phase 2 meeting or as may be agreed between the sponsor and the 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. The FDA may, on its own initiative or at the request of the applicant, grant deferrals for submission of data or full or partial waivers. The 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 studies, and/or other clinical development programs.

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

The FDA reviews all NDAs and BLAs submitted to ensure that they are sufficiently complete for substantive review before it accepts them for filing. The FDA may request additional information rather than accept an application for filing. In this event, the application must be re-submitted with the additional information. The re-submitted application also is subject to review before the FDA accepts it for filing. Once the submission is accepted for filing, the FDA begins an in-depth substantive review.

The FDA reviews an NDA to determine, among other things, whether a product is safe and effective for its intended use and whether its manufacturing is cGMP-compliant. For biologics, the applicant must demonstrate that the product is safe, pure, and potent (interpreted to include effectiveness), and that the facilities designed for its production meet standards to ensure the product will consistently be safe, pure, and potent.

The FDA may approve an NDA or BLA only if the methods used in, and the facilities and controls used for, the manufacture processing, packing, and testing of the product are adequate to ensure and preserve its identity, strength, quality, and purity. Drug cGMPs are established in 21 C.F.R. Parts 210 and 211, and biologic drug products must meet the drug standards as well as the supplemental requirements in 21 C.F.R. Part 600 et seq.

Before approving an NDA or BLA, the FDA often will inspect the facility or facilities where the product is or will be manufactured.

The FDA may refer the NDA or BLA to an advisory committee for review, evaluation and recommendation as to whether the application should be approved and under what conditions. An advisory committee is a panel of experts, including clinicians and other scientific experts, who provide advice and recommendations when requested by the FDA. The FDA is not bound by the recommendation of an advisory committee, but it considers such recommendations when making decisions.

Additionally, before approving an NDA or BLA, the FDA will typically inspect one or more clinical sites to ensure that clinical data supporting the submission were developed in compliance with GCP.

The approval process is lengthy and difficult and the FDA may refuse to approve an NDA or BLA if the applicable regulatory criteria are not satisfied, or may require additional clinical data or other data and information. Even if such data and information are submitted, the FDA may ultimately decide that the NDA or BLA does not satisfy the criteria for approval. Data obtained from clinical studies are not always conclusive and the FDA may interpret data differently than an applicant interprets the same data.

After the FDA's evaluation of an application, the FDA may issue an approval letter, or, in some cases, a complete response letter to indicate that the review cycle is complete and that the application is not ready for approval. A complete response letter generally contains a statement of specific conditions that must be met to secure final approval of the application and may require additional clinical or preclinical testing for the FDA to reconsider the application. The deficiencies identified may be minor, for example, requiring labeling changes, or major, for example, requiring additional clinical studies. Additionally, the complete response letter may include recommended actions that the applicant might take to place the application in a condition for approval. If a complete response letter is issued, the applicant may either resubmit the application, addressing all of the deficiencies identified in the letter, or withdraw the application or request an opportunity for a hearing.

Even with submission of additional information, the FDA ultimately may decide that the application does not satisfy the regulatory criteria for approval. If and when those conditions have been met to the FDA's satisfaction, the FDA will typically issue an approval letter. An approval letter authorizes commercial marketing of the drug with specific prescribing information for specific indications.

If a product receives regulatory approval, the approval may be significantly limited to specific diseases and dosages or the indications for use may otherwise be limited, which could restrict the commercial value of the product. Further, the FDA may require that certain contraindications, warnings or precautions be included in the product labeling. In addition, the FDA may require post-approval studies, including Phase 4 clinical studies, to further assess safety and effectiveness after approval and may require testing and surveillance programs to monitor the safety of approved products that have been commercialized. After approval, some types of changes to the approved product, such as adding new indications, manufacturing changes, and additional labeling claims, are subject to further testing requirements and FDA review and approval.

ANDAs and Section 505(b)(2) New Drug Applications

Most drug products obtain FDA marketing approval pursuant to an NDA or BLA (described above) for innovator products, or an abbreviated new drug application ("ANDA") for generic products. Relevant to ANDAs, the Hatch-Waxman amendments to the FDCA established a statutory procedure for submission and FDA review and approval of ANDAs for generic versions of branded drugs previously approved by the FDA (such previously approved drugs are also referred to as listed drugs). Because the safety and efficacy of listed drugs have already been established by the brand company (sometimes referred to as the innovator), the FDA does not require a demonstration of safety and efficacy of generic products. However, a generic manufacturer is typically required to conduct bioequivalence studies of its test product against the listed drug. The bioequivalence studies for orally administered, systemically available drug products assess the rate and extent to which the API is absorbed into the bloodstream from the drug product and becomes available at the site of action. Bioequivalence is established when there is an absence of a significant difference in the rate and extent for absorption of the generic product and the listed drug. For some drugs (e.g., locally acting drugs like topical anti-fungals), other means of demonstrating bioequivalence may be required by the FDA, especially where rate and/or extent of absorption are difficult or impossible to measure. In addition to the bioequivalence data, an ANDA must contain patent certifications and chemistry, manufacturing, labeling and stability data.

The third alternative is a special type of NDA, commonly referred to as a Section 505(b)(2) NDA, which enables the applicant to rely, in part, on the FDA's findings of safety and efficacy of an existing product, or published literature, in support of its application. Section 505(b)(2) NDAs often provide an alternate path to FDA approval for new or improved formulations or new uses of previously approved products. Section 505(b)(2) permits the filing of an NDA where at least some of the information required for approval comes from studies not conducted by or for the applicant and for which the applicant has not obtained a right of reference. The applicant may rely upon the FDA's findings with respect to certain preclinical or clinical studies conducted for an approved product. The FDA may also require companies to perform additional studies or measurements to support the change from the approved product. The FDA may then approve the new product candidate for all or some of the label indications for which the referenced product has been approved, as well as for any new indication sought by the Section 505(b)(2) applicant.

In seeking approval for a drug through an NDA, including a 505(b)(2) NDA, applicants are required to list with the FDA certain patents of the applicant or that are held by third parties whose claims cover the applicant's product. Upon approval of an NDA, each of the patents listed in the application for the drug is then published in the Orange Book. Any subsequent applicant who files an ANDA seeking approval of a generic equivalent version of a drug listed in the Orange Book or a 505(b)(2) NDA referencing a drug listed in the Orange Book must make one of the following certifications to the FDA concerning patents: (1) the patent information concerning the reference listed drug product has not been submitted to the FDA; (2) any such patent that was filed has expired; (3) the date on which such patent will expire; or (4) such patent is invalid or will not be infringed upon by the manufacture, use or sale of the drug product for which the application is submitted. This last certification is known as a paragraph IV certification. A notice of the paragraph IV certification must be provided to each owner of the patent that is the subject of the certification and to the holder of the approved NDA to which the ANDA or 505(b)(2) application refers. The applicant may also elect to submit a "section viii" statement certifying that its proposed label does not contain (or carves out) any language regarding the patented method-of-use rather than certify to a listed method-of-use patent.

If the reference NDA holder or patent owners assert a patent challenge directed to one of the Orange Book listed patents within 45 days of the receipt of the paragraph IV certification notice, the FDA is prohibited from approving the application until the earlier of 30 months from the receipt of the paragraph IV certification expiration of the patent, settlement of the lawsuit or a decision in the infringement case that is favorable to the applicant. The ANDA or 505(b)(2) application also will not be approved until any applicable non-patent exclusivity listed in the Orange Book for the branded reference drug has expired as described in further detail below. Thus approval of a Section 505(b)(2) NDA or ANDA can be stalled until all the listed patents claiming the referenced product have expired, until any non-patent exclusivity, such as exclusivity for obtaining approval of a new chemical entity, listed in the Orange Book for the referenced product has expired, and, in the case of a Paragraph IV certification and subsequent patent infringement suit, until the earlier of 30 months, settlement of the lawsuit or a decision in the infringement case that is favorable to the ANDA or Section 505(b)(2) applicant.

Expedited Programs

Fast Track Designation

The FDA has a Fast Track program that is intended to expedite or facilitate the process for reviewing new drugs that meet certain criteria. Specifically, new drugs (including biological drug products) are eligible for Fast Track designation if they are intended to treat a serious or life-threatening disease or condition for which there is no effective treatment and demonstrate the potential to address unmet medical needs for the condition. Fast Track designation applies to the combination of the product and the specific indication for which it is being studied. The sponsor of a new drug or biologic

may request the FDA to designate the drug or biologic as a Fast Track product concurrently with, or at any time after, submission of an IND, and the FDA must determine if the product candidate qualifies for Fast Track designation within 60 days of receipt of the sponsor's request.

The FDA may initiate review of sections of a Fast Track drug's NDA or BLA before the application is complete. This rolling review is available if the applicant provides, and the FDA approves, a schedule for the submission of each portion of the NDA or BLA and the applicant pays applicable user fees. However, the FDA's time period goal for reviewing an application does not begin until the last section of the application is submitted. Additionally, the Fast Track designation may be withdrawn by the FDA if the FDA believes that the designation is no longer supported by data emerging in the clinical study process.

Accelerated Approval

Under the FDA's accelerated approval regulations, the FDA may approve a drug or biologic for a serious or life-threatening illness that fills an unmet medical need, providing a meaningful therapeutic benefit to patients over existing treatments, based upon a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, 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. In clinical studies, a surrogate endpoint is a marker, such as a measurement of laboratory or clinical signs of a disease or condition that is thought to predict clinical benefit, but is not itself a measure of clinical benefit. Surrogate endpoints can often be measured more easily or more rapidly than clinical endpoints. A product candidate approved on this basis is subject to rigorous post-marketing compliance requirements, including the completion of post-approval clinical studies sometimes referred to as Phase 4 studies to confirm the effect on the clinical endpoint. Failure to conduct required post-approval studies, or to confirm a clinical benefit during post-marketing studies, will allow the FDA to withdraw the product from the market on an expedited basis. All promotional materials for product candidates approved under accelerated approval regulations are subject to prior review by the FDA.

Breakthrough Designation

The FDASIA amended the FDCA to require the FDA to expedite the development and review of a breakthrough therapy. A drug or biologic product can be designated as a breakthrough therapy if it is intended to treat a serious or life-threatening disease or condition and preliminary clinical evidence indicates that it may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints. A sponsor may request that a drug or biologic product be designated as a breakthrough therapy concurrently with, or at any time after, the submission of an IND, and the FDA must determine if the product candidate qualifies for breakthrough therapy designation within 60 days of receipt of the sponsor's request. If so designated, the FDA shall act to expedite the development and review of the product's marketing application, including by meeting with the sponsor throughout the product's development, providing timely advice to the sponsor to ensure that the development program to gather preclinical and clinical data is as efficient as practicable, involving senior managers and experienced review staff in a cross-disciplinary review, assigning a cross-disciplinary project lead for the FDA review team to facilitate an efficient review of the development program and to serve as a scientific liaison between the review team and the sponsor, and taking steps to ensure that the design of the clinical studies is as efficient as practicable.

Priority Review

Priority review is granted where there is evidence that the proposed product would be a significant improvement in the safety or effectiveness of the treatment, diagnosis, or prevention of a serious condition. If criteria are not met for priority review, the application is subject to the standard FDA review period of 10 months after the FDA accepts the application for filing. Priority review designation does not change the scientific/medical standard for approval or the quality of evidence necessary to support approval.

Post-Approval Requirements

Drugs and biologics manufactured or distributed pursuant to FDA approvals are subject to extensive and continuing regulation by the FDA, including, among other things, requirements relating to recordkeeping (including certain electronic record and signature requirements), periodic reporting, product sampling and distribution, advertising and promotion and reporting of certain adverse experiences, deviations, and other problems with the product. After approval, most changes to the approved product, such as adding new indications or other labeling claims are subject to prior FDA review and approval. There also are continuing, annual user fee requirements for any marketed products and the establishments at which such products are manufactured, as well as new application fees for supplemental applications with clinical data.

The FDA strictly regulates labeling, advertising, promotion and other types of information on products that are placed on the market. Products may be promoted only for the approved indications and in accordance with the provisions of the approved label. Further, manufacturers must continue to comply with cGMP requirements, which are extensive and require considerable time, resources and ongoing investment to ensure compliance. In addition, changes to the manufacturing process generally require prior FDA approval before being implemented and other types of changes to the approved product, such as adding new indications and additional labeling claims, are also subject to further FDA review and approval.

Manufacturers and certain other entities involved in the manufacturing and distribution of approved products are required to register their establishments with the FDA and certain state agencies, and are subject to periodic unannounced inspections by the FDA and certain state agencies for compliance with cGMP and other laws. The cGMP requirements apply to all stages of the manufacturing process, including the production, processing, sterilization, packaging, labeling, storage and shipment of the product. Manufacturers must establish validated systems to ensure that products meet specifications and regulatory standards, and test each product batch or lot prior to its release.

Changes to the manufacturing process are strictly regulated and often require prior FDA approval before being implemented. FDA regulations also require investigation and correction of any deviations from cGMP and impose reporting and documentation requirements upon the sponsor and any third-party manufacturers that the sponsor may decide to use. Accordingly, manufacturers must continue to expend time, money, and effort in the area of production and quality control to maintain cGMP compliance.

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

The FDA may withdraw a product approval if compliance with regulatory requirements is not maintained or if problems occur after the product reaches the market. Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, problems with manufacturing processes, or failure to comply with regulatory requirements, may result in restrictions on the product or even complete withdrawal of the product from the market.

Potential implications include required revisions to the approved labeling to add new safety information; imposition of post-market studies or clinical trials to assess new safety risks; or imposition of distribution or other restrictions under a REMS program. Other potential consequences include, among other things:

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

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

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

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

Patent Term Restoration

Depending upon the timing, duration and specifics of FDA approval of the use of our product 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 Act. The Hatch-Waxman Act permits a patent restoration term of up to five years as compensation for patent term effectively lost during product development and the 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/BLA and the approval of that application, except that the review period is reduced by any time during which the applicant failed to exercise due diligence. Only one patent applicable to an approved drug is eligible for the extension. Extensions are not granted as a matter of right and the extension must be applied for prior to expiration of the patent and within a 60-day period from the date the product is first approved for commercial marketing. The U.S. Patent and Trademark Office, in consultation with the FDA, reviews and approves the application for any patent term extension or restoration. In the future, we may apply for Patent Term Extensions, defined as the length of the regulatory review of products covered by our granted patents, for some of our currently owned or licensed applications and patents to add patent life beyond their current expiration dates. Such extensions will depend on the length of the regulatory review; however, there can be no assurance that any such extension will be granted to us.

Marketing Exclusivity

Market exclusivity provisions under the FDCA can also delay the submission or the approval of certain applications. The specific scope varies, but fundamentally the FDCA provides a five-year period of non-patent marketing exclusivity within the United States to the first applicant to gain approval of an NDA for a new chemical entity. A drug is a new chemical entity if the 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, the FDA may not accept for review an ANDA or a 505(b)(2) NDA submitted by another company for another version of such drug where the applicant does not own or have a legal right of reference to all the data required for approval. However, an application may be submitted after four years if it contains a certification of patent invalidity or non-infringement. The FDCA also provides three years of marketing exclusivity for an NDA, 505(b)(2) 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 the FDA to be essential to the approval of the application, for example, for new indications, dosages or strengths of an existing drug. This three-year exclusivity covers only the conditions of use associated with the new clinical investigations and does not prohibit the FDA from approving applications for drugs containing the original active agent. 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 studies necessary to demonstrate safety and effectiveness.

Pediatric exclusivity is another type of exclusivity in the United States. Pediatric exclusivity, if granted, provides an additional six months to the term of any existing regulatory exclusivity, including the non-patent exclusivity periods described above. This six-month exclusivity may be granted based on the voluntary completion of a pediatric clinical study in accordance with an FDA-issued "Written Request" for such a clinical study.

With respect to biologics, the Patient Protection and Affordable Care Act, as amended by the Healthcare and Education Reconciliation Act (collectively, the PPACA) signed into law on March 23, 2010, includes a subtitle called the Biologics Price Competition and Innovation Act of 2009 (BPCIA), which created an abbreviated licensure pathway for biological products that are biosimilar to or interchangeable with an FDA-licensed reference biological product. To date, only one biosimilar has been licensed under the BPCIA in the United States (in September 2015), with many more well into the process for approval. Numerous biosimilars have already been approved in Europe. The FDA has issued several guidance documents outlining an approach to review and approval of biosimilars, although there has been significant litigation and questions over interpretation of such guidelines.

Biosimilarity, which requires that the product be "highly similar" and there be no clinically significant differences between the biological product and the reference product in terms of safety, purity, and potency, can be shown through analytical studies, animal studies, and a clinical study or studies. Interchangeability requires that a product is biosimilar to the reference product and the product must demonstrate that it can be expected to produce the same clinical results as the reference product in any given patient and, for products that are administered multiple times to an individual, the biologic and the reference biologic may be alternated or switched after one has been previously administered without increasing safety risks or risks of diminished efficacy relative to exclusive use of the reference biologic. However, complexities associated with the larger, and often more complex, structures of biological products, as well as the processes by which such products are manufactured, pose significant hurdles to implementation of the abbreviated approval pathway that are still being worked out by the FDA.

Under the BPCIA, an application for a biosimilar product may not be submitted to the FDA until four years following the date that the reference product was first licensed by the FDA. In addition, the approval of a biosimilar product may not be made effective by the FDA until 12 years from the date on which the reference product was first licensed. During this 12-year period of exclusivity, another company may still market a competing version of the reference product if the FDA approves a full BLA for the competing product containing the sponsor's own preclinical data and data from adequate and well-controlled clinical trials to demonstrate the safety, purity and potency of their product. The BPCIA also created certain exclusivity periods for biosimilars approved as interchangeable products. At this juncture, it is unclear whether products deemed "interchangeable" by the FDA will, in fact, be readily substituted by pharmacies, which are governed by state pharmacy law.

The BPCIA is complex and only beginning to be interpreted and implemented by the FDA. In addition, recent government proposals have sought to reduce the 12-year reference product exclusivity period. Other aspects of the BPCIA, some of which may impact the BPCIA exclusivity provisions, have also been the subject of recent litigation. As a result, the ultimate impact, implementation, and meaning of the BPCIA is subject to significant uncertainty.

Orphan Designation and Exclusivity

Under the Orphan Drug Act, the FDA may grant orphan drug designation to drugs (including biological drug products) intended to treat a rare disease or condition—generally a disease or condition that affects fewer than 200,000 individuals in the United States or that affects more than 200,000 individuals in the United States and for which there is no reasonable expectation that costs of research and development of the drug for the indication can be recovered by sales of the drug in the United States. Orphan drug designation must be requested before submitting an NDA or BLA.

After the FDA grants orphan drug designation, the generic identity of the drug and its potential orphan use are disclosed publicly by the FDA. Orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process. The first applicant to receive FDA approval for a particular active ingredient to treat a particular disease or condition with FDA orphan drug designation is entitled to a seven-year exclusive marketing period in the United States for that product, for that indication. Among the other benefits of orphan drug designation are tax credits for certain research and a waiver of the NDA/BLA application user fee.

During the exclusivity period, the FDA may not approve any other applications to market the same drug for the same disease or condition, except in limited circumstances, such as if the second applicant demonstrates the clinical superiority of its product to the product with orphan drug exclusivity through a demonstration of superior safety, superior efficacy, or a major contribution to patient care, or if the manufacturer of the product with orphan exclusivity is not able to assure sufficient quantities of the product. "Same drug" means a drug that contains the same identity of the active moiety if it is a drug composed of small molecules, or of the principal molecular structural features if it is composed of macromolecules and is intended for the same use as a previously approved drug, except that if the subsequent drug can be shown to be clinically superior to the first drug, it will not be considered to be the same drug. Drug exclusivity does not prevent the FDA from approving a different drug for the same disease or condition, or the same drug for a different disease or condition.

Pharmaceutical Coverage, Pricing and Reimbursement

In the United States, sales of CLOVIQUE and any products for which we may receive regulatory approval for commercial sale will depend in part on the availability of coverage and reimbursement from third-party payors. Third-party payors include government authorities, managed care providers, private health insurers and other organizations.

Significant uncertainty exists as to the coverage and reimbursement status of any products for which we may obtain regulatory approval. The process for determining whether a payor will provide coverage for a biologic or drug may be separate from the process for setting the reimbursement rate that the payor will pay for the product. Some of the additional requirements and restrictions on coverage and reimbursement levels imposed by third-party payors influence the purchase of healthcare services and products. Third-party payors may limit coverage to specific biologics and drugs on an approved list, or formulary, which might not include all of the FDA-approved biologics or drugs for a particular indication, or place biologics and drugs at certain formulary levels that result in lower reimbursement levels and higher cost-sharing obligation imposed on patients. Moreover, a payor's decision to provide coverage for a drug product does not imply that an adequate reimbursement rate will be approved. Adequate third-party reimbursement may not be available to enable us to maintain price levels sufficient to realize an appropriate return on our investment in product development. Further, one payor's determination to provide coverage does not assure that other payors will also provide coverage and reimbursement for the product, and the level of coverage and reimbursement may differ significantly from payor to payor.

Third-party payors are increasingly challenging the price and examining the medical necessity and cost-effectiveness of medical products and services, in addition to their safety and efficacy. In order to obtain and maintain coverage and reimbursement for any product that might be approved for sale, we may need to conduct expensive

pharmacoeconomic studies in order to demonstrate the medical necessity and cost-effectiveness of any products, in addition to the costs required to obtain regulatory approvals. Our product candidates may not be considered medically necessary or cost-effective. If third-party payors do not consider a product to be cost-effective compared to other available therapies, they may not cover the product after approval as a benefit under their plans or, if they do, the level of payment may not be sufficient to allow a company to sell its products at a profit.

The U.S. government and state legislatures have shown significant interest in implementing cost containment programs to limit the growth of government-paid healthcare costs, including price controls, restrictions on reimbursement and coverage and requirements for substitution of generic products for branded prescription drugs. Adoption of government controls and measures, and tightening of restrictive policies in jurisdictions with existing controls and measures, could exclude or limit our drugs and product candidates from coverage and limit payments for pharmaceuticals.

In addition, we expect that the increased emphasis on managed care and cost containment measures in the United States by third-party payors and government authorities to continue and will place pressure on pharmaceutical pricing and coverage. Coverage policies and third-party reimbursement rates may change at any time. Even if favorable coverage and reimbursement status is attained for one or more products for which we receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

Other Healthcare Laws and Compliance Requirements

Healthcare providers, physicians, and third-party payors often play a primary role in the recommendation and prescription of any currently marketed products and product candidates for which we may obtain marketing approval. Our current and future arrangements with healthcare providers, physicians, third-party payors and customers, and our sales, marketing and educational activities, may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations (at the federal and state level) that may constrain our business or financial arrangements and relationships through which we market, sell and distribute our products for which we obtain marketing approval.

In addition, we may be subject to patient privacy regulation by both the federal government and the states in which we conduct our business. The laws that govern certain of our operations include the following, but are not limited to:

a) federal and state laws relating to the Medicare and Medicaid programs and any other federal healthcare program;
b) federal and state laws relating to healthcare fraud and abuse, including, without limitation, the federal Anti-Kickback Statute (42 U.S.C. § 1320a-7b(b)), the federal False Claims Act (31 U.S.C. §§ 3729 et seq.), the False Statements Statute, (42 U.S.C. § 1320a-7b(a)), the Exclusion Laws (42 U.S.C. § 1320a-7), the federal Physician Payment Sunshine Act (42 U.S.C. § 1320a-7h), the Drug Supply Chain Security Act (21 U.S.C. § 351 et seq.), the Anti-Inducement Statute (42 U.S.C. § 1320a-7a(a)(5)), the Civil Monetary Penalties Law (42 U.S.C. § 1320a-7a) and criminal laws relating to healthcare fraud and abuse, including but not limited to 18 U.S.C. §§ 286, 287 and 1001, and the Health Insurance Portability and Accountability Act of 1996, or HIPAA, (Pub.L. 104-191);
c) state laws relating to Medicaid or any other state healthcare or health insurance programs;
d) federal or state laws relating to billing or claims for reimbursement submitted to any third party payor, employer or similar entity, or patient;
e) any other federal or state laws relating to fraudulent, abusive or unlawful practices connected in any way with the provision or marketing of healthcare items or services, including laws relating to the billing or submitting of claims for reimbursement for any items or services reimbursable under any state, federal or other governmental healthcare or health insurance program or any private payor; and
f) federal and state laws relating to health information privacy and security, including HIPAA, and any rules or regulations promulgated thereunder, and the Health Information Technology for Economic and Clinical Health Act, enacted as part of the American Recovery and Reinvestment Act of 2009 and any regulations promulgated thereunder.

If our operations are found to be in violation of any of the health regulatory laws described above or any other laws that apply to us, we may be subject to penalties, including criminal and significant civil monetary penalties, damages, fines, imprisonment, exclusion from participation in government healthcare programs, injunctions, recall or seizure of products, total or partial suspension of production, denial or withdrawal of pre-marketing product approvals, private qui tam actions brought by individual whistleblowers in the name of the government or refusal to allow us to enter into supply contracts, including government contracts and the curtailment or restructuring of our operations, any of which could adversely affect our ability to operate our business and our results of operations.

Foreign Regulation of Drugs and Biologics

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

Employees

As of December 31, 2019, we employed 115 people, including 73 in research and development, 18 in commercial operations and 24 in a general and administrative capacity, including executive officers. We also engage a number of temporary employees and consultants. None of our employees is represented by a labor union with respect to his or her employment with us. We have not experienced any work stoppages, and we consider our relations with our employees to be good.

Emerging Growth Company

We are an “emerging growth company,” as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act. We will remain an emerging growth company until the earlier of (1) December 31, 2021, (2) the last day of the first fiscal year in which our annual gross revenues exceed $1.07 billion, (3) the date on which we become a “large accelerated filer” as defined in Rule 12b-2 under the Securities Exchange Act of 1934, as amended, which would occur if the market value of our common stock that is held by non-affiliates exceeds $700 million as of the last business day of our most recently completed second fiscal quarter or (4) the date on which we have issued more than $1.0 billion in non-convertible debt during the preceding three-year period.

For as long as we remain an “emerging growth company,” we may take advantage of certain exemptions from various reporting requirements that are applicable to public companies that are not “emerging growth companies” including, but not limited to, not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act, reduced disclosure obligations regarding executive compensation and financial statements in our periodic reports and proxy statements, and exemptions from the requirements of holding a nonbinding advisory vote to approve executive compensation and shareholder approval of any golden parachute payments not previously approved. We are choosing to “opt out” of the extended transition periods available under the JOBS Act for complying with new or revised accounting standards, and intend to take advantage of the other reporting exemptions until we are no longer an “emerging growth company.”

Facilities

Our corporate headquarters are located in New York, New York, and consist of approximately 35,771 square feet of space under a lease that expires in October 2025. In addition, we also have locations in Warrendale, Pennsylvania; Cambridge, Massachusetts and Monmouth Junction, New Jersey. We believe that our facilities are adequate for our current needs and for the foreseeable future.

Corporate Information

Our common stock is currently listed on The New York Stock Exchange under the symbol “KDMN.” We are an “emerging growth company” under the Jumpstart Our Business Startups Act of 2012, and therefore we are subject to reduced public company reporting requirements. Our principal executive offices are located at 450 East 29th Street, New York, New York 10016, and our telephone number is (833)-900-5366. Our website address is www.kadmon.com.

Available Information

We make available on or through our website certain reports and amendments to those reports that we file with, or furnish to, the SEC in accordance with the Securities Exchange Act of 1934, as amended, or the Exchange Act. These include our Annual Reports on Form 10-K, our Quarterly Reports on Form 10-Q and our Current Reports on Form 8-K, and amendments to those reports filed or furnished pursuant to Section 13(a) or 15(d) of the Exchange Act. We make this information available on or through our website free of charge as soon as reasonably practicable after we electronically file the information with, or furnish it to, the SEC. Our website address is www.kadmon.com. Copies of this information may be obtained at the SEC’s Public Reference Room at 100 F Street, NE, Washington, D.C. 20549. Information on the operation of the Public Reference Room may be obtained by calling the SEC at 1-800-SEC-0330. The SEC maintains a website that contains reports, proxy and information statements, and other information regarding our filings, at www.sec.gov. The information on, or that can be accessed through, our website is not incorporated by reference into this Annual Report on Form 10-K or any other filings we make with the SEC.

Item 1A. Risk Factors

Described below are various risks and uncertainties that may affect our business. These risks and uncertainties are not the only ones we face. You should recognize that other significant risks and uncertainties may arise in the future, which we cannot foresee at this time. Also, the risks that we now foresee might affect us to a greater or different degree than expected. Certain risks and uncertainties, including ones that we currently deem immaterial or that are similar to those faced by other companies in our industry or business in general, may also affect our business. If any of the risks described below actually occur, our business, financial condition or results of operations could be materially and adversely affected.

Risks Related to Our Financial Position

We have incurred substantial losses since our inception, anticipate that we will continue to incur losses for the foreseeable future and may not achieve or sustain profitability. We expect to continue to incur significant expenses related to the development of our clinical product candidates for at least the next several years, and we anticipate that our expenses will increase substantially as a result of multiple initiatives. These factors individually and collectively raise a substantial doubt about or ability to continue as a going concern.

Since inception, we have incurred substantial operating losses. Our consolidated net losses were $61.4 million and $54.3 million for the years ended December 31, 2019 and 2018, respectively. Our accumulated deficits were $333.1 million and $269.6 million at December 31, 2019 and 2018, respectively. To date, we have financed our clinical development operations primarily through issuance of common stock and other equity securities in public and private offerings and debt financings. We expect to continue to incur significant expenses related to the development of our clinical product candidates for at least the next several years. We anticipate that our expenses will increase substantially as we:

- initiate or continue our clinical trials related to our most advanced product candidates;
- continue the research and development of our other product candidates;
- seek to discover additional product candidates;
- seek regulatory approvals for our product candidates;
- scale up our sales, marketing and distribution infrastructure and product sourcing capabilities to commercialize additional products we may acquire or license from others or for which we may develop and obtain regulatory approval; and/or
- scale up our operational, financial and management information systems and personnel, including personnel to support our product development and planned additional commercialization efforts.

In the absence of substantial revenue from the sale of our products and products that we distribute, or from other sources (the amount, timing, nature or source of which cannot be predicted), we expect our substantial losses to continue as we develop our business and we may need to discontinue operations. Our ability to generate sufficient revenues from our existing products or from any of our product candidates in development, and to transition to profitability and generate consistent positive cash flow is uncertain. We may continue to incur losses and negative cash flow and may never transition to profitability or positive cash flow.

Our independent registered public accounting firm has included an explanatory paragraph in its report as of and for the year ended December 31, 2019 expressing substantial doubt in our ability to continue as a going concern based on our recurring and continuing losses from operations and our need for additional funding to continue operations. Our consolidated financial statements as of December 31, 2019 do not include any adjustments that might result from the outcome of this going concern uncertainty and have been prepared under the assumption that we will continue to operate as a going concern for the next twelve months, which contemplates the realization of assets and the satisfaction of liabilities in the normal course of business. If we are unable to continue as a going concern we may be forced to liquidate our assets which would have an adverse impact on our business and developmental activities. In such a scenario, the values we receive for our assets in liquidation or dissolution could be significantly lower than the values reflected in our financial statements. The reaction of investors to the inclusion of a going concern statement by our independent registered public accounting firm and our potential inability to continue as a going concern may materially adversely affect our stock price and our ability to raise new capital or to enter into strategic alliances.

We will need additional funding in the future, which may not be available to us, and this may force us to delay, reduce or eliminate our product development programs or commercialization efforts.

We will need to expend substantial resources for research and development and commercialization of our marketed products, including costs associated with:

- clinical trials for our product candidates;
- discovery of additional product candidates;
- life-cycle management of our marketed products;

- the continued commercialization of our commercial products; and/or
- preparing for potential commercialization of our late-stage product candidates and, if one or more of those product candidates receive(s) regulatory approval, to fund the launch of that (those) product(s).

We do not expect that our existing cash, cash equivalents and restricted cash will be sufficient to enable us to fund the completion of development and commercialization of any of our product candidates. We do not have any additional committed external source of funds. Our expenses may increase for many reasons, including:

- clinical trial-related expenses for our product candidates;
- the potential launch and marketing of our late-stage product candidates; and/or
- manufacturing scale-up for commercialization of our late-stage product candidates.

To the extent that we need to raise additional capital through the sale of equity or convertible debt securities, investors in our common stock will be diluted, and the terms of any newly issued securities may include liquidation or other preferences that adversely affect the value of our common stock.

Risks Related to Our Clinical Development Pipeline

We depend heavily on the success of KD025. If we are unable to obtain regulatory approval for KD025, our ability to create stockholder value will be limited.

Our most advanced product candidate is KD025, for which we are currently conducting a pivotal trial, ROCKstar (KD025-213), in patients with cGVHD who have received at least two prior lines of systemic therapy. We do not generate meaningful revenues from any FDA-approved drug products. Two of our product candidates, KD025 and tesevatinib, are in clinical trials and we have additional internally developed product candidates such as KD033 and KD045, which are in the early stages of development. There is no guarantee that our clinical trials will be successful or that we will continue with clinical studies to support an approval from the FDA of any of our product candidates for any indication. We note that most drug candidates never reach the clinical development stage and even those that do have only a small chance of successfully completing clinical development and gaining regulatory approval. Therefore, our business currently depends heavily on the successful development, regulatory approval and commercialization of KD025, which may never occur.

Clinical development is a lengthy and expensive process with a potentially uncertain outcome. Our long-term success depends upon the successful development and commercialization of our product candidates, which is highly uncertain.

Clinical testing is expensive and can take many years to complete, and its outcome is inherently uncertain. Failure can occur at any time during the clinical trial process. The results of pre-clinical studies and early clinical trials may not be predictive of the results of later-stage clinical trials. We cannot assure you that the FDA will view the results as we do or that any future trials of our drug candidates will achieve positive results. Product candidates in later stages of clinical trials may fail to show the desired safety and efficacy traits despite having progressed through pre-clinical studies and initial clinical trials. A number of companies in the pharmaceutical industry have suffered significant setbacks in advanced clinical trials due to lack of efficacy or adverse safety profiles, notwithstanding promising results in earlier trials. Any future clinical trial results for our drug candidates may not be successful.

In addition, a number of factors could contribute to a lack of favorable safety and efficacy results for our drug candidates. For example, such trials could result in increased variability due to varying site characteristics, such as local standards of care, differences in evaluation period and surgical technique, and due to varying patient characteristics, including demographic factors and health status.

To date, we have invested a significant portion of our efforts and financial resources in the acquisition and development of our product candidates. Our long-term success depends upon the successful development, regulatory approval and commercialization of these product candidates. If we fail to obtain regulatory approval to market and sell our product candidates, or if approval is delayed, we will be unable to generate revenue from the sale of these products, our potential for generating positive cash flow will be diminished and the capital necessary to fund our operations will be increased. Our business depends significantly on the successful development, regulatory approval and commercialization of our product candidates, which may never occur.

We cannot be certain as to what type and how many clinical trials the FDA will require us to conduct before we may successfully gain approval to market any of our product candidates. Prior to approving a new drug or biologic, the FDA generally requires that the effectiveness of the product candidate (which is not typically fully investigated until Phase 3) be demonstrated in two adequate and well-controlled clinical trials. In some situations, the FDA approves drugs or biologics on the basis of a single well-controlled clinical trial establishing effectiveness. However, if the FDA determines that our Phase 3 clinical trial results do not demonstrate a statistically significant, clinically significant benefit with an acceptable safety profile, or if the FDA requires us to conduct additional Phase 3 clinical trials in order to gain approval, we will incur significant additional development costs and commercialization of these products would be prevented or delayed and our business would be adversely affected.

Our ongoing clinical trials may be subject to delays or setbacks for a variety of common and unpredictable reasons.

We may experience unforeseen delays or setbacks in our ongoing clinical trials, such as trial initiation timing, trial redesign or amendments, timing and availability of patient enrollment or successful trial completion. Such delays and setbacks are common and unpredictable in pharmaceutical drug development. Clinical trials can be delayed for a variety of reasons, including delays related to:

- regulatory objections to commencing a clinical trial, continuing a clinical trial that is underway, or proceeding to the next phase of investigation, including inability to reach agreement with the FDA or non-U.S. regulators regarding the scope or design of our clinical trials or for other reasons such as safety concerns that might be identified through preclinical testing and animal studies or clinical trials, at any stage;
- reaching agreement on acceptable terms with prospective contract research organizations (CROs), and clinical trial sites (the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites);
- failure of CROs or other third-party contractors to comply with contractual and regulatory requirements or to perform their services in a timely or acceptable manner;
- difficulty identifying and engaging qualified clinical investigators;
- obtaining institutional review board (IRB) approval at each site;
- difficulty recruiting and enrolling patients to participate in clinical trials for a variety of reasons, including meeting the enrollment criteria for our study and competition from other clinical trial programs for the same indication as product candidates we seek to commercialize;
- having patients complete a trial or return for post-treatment follow-up;
- clinical sites deviating from trial protocol or dropping out of a trial;
- inability to retain patients in clinical trials due to the treatment protocol, personal issues, side effects from the therapy or lack of efficacy, particularly for those patients receiving a placebo;
- withdrawal of clinical trial sites from our clinical trials as a result of changing standards of care or the ineligibility of a site to participate in our clinical trials;
- adding new clinical trial sites;
- inability to identify and maintain a sufficient number of trial sites, many of which may already be engaged in other clinical trial programs, including some that may be for the same indication as our product candidates;
- changes in applicable regulatory policies and regulations;
- insufficient data to support regulatory approval;
- difficulty in maintaining contact with subjects during or after treatment, which may result in incomplete data; or
- manufacturing sufficient quantities of the product candidate for use in clinical trials.

In late 2019, a novel strain of COVID-19, also known as coronavirus, was reported in Wuhan, China and began spreading to various parts of the world. Epidemics such as this can adversely impact our business as they can cause disruptions, such as interruptions to supply chain and reduction in access to personnel and services, which could result in delays and complications with respect to our research and development programs and clinical trials. In addition, certain of our business partners and vendors are based in areas currently affected by coronavirus, which could cause additional adverse impact on our business.

Patient enrollment, a significant factor in the timing of clinical trials, is affected by many factors including the size and nature of the patient population, the proximity of patients to clinical sites, the eligibility criteria for the trial, the design of the clinical trial, competing clinical trials and clinicians' and patients' perceptions as to the potential advantages of the drug being studied in relation to other available therapies, including any new drugs that may be approved for the indications we are investigating. Furthermore, we rely on clinical trial sites to ensure the proper and timely conduct of our clinical trials and while we have agreements governing their committed activities, we have limited influence over their actual performance.

We could encounter delays if a clinical trial is suspended or terminated by us, by the IRBs of the institutions in which such trials are being conducted, by the Data Safety Monitoring Board for such trial or by the FDA or other regulatory authorities. Such authorities may impose such a suspension or termination due to a number of factors, including:

- failure by us, CROs or clinical investigators to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols;
- failed inspection of the clinical trial operations or trial site by the FDA or other regulatory authorities resulting in the imposition of a clinical hold;
- unforeseen safety or efficacy issues or any determination that a clinical trial presents unacceptable health risks;
- failure to demonstrate a benefit from using a drug; or

- lack of adequate funding to continue the clinical trial due to unforeseen costs resulting from enrollment delays, requirements to conduct additional trials and studies, increased expenses associated with the services of our CROs and other third parties, changes in governmental regulations or administrative actions, or other reasons.

If we experience delays in the completion or termination of any clinical trial of our product candidates, the commercial prospects of our product candidates will be harmed and our ability to generate product revenues from any of these product candidates will be delayed. In addition, any delays in completing our clinical trials will increase our costs, slow down our product candidate development and approval process and jeopardize our ability to commence product sales and generate revenues. Any of these occurrences may harm our business, financial condition and prospects significantly. In addition, many of the factors that cause, or lead to, a delay in the commencement or completion of clinical trials may also ultimately lead to the denial of regulatory approval of our product candidates.

If serious adverse events or other undesirable side effects are identified during the use of product candidates in investigator-sponsored trials, it may adversely affect our development of such product candidates.

Undesirable side effects caused by our product candidates could cause us or regulatory authorities to interrupt, delay or halt non-clinical studies and clinical trials, or could make it more difficult for us to enroll patients in our clinical trials. If serious adverse events or other undesirable side effects, or unexpected characteristics of our product candidates are observed in investigator-sponsored trials, further clinical development of such product candidate may be delayed or we may not be able to continue development of such product candidate at all, and the occurrence of these events could have a material adverse effect on our business. Undesirable side effects caused by our product candidates could also result in the delay or denial of regulatory approval by the FDA or other regulatory authorities or in a more restrictive label than we expect.

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

The time required to obtain approval by the FDA and comparable foreign authorities is unpredictable but typically takes many years following the commencement of clinical trials and depends upon numerous factors, including the substantial discretion of the regulatory authorities. Securing marketing approval requires the submission of extensive preclinical and clinical data and supporting information to regulatory authorities for each therapeutic indication to establish the product 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. In addition, approval policies, regulations or the type and amount of clinical data necessary to gain approval may change during the course of a product candidate's clinical development and may vary among jurisdictions. It is possible that none of our existing product candidates or any product candidates we may seek to develop in the future will ever obtain regulatory approval.

Our product candidates could fail to receive regulatory approval for many reasons, including:

- the FDA or comparable foreign regulatory authorities may disagree with the design or implementation of our clinical trials;
- we may be unable to demonstrate to the satisfaction of the FDA or comparable foreign regulatory authorities that a product candidate is safe and effective for its proposed indication;
- the results of clinical trials may not meet the level of statistical significance required by the FDA or comparable foreign regulatory authorities for approval;
- we may be unable to demonstrate that a product candidate's clinical and other benefits outweigh its safety risks;
- the FDA or comparable foreign regulatory authorities may disagree with our interpretation of data from preclinical studies or clinical trials;
- the data collected from clinical trials of our product candidates may not be sufficient to support a submission for regulatory approval in the United States or elsewhere;
- the FDA or comparable foreign regulatory authorities may fail to approve the manufacturing processes or facilities of third-party manufacturers with which we contract for clinical and commercial supplies; and/or
- the approval policies or regulations of the FDA or comparable foreign regulatory authorities may significantly change in a manner rendering our clinical data insufficient for approval.

This lengthy approval process as well as the unpredictability of future clinical trial results may result in our failing to obtain regulatory approval to market our product candidates, which would significantly harm our business, results of operations and prospects.

In addition, even if we were to obtain approval, regulatory authorities may:

- approve any of our product candidates for fewer or more limited indications than we request;
- may not approve the price we intend to charge for our products;

- may grant approval contingent on the performance of costly post-marketing clinical trials; or
- may approve a product candidate with a label that does not include the labeling claims necessary or desirable for the successful commercialization of that product candidate.

If we do not achieve our projected development goals in the timeframes we announce and expect, or we face significant competition from other biotechnology and pharmaceutical companies, the commercialization of our products may be delayed, our operating results may be lower that we expect, the credibility of our management may be adversely affected and, as a result, the value of our common stock may decline.

Even if we obtain regulatory approval for our product candidates, they may never be successfully launched or become profitable, in which case our business, prospects, operating results and financial condition may be materially harmed.

In order to successfully launch our product candidates and have them become profitable, we anticipate that we will have to dedicate substantial time and resources and hire additional personnel to expand and enhance our commercial infrastructure, which will at a minimum include the following:

- ensure the quality of the product candidate manufactured by our suppliers and by us;
- expand our sales and marketing force;
- expand and enhance programs and other procedures to educate physicians and drive physician adoption of our product candidates;
- create additional policies and procedures, and hire additional personnel to carry out those policies and procedures, to ensure customer satisfaction with our products;
- obtain reimbursement for hospitals and physicians; and/or
- expand and enhance our general and administrative operations to manage our anticipated growth in operations and to support public company activities.

Because of the numerous risks and uncertainties associated with launch and profitability of our product candidates, we are unable to predict the extent of any future losses, or when we will become profitable, if ever.

Our product candidates may have undesirable side effects that may delay or prevent marketing approval or, if approval is obtained, require them to be taken off the market, require them to include safety warnings or otherwise limit their sales.

Undesirable or unexpected side effects caused by our product candidates could cause us or regulatory authorities to interrupt, delay or halt clinical trials and could result in a more restrictive label or the delay or denial of regulatory approval by the FDA or other comparable foreign authorities. Results of our trials could reveal a high and unacceptable severity and prevalence of these or other side effects. In such an event, our trials could be suspended or terminated and the FDA or comparable foreign regulatory authorities could order us to cease further development of or deny approval of our product candidates for any or all targeted indications. The drug-related side effects could affect patient recruitment, the ability of enrolled patients to complete the trial or result in potential product liability claims. Any of these occurrences may harm our business, financial condition and prospects significantly.

Additionally, if one or more of our product candidates receives marketing approval and we or others later identify undesirable or unexpected side effects caused by such products, a number of potentially significant negative consequences could result, including:

- we could be sued and held liable for harm caused to patients;
- sales of the product may decrease significantly; and/or
- our reputation may suffer.

In addition, a regulatory agency may:

- suspend or withdraw approvals of such product;
- suspend any ongoing clinical trials;
- refuse to approve pending applications or supplements to approved applications filed by us, our collaborators or our potential future collaborators;
- require additional warnings on the label;
- require that we create a medication guide outlining the risks of such side effects for distribution to patients;
- issue warning letters;
- mandate modifications to promotional materials or require us to provide corrective information to healthcare practitioners;
- require us or our collaborators to enter into a consent decree, which can include imposition of various fines, reimbursements for inspection costs, required due dates for specific actions and penalties for noncompliance;
- impose other civil or criminal penalties;

- impose restrictions on operations, including costly new manufacturing requirements; and/or
- seize or detain products or require a product recall.

Non-compliance may also result in potential whistleblower lawsuits and the potential for liability under the False Claims Act or other laws and regulations, as discussed above. Any of these events could prevent us from achieving or maintaining market acceptance of the particular product candidate, if approved, and could significantly harm our business, results of operations and prospects.

The results of previous clinical trials may not be predictive of future results, and the results of our current and planned clinical trials may not satisfy the requirements of the FDA or non-U.S. regulatory authorities.

Clinical failure can occur at any stage of clinical development. Clinical trials may produce negative or inconclusive results, and we or any of our current and future collaborators may decide, or regulators may require us, to conduct additional clinical or preclinical testing. In addition, data obtained from tests are susceptible to varying interpretations, and regulators may not interpret data as favorably as we do, which may delay, limit or prevent regulatory approval.

We will be required to demonstrate with substantial evidence through well-controlled clinical trials that our product candidates are safe and effective for use in a diverse population before we can seek regulatory approvals for their commercial sale. Success in early clinical trials does not mean that future larger registration clinical trials will be successful because product candidates in later-stage clinical trials may fail to demonstrate sufficient safety and efficacy to the satisfaction of the FDA and non-U.S. regulatory authorities despite having progressed through initial clinical trials. Product candidates that have shown promising results in early clinical trials may still suffer significant setbacks in subsequent registration clinical trials. Similarly, the outcome of preclinical testing and early clinical trials may not be predictive of the success of later clinical trials, and preliminary and interim results of a clinical trial do not necessarily predict final results. A number of companies in the pharmaceutical industry, including those with greater resources and experience than us, have suffered significant setbacks in advanced clinical trials, even after obtaining promising results in earlier clinical trials.

Further, at various points during the course of the preclinical and clinical trial process, companies must assess both the statistical and clinical significance of trial results. In this context, “statistical significance” refers to the likelihood that a result or relationship is caused by something other than random chance or error. Statistical significance is measured by a “p-value,” which indicates the probability value that the results observed in a study were due to chance alone. A p-value of < 0.05 is generally considered statistically significant, meaning that the probability of the results occurring by chance alone is less than five percent. The lower the p-value, the less likely that the results observed were random. “Clinical significance,” on the other hand, is a qualitative assessment of the results observed. Where we use the term “clinically significant,” we have not necessarily made a formal statistical assessment of the probability that the change in patient status was attributable to the study drug as opposed to chance alone, nor does such a statement necessarily mean that study endpoints have been met or the protocol has been completed. A clinically significant effect is one that is determined to have practical importance for patients and physicians, and includes benefits that are often defined by peer-reviewed literature as having a meaningful impact on a patient’s condition. An effect that is statistically significant may or may not also be clinically significant. When a study fails to result in statistical significance, the FDA may not consider such study to serve as substantial evidence of safety and effectiveness required for approval. Even if a study results in statistical significance, the FDA may also consider clinical significance in evaluating a marketing application. For example, the FDA typically requires more than one pivotal clinical study to support approval of a new drug. However, the FDA has indicated that approval may be based on a single study in limited situations in which a trial has demonstrated a clinically significant effect. In either case, the clinical or statistical significance of a particular study result in no way guarantees that FDA or other regulators will ultimately determine that the drug being investigated is safe and effective.

In addition, the design of a clinical trial can determine whether its results will support approval of a product and flaws in the design of a clinical trial may not become apparent until the clinical trial is well advanced. We may be unable to design and execute a clinical trial to support regulatory approval.

In some instances, there can be significant variability in safety and/or efficacy results between different trials of the same product candidate due to numerous factors, including changes in trial protocols, differences in size and type of the patient populations, adherence to the dosing regimen and other trial protocols and the rate of dropout among clinical trial participants. We do not know whether any Phase 1, Phase 2, Phase 3 or other clinical trials we or any of our collaborators may conduct will demonstrate consistent or adequate efficacy and safety to obtain regulatory approval to market our product candidates.

Further, our product candidates may not be approved even if they achieve their primary endpoints in Phase 3 clinical trials or registration trials. The FDA or other non-U.S. regulatory authorities may disagree with our trial design and our interpretation of data from preclinical studies and clinical trials. In addition, any of these regulatory authorities may change requirements for the approval of a product candidate even after reviewing and providing comments or advice on a protocol for a pivotal Phase 3 clinical trial that has the potential to result in the FDA or other agencies’ approval. In addition, any of these regulatory authorities may also approve a product candidate for fewer or more limited indications than we request or may grant approval contingent on the performance of costly post-marketing clinical trials. The FDA or other non-U.S.

regulatory authorities may not approve the labeling claims that we believe would be necessary or desirable for the successful commercialization of our product candidates.

We may not be successful in our efforts to use and expand our drug discovery platforms to build a pipeline of product candidates.

A key element of our strategy is to leverage our drug discovery platforms to identify and develop new product candidates for additional diseases with significant unmet medical needs. Although our research and development efforts to date have contributed to the development of product candidates directed at inflammatory and fibrotic diseases, oncology and genetic diseases, we may not be able to develop product candidates that are safe and effective. Even if we are successful in continuing to build our pipeline, the potential product candidates that we identify may not be suitable for clinical development, including as a result of being shown to have harmful side effects or other characteristics that indicate that they are unlikely to be products that will receive marketing approval and achieve market acceptance. If we do not continue to successfully develop and begin to commercialize product candidates, we will face difficulty in obtaining product revenues in future periods, which could result in significant harm to our financial position and adversely affect the price of our common stock.

We face substantial competition, which may result in others discovering, developing and commercializing products before or more successfully than our products and product candidates.

The development and commercialization of new therapeutics is highly competitive. We face competition (from major pharmaceutical companies, specialty pharmaceutical companies and biotechnology companies worldwide) with respect to our current product candidates and will face competition with respect to any product candidates that we may seek to develop or products we commercialize in the future. We also face competition from academic research institutions, governmental agencies and other various public and private research institutions. Many of these competitors are attempting to develop therapeutics for our target indications. With the proliferation of new drugs and therapies in these areas, we expect to face increasingly intense competition as new technologies become available. Any product candidates that we successfully develop and commercialize will compete with existing therapies and new therapies that may become available in the future.

There are products already approved for many of the diseases we are targeting. Many of these approved products are well established therapies and are widely accepted by physicians, patients and third-party payors. This may make it difficult for us to achieve our business strategy of replacing existing therapies with our product candidates. Our commercial operations face significant direct competition and our competitors may develop products that are safer, more effective, more convenient or less costly than any that we are developing or that would render our product candidates obsolete or non-competitive. Our competitors may also obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for ours.

Many of our competitors have significantly greater financial, manufacturing, marketing, drug development, technical and human resources 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.

Our product candidates for which we intend to seek approval may face competition sooner than anticipated, and for biologics there is additional uncertainty as the relevant law is relatively new and there is limited precedent.

Although we plan to pursue all available FDA exclusivities for our product candidates, we may face competition sooner than anticipated. Market and data exclusivity provisions under the Federal Food, Drug, and Cosmetic Act (FDCA) can delay the submission or the approval of certain applications for competing products. The FDCA provides a five-year period of non-patent data exclusivity within the United States to the first applicant to gain approval of an NDA for a new chemical entity, running from the time of NDA approval. A drug is a new chemical entity if the 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 five-year exclusivity period for a new chemical entity, the FDA may not accept for review an ANDA or a 505(b)(2) NDA submitted by another company that references the previously approved drug. However, the FDA may accept an ANDA or 505(b)(2) NDA for review after four years if it contains a certification of patent invalidity or non-infringement.

The FDCA also provides three years of marketing exclusivity for an NDA, 505(b)(2) NDA, or supplement to an existing NDA or 505(b)(2) NDA if new clinical investigations, other than bioavailability studies, that were conducted or sponsored by the applicant are deemed by the FDA to be essential to the approval of the application, for example (for new indications, dosages, strengths or dosage forms of an existing drug). This three-year exclusivity covers only the conditions of use associated with the new clinical investigations and, as a general matter, does not prohibit the FDA from approving ANDAs or 505(b)(2) NDAs for generic versions of the original, unmodified drug product.

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.

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

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

We may expend our limited resources to pursue a particular product candidate or indication and fail to capitalize on product 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 our most promising research programs and product candidates. As a result, we may forego or delay pursuit of opportunities with other product 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 product 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 product candidate, we may relinquish valuable rights to that product 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.

We may not be able to attract collaborators or external funding for the development and commercialization of our product candidates.

Our product development programs and potential commercialization of our product candidates will require substantial additional capital to fund expenses. As part of our ongoing strategy, we may seek additional collaborative arrangements with pharmaceutical and biotechnology companies or other third parties or external funding for certain of our development programs and/or seek to expand existing collaborations to cover additional commercialization and/or development activities. We have a number of research programs and early-stage clinical development programs. At any time, we may determine that in order to continue development of a product candidate or program or successfully commercialize a drug we need to identify a collaborator or amend or expand an existing collaboration. Potentially, and depending on the circumstances, we may desire that a collaborator either agree to fund portions of a drug development program led by us, or agree to provide all the funding and directly lead the development and commercialization of a program. We face significant competition in seeking appropriate collaborators. Collaborations are complex and time-consuming to negotiate and document. We may also be restricted under existing collaboration agreements from entering into agreements on certain terms with other potential collaborators. No assurance can be given that any efforts we make to seek additional collaborative arrangements will be successfully completed on acceptable terms, a timely basis or at all.

If we are unable to negotiate favorable collaborations, we may have to curtail the development of a particular product candidate, reduce or delay its development program and its potential commercialization, reduce the scope of our sales or marketing activities, and/or increase our expenditures and undertake development or commercialization activities at our own expense. If we elect to increase our expenditures to fund development or commercialization activities on our own, we may need to obtain additional capital, which may not be available to us on acceptable terms or at all. If we do not have sufficient funds, we will not be able to bring our product candidates to market and generate product revenue.

Risks Related to Our Marketed Products

We cannot be certain how profitable, if at all, the commercialization of our marketed products will be.

We must compete effectively against other therapies with our products or any of our product candidates for which we obtain marketing approval. We may never succeed in these activities and may never generate revenues that are significant or large enough to achieve profitability.

In addition to the risks discussed elsewhere in this section, our ability to continue to generate revenues from our commercialized products will depend on a number of factors, including, but not limited to:

- achievement of broad market acceptance and coverage by third-party payors for our products;
- the effectiveness of our collaborators' efforts in marketing and selling our products;
- our ability to successfully manufacture, or have manufactured, commercial quantities of our products at acceptable cost levels and in compliance with regulatory requirements;
- our ability to maintain a cost-efficient organization and, to the extent we seek to do so, to collaborate successfully with additional third parties;
- our ability to expand and maintain intellectual property protection for our products successfully;
- the efficacy and safety of our products; and/or
- our ability to comply with regulatory requirements, which are subject to change.

Because of the numerous risks and uncertainties associated with our commercialization efforts, we may not be able to achieve profitability. Even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. A failure to become and remain profitable would depress the value of our company and could impair our ability to raise capital, expand our business, diversify our product offerings or continue our operations. A decline in the value of our company could also cause you to lose all or part of your investment.

Our inability to accurately estimate demand for our products, the uptake of new products or the timing of fluctuations in the inventories maintained by customers makes it difficult for us to accurately forecast sales and may cause our financial results to fluctuate.

We may be unable to accurately estimate demand for our products, including uptake from new products, as demand is dependent on a number of factors. We sell products primarily to wholesalers and specialty pharmacies. These customers maintain and control their own inventory levels by making estimates to determine end user demand. Our customers may not be effective in matching their inventory levels to actual end user demand. As a result, changes in inventory levels held by our customers can cause our operating results to fluctuate unexpectedly. Adverse changes in economic conditions or other factors may cause our customers to reduce their inventories of our products, which would reduce their orders from us, even if end user demand has not changed. If our inventory exceeds demand from our customers and exceeds its shelf life, we will be required to destroy unsold inventory and write off its value. As our inventory and distribution channels fluctuate from quarter to quarter, we may continue to see fluctuations in our earnings and a mismatch between prescription demand for our products and our revenues.

If we discover safety issues with any of our products or if we fail to comply with continuing U.S. and applicable foreign regulations, commercialization efforts for the product could be negatively affected, the approved product could be subject to withdrawal of approval or sales could be suspended, and our business could be materially harmed.

Our products are subject to continuing regulatory oversight, including the review of additional safety information. Drugs are more widely used by patients once approval has been obtained and therefore side effects and other problems may be observed after approval that were not seen or anticipated, or were not as prevalent or severe, during pre-approval clinical trials or nonclinical studies. The subsequent discovery of previously unknown problems with a product, or public speculation about adverse safety events, could negatively affect commercial sales of the product, result in restrictions on the product or lead to the withdrawal of the product from the market.

If we or our collaborators fail to comply with applicable continuing regulatory requirements, we or our collaborators may be subject to fines, suspension or withdrawal of regulatory approvals for specific products, product recalls and seizures, injunctions, consent decrees or other operating restrictions and/or criminal prosecutions. In addition, the manufacturers we engage to make our products and the manufacturing facilities in which our products are made are subject to periodic review and inspection by the FDA and foreign regulatory authorities. If problems are identified during the review or inspection of these manufacturers or manufacturing facilities, it could result in our inability to use the facility to make our product or a determination that inventories are not safe for commercial sale.

If physicians, nurses, pharmacists, patients, the medical community and/or third-party payors do not accept our drugs or product candidates, we may be unable to generate significant revenue in future periods.

Our drugs may not gain or maintain market acceptance among physicians, nurses, pharmacists, patients, the medical community and/or third-party payors. Effectively marketing our products and any of our product candidates, if approved, requires substantial efforts and resources, both prior to launch and after approval; and marketing efforts are subject to numerous regulatory restrictions as well as fraud and abuse laws. The demand for our drugs and degree of market acceptance of our product candidates will depend on a number of factors including:

- limitations or warnings contained in the approved labeling for any of our drugs or product candidates;
- changes in the standard of care for the targeted indications for any of our drugs or product candidates;
- lower demonstrated efficacy, safety and/or tolerability compared to other drugs;
- prevalence and severity of adverse events;
- lack of cost-effectiveness;
- limited or lack of reimbursement and coverage from government authorities, managed care plans and other third-party payors;
- a decision to wait for the approval of other therapies in development that have significant perceived advantages over our drug;
- the clinical indications for which the product is approved;
- adverse publicity about any of our drugs or product candidates or favorable publicity about competitive products;
- the timing or market introduction of any approved products as well as competitive products;
- the extent to which our drugs and product candidates are approved for inclusion on formularies of hospitals and manages care organizations;
- whether our drugs and product candidates are designated under physician treatment guidelines as first-line therapies or as a second- or third-line therapies for particular diseases;
- convenience and ease of administration;
- availability of alternative therapies at similar or lower cost, including generic and over-the-counter products;
- other potential advantages of alternative treatment methods;
- ineffective sales, marketing and/or distribution support; and/or
- potential product liability claims.

If any of our drugs or product candidates fails to maintain or achieve, as applicable, market acceptance, we will not be able to generate significant revenue in future periods.

Failure to comply with FDA promotional rules may subject us to withdrawal, and correction, of related product promotion, seizure of product and other administrative or enforcement actions as well as the potential for ancillary liability under the False Claims Act and/or product liability litigation.

The FDA regulates the promotion of our products, which may only be promoted within their approved indication for use. Promotional materials and activity must be presented with fair balance of the risks and benefits of any product in a manner which is not otherwise inaccurate or misleading. The FDCA and the FDA's implementing regulations require that manufacturers label, advertise and promote their products with appropriate safety warnings and adequate directions for their FDA-approved use. However, the FDA does not have the legal authority to regulate the practice of medicine. Although physicians are permitted, based on their medical judgment, to prescribe products for indications other than those approved by the FDA, manufacturers are prohibited from promoting their products for such off-label uses.

If the FDA determines that our promotional materials, training or other activities constitute off-label promotion, it could request that we modify our training or promotional materials or other activities or subject us to regulatory enforcement actions, including the issuance of a warning letter, injunction, seizure, civil fine and criminal penalties. Violation of the FDCA and other statutes, including the False Claims Act, relating to the promotion and advertising of prescription drugs may also lead to investigations or allegations of violations of federal and state healthcare fraud and abuse laws and state consumer protection laws. The FDA or other regulatory authorities could also request that we enter into a consent decree or a corporate integrity agreement, or seek a permanent injunction against us under which specified promotional conduct is monitored, changed or curtailed.

Although recent decisions of the United States Supreme Court, the U.S. Court of Appeals for the Second Circuit and the U.S. District Court for the Southern District of New York have clarified that the United States may not, consistent with

the First Amendment, restrict or punish a pharmaceutical manufacturer's truthful and non-misleading speech promoting the lawful use of an approved drug, there are still significant risks in this area. It is unclear how these court decisions will impact the FDA's enforcement practices, and there is likely to be substantial disagreement and difference of opinion regarding whether any particular statement is truthful and not misleading.

In the past we have been subject to enforcement action relating to allegations of improper promotion of our products, and we may be subject to such action in the future.

If we cannot successfully manage the promotion of our currently marketed products, and product candidates, if approved, we could become subject to significant liability which would materially adversely affect our business and financial condition. It is also possible that other federal, state or foreign enforcement authorities, or private parties, might take action if they believe that an alleged improper promotion led to inappropriate use of one of our products and/or the submission and payment of claims for an off-label use, which could result in significant fines or penalties under other statutory provisions, such as the False Claims Act and similar laws. Even if it is later determined that we were not in violation of these laws, we may face negative publicity, incur significant expenses defending our actions and have to divert significant management resources from other matters. In addition, there are a number of specific FDA requirements related to drug labeling and advertising, and failure to adhere to these requirements could result in our products being deemed "misbranded."

The manufacture of pharmaceutical products is a highly complex process, and if our suppliers encounter problems manufacturing our products, our business could suffer.

The manufacture of pharmaceutical products is a highly complex process, due in part to strict regulatory requirements. Problems may arise during manufacturing for a variety of reasons, including equipment malfunction, failure to follow specific protocols and procedures, problems with raw materials, delays related to the construction of new facilities or the expansion of existing facilities, including those intended to support future demand for our products, changes in manufacturing production sites and limits to manufacturing capacity due to regulatory requirements, changes in the types of products produced, physical limitations that could inhibit continuous supply, man-made or natural disasters and environmental factors. If problems arise during the production of a batch of product, that batch of product may have to be discarded and we may experience product shortages or incur added expenses. This could, among other things, lead to increased costs, lost revenue, damage to customer relationships, time and expense spent investigating the cause and, depending on the cause, similar losses with respect to other batches or products. If problems are not discovered before the product is released to the market, recall and product liability costs may also be incurred.

Risks Related to Government Regulation

If we engage in research or commercial activities involving any of our products or pipeline assets in a manner that violates federal or state healthcare laws, including fraud and abuse laws, false claims laws, disclosure laws, government price reporting and healthcare information privacy and security laws or other similar laws, we may be subject to corporate or individual civil, criminal and administrative penalties, damages, monetary fines, possible exclusion from participation in Medicare, Medicaid and other federal healthcare programs, contractual damages, reputational harm, diminished profits and future earnings, and curtailment of our operations.

Our business operations and activities are subject to extensive federal, state and local fraud and abuse and other healthcare laws and regulations, such as the False Claims Act and the federal Anti-Kickback Statute, the Foreign Corrupt Practices Act ("FCPA"), federal Physician Payment Sunshine Act, the federal Drug Supply Chain Security Act, federal Civil Monetary Penalty statute, the PPACA program integrity requirements, patient privacy laws and regulation, criminal laws relating to healthcare fraud and abuse, including but not limited to the Health Insurance Portability and Accountability Act, federal consumer protection and unfair competition laws, federal government price reporting laws and state law equivalents of each of these. These laws and regulations constrain, among other things, the business or financial arrangements and relationships through which we may research and develop any product candidate, as well as market, sell and distribute any approved products.

In addition, any sales of our products or product candidates, if approved, commercialized outside the United States will also likely subject us to foreign equivalents of the healthcare laws mentioned above, among other foreign laws.

We have entered into consulting agreements, scientific advisory board and other financial arrangements with physicians, including some who prescribe our products and may prescribe our product candidates, if approved. Compensation for some of these arrangements includes the provision of stock options. While these arrangements were structured to comply with all applicable laws, including state and federal anti-kickback laws, to the extent applicable, regulatory agencies may view these arrangements as prohibited arrangements that must be restructured, or discontinued, or for which we could be subject to other significant penalties. Moreover, while we do not submit claims and our customers make the ultimate decision on how to submit claims, we may provide reimbursement guidance and support to our customers and patients. If a government authority were to conclude that we provided improper advice to our customers and/or encouraged the submission of false claims for reimbursement, we could face action against by government authorities.

Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. The sales and marketing practices of our industry are the subject of immense scrutiny from federal and state government agencies. Despite sequestration measures, governmental enforcement funding continues at robust levels and enforcement officials are interpreting fraud and abuse laws broadly. It is possible that governmental authorities will conclude that our business practices do not comply with current or future statutes, regulations or case law interpreting applicable fraud and abuse or other healthcare laws and regulations. The risk of our being found in violation of these laws is increased by the fact that many of them have not been fully interpreted by the regulatory authorities or the courts, and their provisions are subject to a variety of interpretations. Even if we are not determined to have violated these laws, government investigations into these issues typically require the expenditure of significant resources, divert our management's attention from the operation of the business, and generate negative publicity, which could harm our business. If our past or present operations are found to be in violation of any such laws or any other governmental regulations that may apply to us, we may be subject to, without limitation, civil, criminal and administrative penalties, damages, monetary fines, disgorgement, exclusion from participation in Medicare, Medicaid and other federal healthcare programs, contractual damages, reputational harm, diminished profits and future earnings and/or the curtailment or restructuring of our operations. If we were to be excluded from federal healthcare programs, it would mean that no federal healthcare program payment could be made for any of our products.

Our commercial success depends on adequate reimbursement and coverage from third-party commercial and government payors for our products, and changes to coverage or reimbursement policies, as well as healthcare reform measures, may materially harm our sales and potential revenue.

Most patients rely on reimbursement from third-party payors, including government programs (such as Medicare and Medicaid) and private payor healthcare and insurance programs to pay for their medical needs, including any drugs we may market. Coverage and reimbursement for our products can differ significantly from payor to payor. Even when we obtain coverage and reimbursement for our products, we may not be able to maintain adequate coverage and reimbursement in the future.

There is significant uncertainty related to the third-party coverage and reimbursement of newly approved products. We intend to seek approval to market our product candidates in the United States, Europe and other selected foreign jurisdictions. Market acceptance and commercial success of our product candidates in both domestic and international markets will depend significantly on the availability of adequate coverage and reimbursement from third-party payors for any of our product candidates.

Obtaining coverage and reimbursement approval for a product from a government or other third-party payor is a time consuming and costly process that could require us to provide to the payor supporting scientific, clinical and cost-effectiveness data for the use of our products to each third-party payor separately, with no assurance that coverage and adequate reimbursement will be obtained or applied consistently. We may not be able to provide data sufficient to gain acceptance with respect to coverage and reimbursement. Additionally, coverage may be more limited than the purposes for which the product is approved by the FDA or similar regulatory authorities outside of the United States. Assuming that coverage is obtained for a given product, the resulting reimbursement rates might not be adequate or may require co-payments that patients find unacceptably high. Patients, physicians, and other healthcare providers may be less likely to prescribe, dispense or use, as applicable, our products unless coverage is provided and reimbursement is adequate to cover a significant portion of the cost of our products.

Government payors and other third-party payors, such as private health insurers and health maintenance organizations, decide which drugs they will cover and the amount of reimbursement. Coverage decisions may depend upon clinical and economic standards that disfavor new drug or biologic products when more established or lower-cost therapeutic alternatives are already available or subsequently become available. Based upon a number of factors, including clinical and economic standards, our products may not qualify for coverage and reimbursement. Coverage and reimbursement by a third-party payor may depend upon a number of factors, including, but not limited to, the third-party payor's determination that use of a product is:

- a covered benefit under its health plan;
- safe, effective and medically necessary;
- appropriate for the specific patient;
- cost-effective;
- neither experimental nor investigational;
- prescribed by a practitioner acting within the scope of license and health plan participation agreements;
- documented adequately in the patient's medical record;
- dispensed by a participating pharmacy; and/or
- logged and documented appropriately by the dispensing pharmacy.

The market for our products will depend significantly on access to third-party payors' drug formularies for which third-party payors provide coverage and reimbursement. The industry competition to be included in such formularies often leads to downward pricing pressures on pharmaceutical companies. Also, third-party payors may refuse to include a particular branded drug in their formularies or otherwise restrict patient access to a branded drug when a less costly generic equivalent or other alternative is available. If coverage and reimbursement of our future products is unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, we may be unable to achieve or sustain profitability.

In the United States, our products may be subject to discounts from list price and rebate obligations. Third-party payors have from time to time refused to include our products in their formularies, limit the type of patients for whom coverage will be provided, or restrict patient access to our products through formulary control or otherwise, in favor of less-costly generic versions of ribavirin or other treatment alternatives. Any change in formulary coverage, treatment paradigm, reimbursement levels, discounts or rebates offered on our products may impact our anticipated revenues.

In the United States, governmental and commercial third-party payors are developing increasingly sophisticated methods of controlling healthcare costs. We believe that pricing pressure for our products will continue, and future coverage and reimbursement will likely be subject to increased restrictions. For example, the PPACA, which has already imposed significant healthcare cost containment measures, also encourages the development of comparative effectiveness research and any adverse findings for our products from such research may reduce the extent of coverage and reimbursement for our products. The PPACA created the Patient-Centered Outcomes Research Institute to review the effectiveness of treatments and medications in federally-funded healthcare programs. The PCORI publishes the results of its studies. An adverse finding result may result in a treatment or product being removed from Medicare or Medicare coverage.

Managed care organizations continue to seek price discounts and in some cases, to impose restrictions on the coverage of particular drugs. Government efforts to reduce Medicaid expenses may lead to increased use of managed care organizations by Medicaid programs, which may result in managed care organizations influencing prescription decisions for a larger segment of the population, which could constrain pricing, formulary position or reimbursement for our products. Economic pressure on state budgets may also have a similar impact on Medicaid coverage and reimbursement.

In certain countries in the European Union and some other international markets, governments provide healthcare at low-cost to consumers and regulate pharmaceutical pricing, patient eligibility or reimbursement levels to control costs for the government-sponsored healthcare system. We expect to see strong efforts to reduce healthcare costs in our international markets, including: patient access restrictions; suspensions on price increases; prospective and possibly retroactive price reductions, mandatory discounts and rebates, and other recoupments; recoveries of past price increases; and greater importation of drugs from lower-cost countries to higher-cost countries. In addition, certain countries set prices by reference to the prices in other countries where our products are marketed. Thus, our inability to secure adequate prices in a particular country may not only limit the marketing of our products within that country, but may also adversely affect our ability to obtain acceptable prices in other markets.

Healthcare reform measures could hinder or prevent our product candidates' commercial success, if approved, and could increase our costs.

In both the United States and certain foreign jurisdictions, there have been, and we expect there will continue to be, a number of legislative and regulatory changes to the healthcare system that could impact our ability to sell our products profitably. Among policy makers and payors in the United States and elsewhere, there is a significant interest in promoting changes in healthcare systems with the stated goals of containing healthcare costs, improving quality and expanding individual access to healthcare. In the United States, the pharmaceutical industry has been a particular focus of these efforts and has been significantly affected by major legislative initiatives. For example, in 2010, the PPACA was enacted, which was intended to expand healthcare coverage within the United States, primarily through the imposition of health insurance mandates on employers and individuals, strengthening of program integrity measures and enforcement authority, and expansion of the Medicaid program. The PPACA substantially changes the way healthcare is financed by both governmental and private insurers and significantly affects the pharmaceutical industry. Several provisions of the new law, which have varying effective dates, may affect us and will likely increase certain of our costs. In this regard, the PPACA includes the following provisions:

- an annual, non-deductible fee on any entity that manufactures or imports certain branded prescription drugs and biologic agents, apportioned among these entities according to their market share in certain government healthcare programs that began in 2011;
- an increase in the rebates a manufacturer must pay under the Medicaid Drug Rebate Program to 23.1% and 13% of the average manufacturer price for branded and generic drugs, respectively;
- an extension of manufacturers' Medicaid rebate liability to covered drugs dispensed to individuals who are enrolled in Medicaid managed care organizations;

- new methodologies by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected, and for drugs that are line extensions;
- changes to the Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 50% point-of-sale discounts to 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;
- expansion of the entities eligible for discounts under the Public Health Service pharmaceutical pricing program;
- expansion of eligibility criteria for Medicaid programs by, among other things, allowing states to offer Medicaid coverage to additional individuals and by adding new mandatory eligibility categories for certain individuals with income at or below 133% of the Federal Poverty Level, thereby potentially increasing manufacturers' Medicaid rebate liability;
- a new requirement to annually report drug samples that manufacturers and distributors provide to licensed practitioners or to pharmacies of hospitals or other healthcare entities;
- a licensure framework for follow-on biologic products;
- a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research; and
- creation of the Independent Payment Advisory Board which has the authority to recommend certain changes to the Medicare program that could result in reduced payments for prescription drugs.

The reforms imposed by the new law will significantly impact the pharmaceutical industry; however, the full effects of the PPACA cannot be known until these provisions are implemented and the CMS and other federal and state agencies issue and finalize all applicable regulations or guidance. We will continue to evaluate the PPACA, the implementation of regulations or guidance related to various provisions of the PPACA by federal agencies, as well as trends and changes that may be encouraged by the legislation and that may potentially have an impact on our business over time. The cost of implementing more detailed record keeping systems and otherwise complying with these regulations could substantially increase our costs. The changes to the way our products are reimbursed by the CMS could reduce our revenues. Both of these situations could adversely affect our results of operations. There have been judicial and Congressional challenges to certain aspects of the PPACA, and we expect there will be additional challenges and amendments to the PPACA in the future. Significant uncertainty exists regarding the effect of the PPACA, particularly in light of the recent election and campaign pledges to repeal or reform the PPACA.

In addition, other legislative changes have been proposed and adopted since the PPACA was enacted. These changes included aggregate reductions to Medicare payments to providers and suppliers of up to 2% per fiscal year, which went into effect in April 2013 and, due to subsequent legislative amendments to the statute, will remain in effect through 2025 unless additional Congressional action is taken. In January 2013, President Obama signed into law the American Taxpayer Relief Act of 2012, which, among other things, further reduced Medicare payments to several providers and suppliers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These new laws and future healthcare reform laws may result in additional reductions in Medicare and other healthcare funding.

There also have been, and likely will continue to be, legislative and regulatory proposals at the federal and state levels and elsewhere directed at broadening the availability of healthcare and containing or lowering the cost of healthcare. In May 2018, President Trump announced a Blueprint to Lower Drug Prices and Reduce Out-of-Pocket Costs. On October 25, 2018, President Trump announced certain actions that are intended to reduce the prices Medicare will pay for drugs.

The Trump Administration has also taken executive actions to undermine or delay implementation of the PPACA. In January 2017, President Trump signed an Executive Order directing federal agencies with authorities and responsibilities under the PPACA to waive, defer, grant exemptions from, or delay the implementation of any provision of the PPACA that would impose a fiscal or regulatory burden on states, individuals, healthcare providers, health insurers, or manufacturers of pharmaceuticals or medical devices. In October 2017, the President signed a second Executive Order allowing for the use of association health plans and short-term health insurance, which may provide fewer health benefits than the plans sold through the PPACA exchanges. At the same time, the Administration announced that it will discontinue the payment of cost-sharing reduction, or CSR, payments to insurance companies until Congress approves the appropriation of funds for such CSR payments. The loss of the CSR payments is expected to increase premiums on certain policies issued by qualified health plans under the PPACA. A bipartisan bill to appropriate funds for CSR payments was introduced in the Senate, but the future of that bill is uncertain.

More recently, with enactment of the Tax Cuts and Jobs Act of 2017, which was signed by the President on December 22, 2017, Congress repealed the "individual mandate." The repeal of this provision, which requires most Americans to carry a minimal level of health insurance, became effective in 2019. According to the Congressional Budget

Office, the repeal of the individual mandate will cause 13 million fewer Americans to be insured in 2027 and premiums in insurance markets may rise. Further, each chamber of the Congress has put forth multiple bills designed to repeal or repeal and replace portions of the PPACA. Although none of these measures has been enacted by Congress to date, Congress may consider other legislation to repeal and replace elements of the PPACA.

Government price controls or other changes in pricing regulation could restrict the amount that we are able to charge for our current and future products.

International operations are also generally subject to extensive price and market regulations and there are many proposals for additional cost-containment measures, including proposals that would directly or indirectly impose additional price controls or reduce the value of our intellectual property portfolio or may make it economically unsound to launch our products in certain countries. We cannot predict the extent to which our business may be affected by these or other potential future legislative or regulatory developments. Future price controls or other changes in pricing regulation could restrict the amount that we are able to charge for our current and future products, which would adversely affect our revenue and results of operations.

Additionally, in some countries, pricing negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a product candidate. Political, economic and regulatory developments may further complicate pricing negotiations, and pricing negotiations may continue after coverage and reimbursement have been obtained. Reference pricing used by various European Union member states and parallel distribution or arbitrage between low-priced and high-priced member states, can further reduce prices. To obtain reimbursement or pricing approval in some countries, we may be required to conduct additional clinical trials that compare the cost-effectiveness of our product candidates to other available therapies, which is time-consuming and costly. If reimbursement of our product candidates is unavailable or limited in scope or amount in a particular country, or if pricing is set at unsatisfactory levels, we may be unable to achieve or sustain profitability of our products in such country.

We could be adversely affected by violations of the FCPA and similar worldwide anti-bribery laws.

We are subject to the FCPA, which generally prohibits companies and their intermediaries from making payments to non-U.S. government officials for the purpose of obtaining or retaining business or securing any other improper advantage. We are also subject to anti-bribery laws in the jurisdictions in which we operate. Although we have policies and procedures designed to ensure that we, our employees and our agents comply with the FCPA and other anti-bribery laws, there is no assurance that such policies or procedures will protect us against liability under the FCPA or other laws for actions taken by our agents, employees and intermediaries with respect to our business or any businesses that we acquire. We do business in a number of countries in which FCPA violations have recently been enforced. Failure to comply with the FCPA, other anti-bribery laws or other laws governing the conduct of business with foreign government entities, including local laws, could disrupt our business and lead to severe criminal and civil penalties, including imprisonment, criminal and civil fines, loss of our export licenses, suspension of our ability to do business with the federal government, denial of government reimbursement for our products and/or exclusion from participation in government healthcare programs. Other remedial measures could include further changes or enhancements to our procedures, policies, and controls and potential personnel changes and/or disciplinary actions, any of which could have a material adverse effect on our business, financial condition, results of operations and liquidity. We could also be adversely affected by any allegation that we violated such laws.

If our processes and systems are not compliant with regulatory requirements, we could be subject to restrictions on marketing our products or could be delayed in submitting regulatory filings seeking approvals for our product candidates.

We have a number of regulated processes and systems that are required to obtain and maintain regulatory approval for our drugs and product candidates. These processes and systems are subject to continual review and periodic inspection by the FDA and other regulatory bodies. If compliance issues are identified at any point in the development and approval process, we may experience delays in filing for regulatory approval for our product candidates, or delays in obtaining regulatory approval after filing. Any later discovery of previously unknown problems or safety issues with approved drugs or manufacturing processes, or failure to comply with regulatory requirements, may result in restrictions on such drugs or manufacturing processes, withdrawal of drugs from the market, the imposition of civil or criminal penalties or a refusal by the FDA and/or other regulatory bodies to approve pending applications for marketing approval of new drugs or supplements to approved applications, any of which could have a material adverse effect on our business. Given the number of high profile adverse safety events associated with certain drug products, regulatory authorities may require, as a condition of approval, costly risk evaluation and mitigation strategies, which may include safety surveillance, restricted distribution and use, patient education, enhanced labeling, expedited reporting of certain adverse events, pre-approval of promotional materials and restrictions on direct-to-consumer advertising. For example, any labeling approved for any of our product candidates may include a restriction on the term of its use, or it may not include one or more intended indications. Furthermore, any new legislation addressing drug safety issues could result in delays or increased costs during the period of product development, clinical trials and regulatory review and approval, as well as increased costs to assure compliance with

any new post-approval regulatory requirements. Any of these restrictions or requirements could force us or our collaborators to conduct costly studies.

In addition, we are a party to agreements that transfer responsibility for complying with specified regulatory requirements, such as packaging, storage, advertising, promotion, record-keeping and submission of safety and other post-market information on the product or compliance with manufacturing requirements, to our collaborators and third-party manufacturers. Approved products, manufacturers and manufacturers' facilities are required to comply with extensive FDA requirements, including ensuring that quality control and manufacturing procedures conform to current good manufacturing practices ("cGMP"). As such, we and our contract manufacturers, which we are responsible for overseeing and monitoring for compliance, are subject to continual review and periodic inspections to assess compliance with cGMP. Accordingly, we and others with whom we work must continue to expend time, money and effort in all areas of regulatory compliance, including manufacturing, production and quality control. The FDA may hold us responsible for any deficiencies or noncompliance of our contract manufacturers in relation to our product candidates and commercial products. If our collaborators or third-party manufacturers do not fulfill these regulatory obligations, any drugs we market or for which we or they obtain approval may be deemed adulterated, which carries significant legal implications, and may be subject to later restrictions on manufacturing or sale, which could have a material adverse effect on our business.

Risks Related to Our Intellectual Property Rights

If we are unable to obtain and maintain patent protection for our products and product candidates, or if the scope of the patent protection obtained is not sufficiently broad, our competitors could develop and commercialize products and product candidates similar or identical to ours, and our ability to successfully commercialize our products and product candidates may be adversely affected.

Our commercial success will depend, in part, on our ability to obtain and maintain patent protection in the United States and other countries with respect to our products and product candidates. We seek to protect our proprietary position by filing patent applications in the United States and abroad related to our products and product candidates that are important to our business. We cannot be certain that patents will be issued or granted with respect to applications that are currently pending or that we apply for in the future with respect to one or more of our products and product candidates, or that issued or granted patents will not later be found to be invalid and/or unenforceable.

The patent prosecution process is expensive and time-consuming, and we may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. Although we enter into non-disclosure and confidentiality agreements with parties who have access to patentable aspects of our research and development output, such as our employees, collaboration partners, consultants, advisors and other third parties, 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.

We may license patent rights that are valuable to our business from third parties, in which event we may not have the right to control the preparation, filing and prosecution of patent applications, or to maintain the patents, covering technology or medicines underlying such licenses. We cannot be certain that these patents and applications will be prosecuted and enforced in a manner consistent with the best interests of our business. If any such licensors fail to maintain such patents, or lose rights to those patents, the rights we have licensed may be reduced or eliminated and our right to develop and commercialize any of our products that are the subject of such licensed rights could be adversely affected. In addition to the foregoing, the risks associated with patent rights that we license from third parties also apply to patent rights we own.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal and factual questions and 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. Our pending and future patent applications may not result in patents being issued, and even if issued, the patents may not meaningfully protect our products or product candidates, effectively prevent competitors and third parties from commercializing competitive products or otherwise provide us with any competitive advantage. Our competitors or other third parties may be able to circumvent our patents by developing similar or alternative products in a non-infringing manner. Changes in the patent laws, implementing regulations or interpretation of the patent laws in the United States and other countries may also diminish the value of our patents or narrow the scope of our patent protection.

The laws of foreign countries may not protect our rights to the same extent as the laws of the United States, and many companies have encountered significant difficulties in protecting and defending such rights in foreign jurisdictions. For those countries where we do not have granted patents, we may not have any ability to prevent the unauthorized use or sale of our proprietary medicines and technology or to prevent third parties from selling or importing products made using our inventions in and into the United States and other jurisdictions. 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, but enforcement is not as strong as that in the United States. These products

may compete with our products and our intellectual property rights may not be effective or sufficient to prevent them from competing.

We may not be aware of all third-party intellectual property rights potentially relating to our product candidates. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing, or in some cases not at all. Therefore, we cannot be certain that we were the first to make the inventions claimed in our owned or any licensed patents or pending patent applications, or that we were the first to file for patent protection of such inventions.

Assuming the other requirements for patentability are met, prior to March 2013, in the United States, the first to make the claimed invention was entitled to the patent, while outside the United States, the first to file a patent application was entitled to the patent. Beginning in March 2013, the United States transitioned to a first-inventor-to-file system in which, assuming the other requirements for patentability are met, the first-inventor-to-file a patent application will be entitled to the patent. We may be subject to a third-party preissuance submission of prior art to the U.S. Patent and Trademark Office (U.S. PTO) or become involved in opposition, derivation, revocation, reexamination, post-grant and *inter partes* review or interference proceedings challenging our patent rights or the patent rights of others. Participation in these proceedings can be very complex, expensive and may divert our management's attention from our core business. Furthermore, an adverse determination in any such submission, proceeding or litigation could reduce the scope of, or invalidate, our patent rights, allow third parties to commercialize our technology or products and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize medicines without infringing third-party patent rights.

Even if our patent applications do issue as patents, they may not issue in a form that will provide us with any meaningful protection, prevent competitors or other third parties from competing with us or otherwise provide us with any competitive advantage. Our competitors or other third parties may be able to circumvent our patents by developing similar or alternative technologies or products in a non-infringing manner.

The issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, and our patents may be challenged in the courts or patent offices in the United States and abroad. Such challenges may result in loss of exclusivity or in patent claims being narrowed, invalidated or held unenforceable, which could limit our ability to stop others from using or commercializing similar or identical products, or limit the duration of the patent protection of our products and product candidates. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our intellectual property may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours. Patent protection may not be available for some of our products or the processes under which they are used or manufactured.

Issued patents covering one or more of our products could be found invalid or unenforceable if challenged in court.

If we or one of our licensing partners initiated legal proceedings against a third party to enforce a patent covering one of our product candidates, the defendant could counterclaim that the patent covering our product candidate is invalid and/or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity and/or unenforceability are commonplace. Although we have conducted due diligence on patents we have exclusively in-licensed, the outcome following legal assertions of invalidity and unenforceability during patent litigation is unpredictable. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including lack of novelty, obviousness or non-enablement. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld relevant information from the U.S. PTO, or made a misleading statement, during prosecution. Third parties may also raise similar claims before administrative bodies in the United States or abroad, even outside the context of litigation. Such mechanisms include re-examination, post-grant review, inter partes review, interference proceedings, derivation proceedings, and equivalent proceedings in foreign jurisdictions (e.g., opposition proceedings). Such proceedings could result in revocation or amendment to our patents in such a way that they no longer cover our products and product candidates. The outcome following legal assertions of invalidity and unenforceability is unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art of which we and the patent examiner were unaware during prosecution. If a defendant were to prevail on a legal assertion of invalidity and/or unenforceability, we would lose at least part, and perhaps all, of the patent protection on our product candidates. Such a loss of patent protection would have a material adverse impact on our business.

Third-party claims of intellectual property infringement, misappropriation or other violation may prevent or delay our development and commercialization efforts.

Our commercial success depends in part on our avoiding infringement of the patents and proprietary rights of third parties. There is a substantial amount of litigation, both within and outside the United States, involving patent and other intellectual property rights in the pharmaceutical industries, including patent infringement lawsuits, interferences, oppositions and *inter partes* reexamination proceedings before the U.S. PTO, and corresponding foreign patent offices. Numerous U.S. and foreign issued patents and pending patent applications, which are owned by third parties, exist in the fields in which we are pursuing development candidates. As the biotechnology and pharmaceutical industries expand and more patents are

issued, the risk increases that our product candidates may be subject to claims of infringement of the patent rights of third parties.

Third parties may assert that we are employing their proprietary technology without authorization and may assert infringement claims against us based on existing patents or patents that may be granted in the future, regardless of their merit. There may be third-party patents or patent applications with claims to materials, formulations, methods of manufacture or methods for treatment related to the use or manufacture of our product candidates. Because patent applications can take many years to issue, there may be currently pending patent applications which may later result in issued patents that our product candidates may infringe. In addition, third parties may obtain patents in the future and claim that use of our technologies infringes upon these patents. If any third-party patents were held by a court of competent jurisdiction to cover the manufacturing process of any of our product candidates, any molecules formed during the manufacturing process or any final product itself, the holders of any such patents may be able to block our ability to commercialize such product unless we obtained a license under the applicable patents, or until such patents expire.

Similarly, if any third-party patents were held by a court of competent jurisdiction to cover aspects of our formulations, processes for manufacture or methods of use, the holders of any such patents may be able to block our ability to develop and commercialize the applicable product unless we obtained a license or until such patent expires. In either case, such a license may not be available on commercially reasonable terms or at all. Even if we or our future strategic collaborators were able to obtain a license, the rights may be nonexclusive, which could result in our competitors gaining access to the same intellectual property and it could require us to make substantial licensing and royalty payments.

Parties making claims against us may obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize one or more of our product candidates. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee resources from our business. In the event of a successful claim of infringement against us, we may have to pay substantial damages, including treble damages and attorneys' fees for willful infringement, pay royalties, redesign our infringing products or obtain one or more licenses from third parties, which may be impossible or require substantial time and monetary expenditure. 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.

Most of our competitors are larger than we are and have substantially greater resources and may be able to sustain the costs of complex patent litigation longer than we could. The uncertainties associated with litigation could have a material adverse effect on our ability to raise the funds necessary to continue our clinical trials, continue our internal research programs, in-license needed technology or enter into strategic collaborations that would help us bring our product candidates to market.

We may be involved in lawsuits to protect or enforce our patents or the patents of our licensors, which could be expensive, time-consuming and unsuccessful.

Competitors may infringe our patents or the patents of our licensors. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time-consuming. In addition, in an infringement proceeding, a court may decide that a patent of ours or our licensors is not valid, is unenforceable and/or is not infringed, or may refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the technology in question. Interference proceedings provoked by third parties or brought by us may be necessary to determine the priority of inventions with respect to our patents or patent applications or those of our licensors. An adverse result in any litigation or defense proceedings could put one or more of our patents at risk of being invalidated or interpreted narrowly and could put our patent applications at risk of not issuing.

An unfavorable outcome could require us to cease using the related technology or to attempt to license rights to it from the prevailing party. Our business could be harmed if the prevailing party does not offer us a license on commercially reasonable terms. Our defense of litigation or interference proceedings may fail and, even if successful, may result in substantial costs and distract our management and other employees. We may not be able to prevent, alone or with our licensors, misappropriation of our intellectual property rights, particularly in countries where the laws may not protect those rights as fully as in the United States.

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

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

In addition to patents, we rely on trade secrets, technical know-how and proprietary information concerning our business strategy in order to protect our competitive position in medical research and development. Trade secrets are difficult

to protect, and it is possible that our trade secrets and know-how will over time be disseminated within the industry through independent development and intentional or inadvertent disclosures.

We seek to protect our trade secrets, in part, by entering into non-disclosure and confidentiality agreements with parties who have access to them, such as our employees, collaboration partners, consultants, advisors and other third parties. We also enter into confidentiality and invention or patent assignment agreements with our employees and 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. Our agreements with research and development collaboration partners contain contractual limitations regarding the publication and public disclosure of data and other information generated during the course of research. Despite these efforts, any of these parties may breach the agreements and intentionally or inadvertently disclose or use 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 third party illegally obtained and is using any of our trade secrets is expensive and time consuming and the outcome is unpredictable. In addition, some courts inside and outside the United States are less willing or unwilling to protect trade secrets. If any of our trade secrets or the equivalent knowledge, methods and know-how were to be lawfully obtained or independently developed by a competitor or other third party, we would have no right to prevent them from using that technology or information to compete with us. If any of our trade secrets were to be disclosed to or independently developed by a competitor or other third party, our competitive position would be harmed. If we do not apply for patent protection prior to such publication or if we cannot otherwise maintain the confidentiality of our proprietary technology and other confidential information, then our ability to obtain patent protection or to protect our trade secret information may be jeopardized.

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

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

- others may be able to make compounds that are similar to our product candidates but that are not covered by the claims of the patents that we own or have exclusively licensed;
- we or our licensors or collaboration partners might not have been the first to make the inventions covered by the issued patent or pending patent application that we own or have exclusively licensed;
- we or our licensors or collaboration partners 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 our intellectual property rights;
- it is possible that our pending patent applications will not lead to issued patents;
- issued patents that we own or have exclusively licensed may not provide us with any competitive advantages or may be held invalid or unenforceable, as a result of legal challenges by our competitors;
- our competitors 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; and/or
- the patents of others may have an adverse effect on our business.

Should any of these events occur, they could significantly harm our business, results of operations and prospects.

We may be subject to claims that our employees, consultants or independent contractors have wrongfully used or disclosed confidential information of third parties or that our employees have wrongfully used or disclosed alleged trade secrets of their former employers.

As is common in the biotechnology and pharmaceutical industry, we employ individuals who were previously employed at universities or other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that our employees, consultants and independent contractors 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 our employees, consultants or independent contractors have inadvertently or otherwise used or disclosed intellectual property, including trade secrets or other proprietary information, of any of our employees' former employer or other third parties. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel, which could adversely impact our business. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees.

We may be subject to claims challenging the inventorship or ownership of our patents and other intellectual property.

We may also be subject to claims that former employees, collaborators or other third parties have an ownership interest in our patents or other intellectual property. We may also have, in the future, ownership disputes arising, for example, from conflicting obligations of consultants or others who are involved in developing our product candidates. Litigation may be necessary to defend against these and other claims challenging inventorship or ownership. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of, or right to use, valuable intellectual property. Such an outcome could have a material adverse effect on our business. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees.

If we do not obtain patent term extension for any product candidates we may develop, our business may be materially harmed.

Depending upon the timing, duration and specifics of any FDA marketing approval of any product candidates we may develop, 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. The Hatch-Waxman Amendments permit a patent extension term of up to five years as compensation for patent term lost during the FDA 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. However, 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. Moreover, the applicable time period or the scope of patent protection afforded could be less than we request. If we are unable to obtain any patent term extension or the term of any such extension is less than we request, third parties may obtain approval of competing products following our patent expiration, and our business, financial condition, results of operations, and prospects could be materially harmed.

Risks Related to Our Dependence on Third Parties

We expect to continue to contract with third-party suppliers for the production of our commercial product portfolio as well as our developmental product candidates for clinical trial use and, if approved, for commercialization.

We currently employ third parties for the manufacturing of our commercial products and product candidates. This increases the risk that we will not have sufficient quantities of our products or product candidates within the timeframe and at an acceptable cost which could delay, prevent or impair our development or commercialization efforts. Additionally, we may not be able to quickly respond to changes in customer demand which could harm our business as a result of the inability to supply the market or an excess of inventory that we are unable to sell.

The facilities used by our contract manufacturers to manufacture our product candidates must adhere to FDA requirements, and are subject to inspections that may be conducted after we submit our marketing applications to the FDA in connection with review of our application, and on an ongoing basis relevant to postmarketing compliance. Although we are subject to regulatory responsibility for the quality of products manufactured by our contract manufacturers and oversight of their activities, we do not control the manufacturing process of, and are completely dependent on, our contract manufacturing partners for compliance with the regulatory requirements, known as cGMPs, for manufacture of both active drug substances and finished drug products. If our contract manufacturers cannot successfully manufacture material that conforms to our specifications and the strict regulatory requirements of the FDA or others, they will be subject to enforcement action, and if substantial noncompliance is identified and not corrected, they may be precluded from manufacturing product for the United States or other markets. In addition, although the FDA will hold us responsible for due diligence in the selection of, and oversight in the operations of, our contract manufacturers, we do not have direct control over the ability of our contract manufacturers to maintain adequate quality control, quality assurance and qualified personnel. If the FDA or a comparable foreign regulatory authority identified significant compliance concerns with our contract manufacturers, we may need to find alternative manufacturing facilities, which would significantly impact our ability to develop, obtain regulatory approval for or market our products or product candidates, if approved.

We have agreements with third-party manufacturers for the provision of active pharmaceutical ingredient (API), drug product manufacturing and packaging of our commercial products. Reliance on third-party manufacturers carries additional risks, such as not being able to comply with cGMP or similar regulatory requirements outside 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 fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, license revocation, seizures or recalls of products, operating restrictions and criminal prosecutions, any of which could significantly and adversely affect supplies of our products.

Due to FDA requirements and other factors, we are generally unable to make changes to our supplier arrangements without delay. Manufacturing services related to each of our pharmaceutical products are primarily provided by a single

source. Each of our raw materials are also provided by a single source. We attempt to mitigate this risk through long-term contracts and inventory safety stock. In the event that any of these third-party manufacturers fail regulatory compliance, fail to meet quality assurance specifications or experience an unavoidable extraordinary event, our business could be adversely affected.

Any products that we may develop may compete with other product candidates and commercialized products for access to manufacturing facilities. There are a limited number of manufacturers that operate under cGMP regulations and that might be capable of manufacturing for us. Any performance failure or refusal to supply on the part of our existing or future suppliers could delay clinical development, marketing approval or commercialization of our products. If our current suppliers cannot perform as agreed, we may be required to replace one or more of these suppliers. Although we believe that there are a number of potential long-term replacements to each supplier, we may incur added costs and delays in identifying and qualifying any such replacements.

We rely on third parties to store and distribute supplies for our clinical trials and for the manufacture of our product candidates. Any performance failure on the part of our existing or future distributors could delay clinical development or regulatory approval or our product candidates or commercialization of our products, producing additional losses and depriving us of potential product revenue.

We have acquired or in-licensed many of our products from external sources and may owe milestones or royalties based on the achievement of future successes or penalties if certain diligence requirements are not met.

In certain cases, our license or acquisition agreements require us to conduct research or clinical trials within a specified time frame, or we may owe a penalty or lose the right to the product for development. If we do not conduct the necessary research or clinical trials within the specified time frame, we may be required to pay cash penalties to extend the time frame during which studies may be conducted, our licensors may exercise a right to have the product returned or may have the right to terminate the agreement, in which event we would not be able to market products covered by such agreement.

On some of the products we have licensed, we may be obligated in future periods to make significant development and commercial milestone payments as well as royalties. As a result, we may have to raise additional capital (which would likely cause our equity holders to experience dilution) to cover the required milestone payments. The milestone payments and royalties we may owe on the sale of our products may reduce the overall profitability of our operations and if we are unable to sell sufficient product to cover the costs of these milestone payments, our operating profitability, business and value of our equity securities may be adversely impacted.

We depend on intellectual property licensed from third parties and termination of any of these licenses could result in the loss of significant rights, which would harm our business.

We are dependent on patents, know-how and proprietary technology, both our own and licensed from others. We are party to intellectual property license agreements with third parties and expect to enter into additional license agreements in the future. Our current license agreements impose, and we expect that future license agreements will impose, various diligence, development, commercialization, payment and other obligations. If we fail to comply with our obligations under these agreements, the licensor may have the right to terminate the license agreement or may exercise a right to have the intellectual property that we license returned. For example, under our exclusive sub-license agreement for KD025 with NT Life and SLx, if we fail to comply with our diligence obligations, the former owners of the intellectual property licensed under such agreement may require us and our licensors to return such intellectual property, in which case our license to such intellectual property would terminate. Any termination of these licenses could result in the loss of significant rights and could have a material adverse effect on our ability to commercialize our product candidates, including KD025.

Disputes may also arise between us and our licensors regarding intellectual property subject to a license agreement, including those relating to:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- whether and the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the license agreement;
- our right to sublicense patent and other rights to third parties under collaborative development relationships;
- whether we are complying with our diligence obligations with respect to the use of the licensed technology in relation to our development and commercialization of our product candidates; and/or
- the allocation of ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and by us and our collaboration partners.

If disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates. We are generally also subject to all of the same risks with respect to protection of intellectual property

that we license as we are for intellectual property that we own. If we or our licensors fail to adequately protect this intellectual property, our ability to commercialize our products could suffer.

We depend, in part, on our licensors to file, prosecute, maintain, defend and enforce patents and patent applications that are material to our business.

In certain of our license agreements, the patents relating to our product candidates are controlled by certain of our licensors. Such licensors generally have rights to file, prosecute, maintain and defend the patents we have licensed from such licensors. We generally have the first right to enforce our patent rights, although our ability to settle such claims often requires the consent of the licensor. If our licensors or any future licensees having rights to file, prosecute, maintain or defend our patent rights fail to conduct these activities for patents or patent applications covering any of our product candidates, our ability to develop and commercialize those product candidates may be adversely affected and we may not be able to prevent competitors from making, using or selling competing products. We cannot be certain that such activities by our licensors have been or will be conducted in compliance with applicable laws and regulations or will result in valid and enforceable patents or other intellectual property rights.

Pursuant to the terms of the license agreements with some of our licensors, the licensors may have the right to control enforcement of our licensed patents or defense of any claims asserting the invalidity of these patents and, even if we are permitted to pursue such enforcement or defense, we cannot ensure the cooperation of our licensors. We cannot be certain that our licensors will allocate sufficient resources or prioritize their or our enforcement of such patents or defense of such claims to protect our interests in the licensed patents. Even if we are not a party to these legal actions, an adverse outcome could harm our business because it might prevent us from continuing to license intellectual property that we may need to operate our business. In addition, even when we have the right to control patent prosecution of licensed patents and patent applications, enforcement of licensed patents, or defense of claims asserting the invalidity of those patents, we may still be adversely affected or prejudiced by actions or inactions of our licensors and their counsel that took place prior to or after our assuming control.

We rely in part 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 do not independently conduct clinical trials of our product candidates. We rely on third parties, such as medical institutions and clinical investigators, and may in the future rely on other third parties, to perform this function. Our reliance on these third parties for clinical development activities reduces our control over these activities but does not relieve us of our responsibilities. We remain responsible for ensuring that each of our clinical trials is conducted in accordance with the general investigational plan and protocols for the trial. Moreover, we, along with medical institutions and clinical investigators, are required to comply with “good clinical practices” or “GCP,” which is an international ethical and scientific quality standard for designating, recording and reporting trials that involve the participation of human subjects, and which is implemented via regulations and guidelines enforced by, among others, the FDA, the EMA, the Competent Authorities of the Member States of the European Economic Area (EEA), and comparable foreign regulatory authorities for all of our products in clinical development. GCP is designed to assure that data and reported results are credible and accurate and that the rights, integrity and confidentiality of patients in clinical trials are protected. Regulatory authorities enforce these GCPs through periodic inspections of trial sponsors, principal investigators and trial sites. If we or any of our CROs, study sites, or clinical investigators fail to comply with applicable GCPs, the clinical data generated in our clinical trials may be deemed unreliable and the FDA, EMA or comparable foreign regulatory authorities may require us to perform additional clinical trials before approving our marketing applications. We cannot assure you that upon inspection by a given regulatory authority, such regulatory authority will determine that any of our clinical trials comply with GCP regulations. In addition, our clinical trials must be conducted with product produced under cGMP regulations. Our failure to comply with these regulations may require us to repeat clinical trials and create other regulatory and litigation exposure, which would among other things delay the regulatory approval process.

We face risks in connection with existing and future collaborations with respect to the development, manufacture and commercialization of our products and product candidates.

The risks that we face in connection with our current and any future collaborations include the following:

- Our collaborators may change the focus of their development and commercialization efforts or may have insufficient resources to effectively develop our product candidates. The ability of some of our products and product candidates to reach their potential could be limited if collaborators decrease or fail to increase development or commercialization efforts related to those products or product candidates.
- Any future collaboration agreements may have the effect of limiting the areas of research and development that we may pursue, either alone or in collaboration with third parties.
- Collaborators may develop and commercialize, either alone or with others, drugs that are similar to or competitive with the drugs or product candidates that are the subject of their collaborations with us.

Our collaboration agreements are subject to termination under various circumstances.

Risks Related to Our Operations

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

The biopharmaceutical industry has experienced a high rate of turnover of management personnel in recent years. Our ability to compete in the highly competitive biotechnology and pharmaceuticals industries depends upon our ability to attract and retain highly qualified managerial, scientific and medical personnel.

Recruiting and retaining qualified scientific, clinical, manufacturing and sales and marketing personnel will also be critical to our success. We may not be able to attract and retain these 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. This may limit their availability to us.

In order to induce valuable employees to continue their employment with us, we have provided equity incentives that vest over time. The value to employees of equity incentives that vest over time is significantly affected by the success of our operations and clinical trials for our product candidates, much of which is beyond our control, and may at any time be insufficient to counteract more lucrative offers from other companies.

Despite our efforts to retain valuable employees, members of our management, scientific and development teams may terminate their employment with us on short notice. Our employment arrangements generally provide for at-will employment, which means that any of our employees could leave our employment at any time, with or without notice. The loss of the services of any of our executive officers or other key employees and our inability to find suitable replacements could potentially harm our business, financial condition and prospects. Our success also depends on our ability to continue to attract, retain and motivate highly skilled junior, mid-level and senior managers as well as junior, mid-level and senior scientific and medical personnel.

We may not be able to attract or retain qualified management and scientific personnel in the future due to the intense competition for a limited number of qualified personnel among biopharmaceutical, biotechnology, pharmaceutical and other businesses and institutions. Many of the other companies and institutions that we compete with for qualified personnel have greater financial and other resources, different risk profiles and a longer history in the industry than we do. They also may provide more diverse opportunities and better chances for career advancement. Some of these characteristics may be more appealing to high quality candidates than what we have to offer. If we are unable to continue to attract and retain high quality personnel, the rate and success at which we can develop and commercialize product candidates will be limited.

Our employees, independent contractors, principal investigators, agents, consultants, commercial partners and vendors may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements, which could have a material adverse effect on our business.

We are exposed to the risk that our employees, independent contractors, principal investigators, agents, consultants, commercial partners and vendors may engage in fraudulent conduct or other illegal activity. Misconduct by these parties could include intentional, reckless and/or negligent failures to:

- comply with regulations by the FDA and other similar foreign regulatory bodies;
- provide true, complete and accurate information to the FDA and other similar foreign regulatory bodies;
- comply with manufacturing standards;
- comply with federal and state data privacy, security, fraud and abuse and other healthcare laws and regulations in the United States and similar foreign laws;
- report financial information or data accurately; and/or
- disclose unauthorized activities to us.

In particular, the promotion, sales and marketing of healthcare items and services, as well as certain business arrangements in the healthcare industry, are subject to extensive laws and regulations intended to prevent fraud, misconduct, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, including off-label uses of our products, structuring and commission(s), certain customer incentive programs, patient assistance programs, and other business arrangements generally. Activities subject to these laws also involve the improper use or misrepresentation of information obtained in the course of clinical trials, creating fraudulent data in our preclinical studies or clinical trials or illegal misappropriation of drug product, which could result in regulatory sanctions and serious harm to our reputation. We have adopted a Code of Business Ethics. However, it is not always possible to identify and deter misconduct by employees and other third-parties, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in

protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws or regulations. Additionally, we are subject to the risk that a person could allege such fraud or other misconduct, even if none occurred. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business and results of operations, including the imposition of significant civil, criminal and administrative penalties, damages, fines, possible exclusion from participation in Medicare, Medicaid and other federal healthcare programs or other sanctions, contractual damages, reputational harm, diminished profits and future earnings, and curtailment of our operations.

If product liability lawsuits are brought against us, we may incur substantial liabilities and may be required to limit commercialization of our product candidates and marketed products.

We face an inherent risk of product liability as a result of the clinical testing of our product candidates, whether by us, on our behalf or by unaffiliated third parties or investigators, and will face an even greater risk for any products that we commercialize. For example, we may be sued if any product we develop or sell allegedly causes injury or is found to be otherwise unsuitable during product testing, manufacturing, marketing or sale. Any such product liability claims may include allegations of defects in manufacturing, defects in design, a failure to warn of dangers inherent in the product, negligence, strict liability, and a breach of warranties. Claims could also be asserted under state consumer protection acts. If we cannot successfully defend ourselves against product liability claims, we may incur substantial liabilities or be required to limit commercialization of our product candidates, if approved, or our other marketed products. Even a successful defense would require significant financial and management resources. Regardless of the merits or eventual outcome, liability claims may result in:

- decreased demand for our product candidates or products that we may develop or sell;
- injury to our reputation;
- withdrawal of clinical trial participants;
- initiation of investigations by regulators;
- costs to defend the related litigation;
- a diversion of management's time and our resources;
- substantial monetary awards to trial participants or patients;
- product recalls, withdrawals or labeling, marketing or promotional restrictions;
- loss of revenues from product sales; and/or
- the inability to commercialize our product candidates or our marketed products.

Our inability to obtain and retain sufficient product liability insurance at an acceptable cost to protect against potential product liability claims could prevent or inhibit the commercialization of products we develop. We currently carry an aggregate of $10.0 million of product liability insurance, which we believe is adequate for our commercial products and our clinical trials. Although we maintain such insurance, any claim that may be brought against us could result in a court judgment or settlement in an amount that is not covered, in whole or in part, by our insurance or that is in excess of the limits of our insurance coverage. Our insurance policies also have various exclusions and we may be subject to a product liability claim for which we have no coverage. We will have to pay any amounts awarded by a court or negotiated in a settlement that exceed our coverage limitations or that are not covered by our insurance, and we may not have, or be able to obtain, sufficient capital to pay such amounts.

Our operating results are subject to significant fluctuations.

Our quarterly revenues, expenses and net income (loss) have fluctuated in the past and are likely to fluctuate significantly in the future due to the timing of charges and expenses that we may encounter. In recent periods, for instance, we have recorded charges that include:

- unrealized gain (losses) on investments in equity securities; and
- gains (losses) related to the change in fair value of financial instruments

Our quarterly revenues, expenses and net income (loss) may fluctuate significantly from quarter to quarter and year to year, such that a period to period comparison of our results of operations may not be a good indication of our future performance.

Specifically, the market price of our investment in MeiraGTx may be volatile and fluctuate substantially, which could result in significant changes to the fair value of our investment and limit our ability to sell those securities. The stock market in general and the market for smaller biopharmaceutical companies in particular have experienced extreme volatility that has often been unrelated or disproportionate to the operating performance of particular companies. Broad market and industry factors may negatively affect the market price of MeiraGTx common stock, regardless of its actual operating performance. Further, a decline in the financial markets and related factors beyond our control or the control of MeiraGTx may cause the price of MeiraGTx to decline rapidly and unexpectedly. In addition, MeiraGTx may require significant

additional capital to continue its planned operations. To raise capital, MeiraGTx may sell equity securities, convertible securities or other securities in one or more transactions, which may result in material dilution of our investment in MeiraGTx and result in additional volatility in the fair value of our investment in MeiraGTx. As a result of this volatility, the fair value of our investment in MeiraGTx may be significantly and adversely affected. Investments in common stock of companies traded on public markets, including our MeiraGTx investment, are reflected on our balance sheet at fair value based on the closing price of the shares owned on the last trading day before the date of the balance sheet. Fluctuations in the underlying bid price of the shares result in unrealized gains or losses. In accordance with FASB ASC 321, Investments – Equity Securities, we recognize these fluctuations in value as other expense (income). Accordingly, a decline in the trading price of MeiraGTx would require us to recognize unrealized losses, which could result in significant harm to our financial position and adversely affect the price of our common stock. Furthermore, as a result of this volatility, we may not be able to sell our common stock of MeiraGTx at prices we find attractive, or we may be required to recognize realized losses if we sell MeiraGTx common stock. In October 2019, the Company entered into a transaction pursuant to which it sold approximately 1.4 million ordinary shares of MeiraGTx for gross proceeds of $22.0 million. After consummation of the transaction, the Company held approximately 5.7% of the outstanding ordinary shares of MeiraGTx with a fair value of $42.0 million at December 31, 2019.

If we are unable to successfully implement our strategic plan, our business may be materially harmed.

We plan to continue to develop and commercialize novel drugs for significant unmet medical needs while we continue to market our commercial products to eligible patients to generate revenue. Absent a successful launch of one or more of our product candidates, we expect our total revenue to decline significantly. In order to maintain a strong financial position, we are focusing our investment on development programs for our most advanced product candidates. In an effort to mitigate our drug development risk and improve our chance of ultimate commercial success, we are developing multiple product candidates in a variety of disease indications. There can be no assurance that our development programs will be successful or that our research programs will result in drugs that we can successfully develop and commercialize.

Our business may become subject to economic, political, regulatory and other risks associated with international operations.

Our business is subject to risks associated with conducting business internationally. Some of our suppliers and collaborative and clinical trial relationships are located outside the United States. Accordingly, our future results could be harmed by a variety of factors, including:

- economic weakness, including inflation, or political instability in particular foreign economies and markets;
- differing regulatory requirements for drug approvals in foreign countries;
- potentially reduced protection for intellectual property rights;
- difficulties in compliance with non-U.S. laws and regulations;
- changes in non-U.S. regulations and customs, tariffs and trade barriers;
- changes in non-U.S. currency exchange rates and currency controls;
- changes in a specific country’s or region’s political or economic environment;
- trade protection measures, import or export licensing requirements or other restrictive actions by U.S. or non-U.S. governments;
- negative consequences from changes in tax laws;
- compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;
- workforce uncertainty in countries where labor unrest is more common than in the United States;
- difficulties associated with staffing and managing foreign operations, including differing labor relations;
- production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad; and/or
- business interruptions resulting from geopolitical actions, including war and terrorism, or natural disasters including earthquakes, typhoons, floods and fires.

If we engage in future acquisitions or strategic collaborations, this may increase our capital requirements, dilute our equity holders, cause us to incur debt or assume contingent liabilities, and subject us to other risks.

We may evaluate various acquisitions and strategic collaborations, including licensing or acquiring complementary products, intellectual property rights, technologies or businesses. Any potential acquisition or strategic collaboration may entail numerous risks, including:

- increased operating expenses and cash requirements;
- the assumption of additional indebtedness or contingent liabilities;

- assimilation of operations, intellectual property and products of an acquired company, including difficulties associated with integrating new personnel;
- the diversion of our management's attention from our existing product programs and initiatives in pursuing such a strategic merger or acquisition;
- retention of key employees, the loss of key personnel, and uncertainties in our ability to maintain key business relationships;
- risks and uncertainties associated with the other party to such a transaction, including the prospects of that party and their existing products or product candidates and regulatory approvals; and/or
- our inability to generate revenue from acquired technology and/or products sufficient to meet our objectives in undertaking the acquisition or even to offset the associated acquisition and maintenance costs.

In addition, if we undertake acquisitions, we may issue dilutive securities, assume or incur debt obligations, incur large one-time expenses and acquire intangible assets that could result in significant future amortization expense. Moreover, we may not be able to locate suitable acquisition opportunities and this inability could impair our ability to grow or obtain access to technology or products that may be important to the development of our business.

If we acquire or license technologies, products or product candidates, we will incur a variety of costs and may never realize benefits from the transaction.

If appropriate opportunities become available, we might license or acquire technologies, resources, drugs or product candidates. We might never realize the anticipated benefits of such a transaction, and we may later incur impairment charges related to assets acquired in any such transaction. In particular, due to the risks inherent in drug development, we may not successfully develop or obtain marketing approval for the product candidates we acquire. Future licenses or acquisitions could result in potentially dilutive issuances of equity securities, the incurrence of debt, the creation of contingent liabilities, impairment expenses related to goodwill, and impairment or amortization expenses related to other intangible assets, which could harm our financial condition.

We will need to grow our organization, and we may experience difficulties in managing this growth, which could disrupt our operations.

At December 31, 2019, we had 115 full-time employees. As our development and commercialization plans and strategies develop, we expect to expand our employee base for managerial, operational, sales, marketing, financial and other resources. Future growth would impose significant added responsibilities on members of management, including the need to identify, recruit, maintain, motivate and integrate additional employees. Also, our management may need to divert a disproportionate amount of their attention away from our day-to-day activities and devote a substantial amount of time to managing these growth activities. We may not be able to effectively manage the expansion of our operations which may result in weaknesses in our infrastructure, give rise to operational errors, loss of business opportunities, loss of employees and reduced productivity among remaining employees. Our expected growth could require significant capital expenditures and may divert financial resources from other projects, such as the development of existing and additional product candidates. If our management is unable to effectively manage our expected growth, our expenses may increase more than expected, our ability to generate and/or grow revenue could be reduced and we may not be able to implement our business strategy. Our future financial performance and our ability to commercialize our product candidates and compete effectively with others in our industry will depend, in part, on our ability to effectively manage any future growth.

A disruption in our computer networks, including those related to cybersecurity, could adversely affect our financial performance.

We rely on sophisticated information technology systems to operate our business. These systems are potentially vulnerable to malicious intrusion, random attack, loss of data privacy, or breakdown. Although we have invested in the protection of our data and information technology and also monitor our systems on an ongoing basis, there can be no assurance that these efforts will prevent breakdowns or breaches in our information technology systems that could adversely affect our business.

We rely on our computer networks and systems, some of which are managed by third parties, to manage and store electronic information (including sensitive data such as confidential business information and personally identifiable data relating to employees, customers and other business partners), and to manage or support a variety of critical business processes and activities. We may face threats to our networks from unauthorized access, security breaches and other system disruptions. Despite our security measures, our infrastructure may be vulnerable to external or internal attacks. Any such security breach may compromise information stored on our networks and may result in significant data losses or theft of sensitive or proprietary information. In addition, a cybersecurity attack could result in other negative consequences, including disruption of our internal operations, increased cybersecurity protection costs, lost revenue, regulatory actions or litigation. Any disruption could also have a material adverse impact on our operations. We have not experienced any known attacks on our information technology systems that have resulted in any material system failure, accident or security breach to date.

We may be subject to security breaches or other cybersecurity incidents that could compromise our information and expose us to liability.

We routinely collect and store sensitive data (such as intellectual property, proprietary business information and personally identifiable information) for the Company, its employees and its suppliers and customers. We make significant efforts to maintain the security and integrity of our computer systems and networks and to protect this information. We have not experienced any known attacks on our information technology systems that have resulted in any material system failure, accident or security breach to date. However, like other companies in our industry, our networks and infrastructure may be vulnerable to cyber-attacks or intrusions, including by computer hackers, foreign governments, foreign companies or competitors, or may be breached by employee error, malfeasance or other disruption. Any such breach could result in unauthorized access to (or disclosure of) sensitive, proprietary or confidential information of ours, our employees or our suppliers or customers, and/or loss or damage to our data. If personal information or protected health information is improperly accessed, tampered with or disclosed as a result of a security breach, we may incur significant costs to notify and mitigate potential harm to the affected individuals, and we may be subject to sanctions and civil or criminal penalties if we are found to be in violation of the privacy or security rules under HIPAA or other similar federal or state laws protecting confidential personal information. Any such unauthorized access, disclosure, or loss of information could cause competitive harms, result in legal claims or proceedings, liability under laws that protect the privacy of personal information, and/or cause reputational harm.

Risks Related to Our Common Stock

We expect that our stock price will fluctuate significantly.

The trading prices of the securities of pharmaceutical and biotechnology companies have been highly volatile. The trading price of our common stock also may be highly volatile and could be subject to wide fluctuations in response to various factors, some of which are beyond our control. In addition to the factors discussed in this “Risk Factors” section, these factors include:

- adverse results or delays in the planned clinical trials of our product candidates or any future clinical trials we may conduct, or changes in the development status of our product candidates;
- any delay in our regulatory filings for our product candidates and any adverse development or perceived adverse development with respect to the applicable regulatory authority’s review of such filings, including without limitation the FDA’s issuance of a “refusal to file” letter or a request for additional information;
- regulatory or legal developments in the United States and other countries, especially changes in laws or regulations applicable to our products and product candidates, including clinical trial requirements for approvals;
- our inability to obtain or delays in obtaining adequate product supply for any approved product or inability to do so at acceptable prices;
- failure to commercialize our product candidates or if the size and growth of the markets we intend to target fail to meet expectations;
- additions or departures of key scientific or management personnel;
- unanticipated serious safety concerns related to the use of our product candidates;
- introductions or announcements of new products offered by us or significant acquisitions, strategic collaborations, joint ventures or capital commitments by us, our collaborators or our competitors and the timing of such introductions or announcements;
- our ability or inability to effectively manage our growth;
- changes in the structure of healthcare payment systems;
- our failure to meet the estimates and projections of the investment community or that we may otherwise provide to the public;
- publication of research reports about us or our industry, or positive or negative recommendations or withdrawal of research coverage by securities analysts;
- market conditions in the pharmaceutical and biotechnology sectors or the economy generally;
- our ability or inability to raise additional capital through the issuance of equity or debt or collaboration arrangements and the terms on which we raise it;
- trading volume of our common stock;
- disputes or other developments relating to proprietary rights, including patents, litigation matters and our ability to obtain patent protection for our technologies; and/or
- significant lawsuits, including patent or stockholder litigation.

The stock market in general, and market prices for the securities of biopharmaceutical companies like ours in particular, have from time to time experienced volatility that often has been unrelated to the operating performance of the underlying companies. These broad market and industry fluctuations may adversely affect the market price of our common stock, regardless of our operating performance. Stock prices of many pharmaceutical companies have fluctuated in a manner unrelated or disproportionate to the operating performance of those companies. In several recent situations when the market price of a stock has been volatile, holders of that stock have instituted securities class action litigation against the company that issued the stock. If any of our stockholders were to bring a lawsuit against us, the defense and disposition of the lawsuit could be costly and divert the time and attention of our management and harm our operating results.

If securities or industry analysts do not publish research reports about our business, or if they issue an adverse opinion about our business, our stock price and trading volume could decline.

The trading market for our common stock may be influenced by the research and reports that industry or securities analysts publish about us or our business. We do not currently have, and may never obtain research coverage by securities and industry analysts. If no or few analysts commence research coverage of us, or one or more of the analysts who cover us issues an adverse opinion about our company, our stock price would likely decline. If one or more of these analysts ceases research coverage of us or fails to regularly publish reports on us, we could lose visibility in the financial markets, which in turn could cause our stock price or trading volume to decline.

Future sales of our common stock or securities convertible into our common stock in the public market could cause our stock price to fall.

Our stock price could decline as a result of sales of a large number of shares of our common stock or securities convertible into our common stock or the perception that these sales could occur. These sales, or the possibility that these sales may occur, also might make it more difficult for us to sell equity securities in the future at a time and at a price that we deem appropriate.

Certain holders of our shares have rights requiring us to file registration statements covering the sale of their shares or to include their shares in registration statements that we may file for ourselves or other stockholders, subject to certain conditions. Shares issued upon the exercise of stock options outstanding under our equity incentive plans or pursuant to future awards granted under those plans will become available for sale in the public market to the extent permitted by the provisions of applicable vesting schedules, any applicable market stand-off and lock-up agreements, Rule 144 and Rule 701 under the Securities Act, as well as, to the extent applicable, under the registration statement on Form S-8 that we have filed.

Once we register the offer and sale of shares for the holders of registration rights and shares to be issued under our equity incentive plans, they can be freely sold in the public market upon issuance or resale (as applicable).

In addition, in the future, we may issue additional shares of common stock or other equity or debt securities convertible into common stock in connection with a financing, acquisition, litigation settlement, employee arrangements or otherwise. Any such issuance could result in substantial dilution to our existing stockholders and could cause our stock price to decline.

If we fail to maintain an effective system of internal control over financial reporting, we may not be able to accurately report our financial results or prevent fraud. As a result, stockholders could lose confidence in our financial and other public reporting, which would harm our business and the trading price of our common stock.

Effective internal controls over financial reporting are necessary for us to provide reliable financial reports and, together with adequate disclosure controls and procedures, are designed to prevent fraud. Any failure to implement required new or improved controls, or difficulties encountered in their implementation could cause us to fail to meet our reporting obligations. Inferior internal controls could also cause investors to lose confidence in our reported financial information, which could have a negative effect on the trading price of our common stock. In addition, any future testing by us conducted in connection with Section 404 of the Sarbanes-Oxley Act, or the subsequent testing by our independent registered public accounting firm, may reveal deficiencies in our internal controls over financial reporting that are deemed to be material weaknesses or that may require prospective or retroactive changes to our financial statements or identify other areas for further attention or improvement.

We are required, pursuant to Section 404 of the Sarbanes-Oxley Act, to furnish a report by management on, among other things, the effectiveness of our internal control over financial reporting. However, for as long as we are an “emerging growth company” under the JOBS Act, our independent registered public accounting firm will not be required to attest to the effectiveness of our internal controls over financial reporting pursuant to Section 404. We could be an emerging growth company for up to five years following the date of our IPO. An independent assessment of the effectiveness of our internal controls could detect problems that our management’s assessment might not. Undetected material weaknesses in our internal controls could lead to financial statement restatements and require us to incur the expense of remediation.

The holders of the convertible preferred stock will be entitled to be paid a liquidation preference, which under some circumstances will include a substantial premium.

In the event of a liquidation (as defined in the certificate of designations governing our convertible preferred stock), certain bankruptcy events or a failure to make any payment due on our or our subsidiaries' indebtedness after giving effect to any applicable cure period, the holders of the convertible preferred stock will be entitled to payment of a liquidation preference. The liquidation preference for each share of convertible preferred stock will equal the greater of (i) (A) (I) the original purchase price per share of convertible preferred stock plus dividend arrearages thereon in cash *plus* (II) any dividends accrued and unpaid thereon from the last dividend payment date to the date of the final distribution to such holder *plus* (B) in the majority of the events identified in the previous sentence, a premium equal to 20.2% of the amount described in clause (i)(A) of this sentence at such time or (ii) an amount per share of convertible preferred stock equal to the amount which would have been payable or distributable if each share of convertible preferred stock been converted into shares of our common stock immediately before the liquidation event.

Until the holders of the convertible preferred stock have been paid their liquidation preference in full, no payment will be made to any holder of common stock. If our assets, or the proceeds from their sale, distributable among the holders of the convertible preferred stock are not sufficient to pay the liquidation preference in full and the liquidating payments on any parity securities, then those assets or proceeds will be distributed among the holders of the convertible preferred stock and those parity securities on a pro rata basis. In that case, there would be no assets or proceeds remaining to be distributed to holders of our common stock, which would have a material adverse effect on the trading price of our common stock.

The holders of the convertible preferred stock are entitled to have their shares of convertible preferred stock redeemed at a substantial premium in certain events

Our convertible preferred stock is redeemable if we or our significant subsidiaries are the subject of certain bankruptcy events and upon the failure to make payments of amounts due on our or any of our subsidiaries' indebtedness after giving effect to any applicable cure period. Upon the occurrence of any of these events, the holders of our convertible preferred stock shall, in their sole discretion, be entitled to receive an amount equal to the original purchase price per share of convertible preferred stock plus dividend arrearages thereon *plus* any dividends accrued and unpaid thereon from the last dividend payment date to, but excluding, the date of such redemption *plus* the premium described under "The holders of the convertible preferred stock will be entitled to be paid a liquidation preference, which under some circumstances will include a substantial premium." If we were to become obligated to redeem all or a substantial portion of the outstanding convertible preferred stock, that could have a material adverse effect on the trading price of our common stock.

Shares of our convertible preferred stock are convertible into shares of our common stock and, upon conversion, will dilute your percentage of ownership.

Concurrently with the closing of our IPO, we issued 30,000 shares of our convertible preferred stock pursuant to an exchange agreement with holders of our Senior Convertible Term Loan. Holders of the convertible preferred stock shall be entitled to receive a cumulative dividend at an annual rate of 5% of the sum of the original purchase price per share of convertible preferred stock plus any dividend arrearages. In addition, holders of the convertible preferred stock shall be entitled to receive dividends paid or payable on our common stock with respect to the number of shares of our common stock into which each share of convertible preferred stock is then convertible at the then applicable conversion price. Shares of our convertible preferred stock are convertible at any time at the option of the holder into shares of our common stock at a conversion price equal to their original purchase price plus any accrued but unpaid dividends. In May 2019, a holder of 1,292 shares of our 5% convertible preferred stock exercised its right to convert such shares into 154,645 shares of our common stock. At December 31, 2019, 3,536,125 shares of our common stock are issuable upon conversion of our convertible preferred stock. This issuance of common stock upon the conversion would dilute the percentage ownership of holders of our common stock by approximately 2.2% as of December 31, 2019. The dilutive effect of the conversion of these securities may adversely affect our ability to obtain additional equity financing on favorable terms or at all.

Holders of the convertible preferred stock may exert substantial influence over us and may exercise their control in a manner adverse to your interests.

So long as shares of our convertible preferred stock remain outstanding, without the consent of at least a majority of the then outstanding shares of the convertible preferred stock, we may not (i) authorize or approve the issuance of any convertible preferred stock, senior securities or parity securities (or, in each case, any security convertible into, or convertible or exchangeable therefor or linked thereto) or authorize or create or increase the authorized amount of any convertible preferred stock, senior securities or parity securities (or, in each case, any security convertible into, or convertible or exchangeable therefor or linked thereto); (ii) authorize or approve the purchase or redemption of any parity securities or junior securities; (iii) amend, alter or repeal any of the provisions of the certificate of designations, our certificate of incorporation or our by-laws in a manner that would adversely affect the powers, designations, preferences and rights of the convertible preferred stock; (iv) contract, create, incur, assume or suffer to exist any indebtedness or guarantee any such indebtedness with an aggregate value of more than $5,000,000 (subject to certain exceptions); or (v) agree to take any of the above actions. The holders of convertible preferred stock will have one vote for each share of common stock into which such

holders' shares could then be converted at the time, and with respect to such vote, will have voting rights and powers equal to the voting rights and powers of the holders of our common stock.

The certificate of designations governing the convertible preferred stock also provides that no amendment or waiver of any provision of the certificate of designations or our charter or bylaws shall, without the prior written consent of all holders of the convertible preferred stock who are known to us to hold, together with their affiliates, more than 5% of the convertible preferred stock then outstanding (i) reduce any amounts payable or that may become payable to holders of the convertible preferred stock; (ii) postpone the payment date of any amount payable to holders of the convertible preferred stock or waive or excuse any payment; (iii) modify or waive the conversion rights of the convertible preferred stock in a manner that would adversely affect any holder of the convertible preferred stock; or (iv) change any of the voting-related provisions or any other provision of the certificate of designations specifying the number or percentage of holders of the convertible preferred stock which are required to waive, amend or modify any rights under the certificate of designations or make any determination or grant any consent under that document.

We will require additional capital in the future, which may not be available to us. Issuances of our equity securities to provide this capital may dilute your ownership in us.

We will need to raise additional funds through public or private debt or equity financings in order to:

- take advantage of expansion opportunities;
- acquire complementary products, product candidates or technologies;
- develop new products or technologies; and/or
- respond to competitive pressures.

Any additional capital raised through the issuance of our equity securities would dilute your percentage ownership interest in us. Furthermore, any additional financing we may need may not be available on terms favorable to us or at all. The unavailability of needed financing could adversely affect our ability to execute our business strategy.

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

Our executive officers, directors and holders of 5% or more of our capital stock, together with their respective affiliates, beneficially owned 50.1% of our capital stock as of March 2, 2020, of which 2.7% is beneficially owned by our executive officers. Accordingly, our executive officers, directors and principal stockholders are able to determine the composition of the board of directors, retain the voting power to approve all matters requiring stockholder approval, including mergers and other business combinations, and continue to have significant influence over our operations. This concentration of ownership could have the effect of delaying or preventing a change in our control or otherwise discouraging a potential acquirer from attempting to obtain control of us that you may believe are in your best interests as one of our stockholders. This in turn could have a material adverse effect on our stock price and may prevent attempts by our stockholders to replace or remove the board of directors or management.

Anti-takeover provisions in our charter documents and under Delaware law could make an acquisition of us difficult, limit attempts by our stockholders to replace or remove our current management and adversely affect our stock price.

Provisions of our certificate of incorporation and bylaws may delay or discourage transactions involving an actual or potential change in our control or change in our management, including transactions in which stockholders might otherwise receive a premium for their shares, or transactions that our stockholders might otherwise deem to be in their best interests. Therefore, these provisions could adversely affect the price of our stock. Among other things, our certificate of incorporation and bylaws:

- permit the board of directors to issue up to 10,000,000 shares of preferred stock, with any rights, preferences and privileges as they may designate;
- provide that the authorized number of directors may be changed only by resolution of the board of directors;
- provide that all vacancies, including newly-created directorships, may, except as otherwise required by law, be filled by the affirmative vote of a majority of directors then in office, even if less than a quorum; and
- require that any action to be taken by our stockholders must be effected at a duly called annual or special meeting of stockholders and may not be taken by written consent.

In addition, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, which generally prohibits a Delaware corporation from engaging in any of a broad range of business combinations with any stockholder owning in excess of 15.0% of our outstanding stock for a period of three years following the date on which the stockholder obtained such 15.0% equity interest in us.

We will continue to incur significant costs by being a public company.

As a public company, we incur significant legal, accounting and other expenses, including costs associated with public company reporting requirements. We also incur costs associated with corporate governance requirements, including requirements of the SEC and the New York Stock Exchange ("NYSE"). We expect these rules and regulations to continue to increase our legal and financial compliance costs and to make some activities more time-consuming and costly. We also expect that these rules and regulations may continue to make it more difficult and expensive for us to obtain director and officer liability insurance and we may be required to accept reduced policy limits and coverage or incur substantially higher costs to obtain the same or similar coverage. As a result, it may be more difficult for us to attract and retain qualified individuals to serve on our board of directors or as executive officers.

When we cease to be an "emerging growth company" and when our independent registered public accounting firm is required to undertake an assessment of our internal control over financial reporting, the cost of our compliance with Section 404 will correspondingly increase. Moreover, if we are not able to comply with the requirements of Section 404 applicable to us in a timely manner, or if we or our independent registered public accounting firm identifies deficiencies in our internal control over financial reporting that are deemed to be material weaknesses, the market price of our stock could decline and we could be subject to sanctions or investigations by the SEC or other regulatory authorities, which would require additional financial and management resources.

We are an "emerging growth company," as defined in the JOBS Act, and as a result of the reduced disclosure and governance requirements applicable to emerging growth companies, our common stock may be less attractive to investors.

We are an "emerging growth company," as defined in the JOBS Act, and we take advantage of certain exemptions from various reporting requirements that are applicable to other public companies that are not "emerging growth companies" including, but not limited to, not being required to comply with the auditor attestation requirements of Section 404, reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements, and exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved. We cannot predict if investors will find our common stock less attractive because 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. We will take advantage of these reporting exemptions until we are no longer an "emerging growth company." We will remain an "emerging growth company" until the earliest of (i) the last day of the fiscal year in which we have total annual gross revenues of $1.07 billion or more, (ii) the last day of our fiscal year following the fifth anniversary of the date of the completion of our IPO, which is December 31, 2021, (iii) the date on which we have issued more than $1.0 billion in nonconvertible debt during the previous three years or (iv) the date on which we are deemed to be a large accelerated filer under the rules of the SEC.

Our management has broad discretion in using cash and cash equivalents and our other capital resources.

We expect to continue to use our cash and cash equivalents and our other capital resources to fund the clinical development of our pipeline and for general corporate purposes. Our management has broad discretion in the application of our cash and cash equivalents and our other capital resources and could spend the funds in ways that do not improve our results of operations or enhance the value of our equity. The failure by our management to apply these funds effectively could result in financial losses that could have a material adverse effect on our business, diminish available cash flows available to service our debt, cause the value of our equity to decline and delay the development of our product candidates. Pending their use, we may invest cash and cash equivalents and our other capital resources in a manner that does not produce income or that loses value.

We could be classified as an inadvertent investment company.

We are not engaged in the business of investing, reinvesting or trading in securities, and we do not hold ourselves out as being engaged in those activities. However, due to various strategic collaborations and corporate transactions customary in our industry, we own, and may come to own, securities of third parties. As such, there can be no assurance that we will be able to avoid being inadvertently deemed an investment company under the Investment Company Act of 1940 (the "Investment Company Act"). If we were deemed to be an investment company, we would be subject to burdensome compliance requirements and restrictions that would limit our activities, including limitations on our capital structure, our ability to sell our securities and our ability to transact business, which would have a material adverse effect on our financial condition and results of operations. To avoid being deemed an investment company, we may be required to sell certain of our investments or to conduct our business in a manner that does not subject us to the requirements of the Investment Company Act, which could have an adverse effect on our business, financial condition and results of operations.

Because we do not anticipate paying any cash dividends on our common 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 common 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 existing or any future debt agreements may preclude us from paying dividends. As a result, capital appreciation, if any, of our equity securities will likely be your sole source of gain for the foreseeable future.

Future sales and issuances of equity securities, convertible securities or other securities could result in additional dilution of the percentage ownership of holders of our common stock.

We expect that additional capital will be needed in the future to continue our planned operations. To raise capital, we may sell equity securities, convertible securities or other securities in one or more transactions at prices and in a manner we determine from time to time. If we sell equity securities, convertible securities or other securities in more than one transaction, investors in such future offerings may be materially diluted by subsequent sales. Such sales would also likely result in material dilution to our existing equity holders, and new investors could gain rights, preferences and privileges senior to those of holders of our existing equity securities.

Item 1B. Unresolved Staff Comments.

None.

Item 2. Properties.

We have three primary operating locations which are occupied under long-term leasing arrangements. In October 2010, Kadmon Corporation, LLC entered into a corporate headquarters and laboratory lease in New York, New York, expiring in February 2021 and opened a secured letter of credit with a third party financial institution in lieu of a security deposit for $2.0 million. Since inception, there have been six amendments to this lease agreement, which have altered office and laboratory capacity and extended the lease term through October 2025. We have the ability to extend portions of the lease on the same terms and conditions as the current lease, except that the base rent will be adjusted to the fair market rental rate for the building based on the rental rate for comparable space in the building at the time of extension.

We are party to an operating lease in Warrendale, Pennsylvania (our specialty-focused commercial operation), which expires on September 30, 2022, with a two five-year renewal options, which would extend the term to September 30, 2032, if exercised. Rental payments under the renewal period will be at market rates determined from the average rentals of similar tenants in the same industrial park.

In August 2015, we entered into an office lease agreement in Cambridge, Massachusetts (our clinical office) effective January 2016 and expiring in April 2023. We opened a secured letter of credit with a third party financial institution in lieu of a security deposit for $91,000.

For additional information, see Contractual Obligations and Commitments in Part II, Item 7 of this Annual Report on Form 10-K.

Item 3. Legal Proceedings

We are not currently subject to any material legal proceedings. However, we may from time to time become a party to various legal proceedings arising in the ordinary course of our business.

Item 4. Mine Safety Disclosures.

Not applicable.

PART II

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

Market Information

Our common stock is listed on the NYSE under the symbol "KDMN".

Holders of Record

On March 2, 2020, there were approximately 212 stockholders of record of our common stock and the closing price of our common stock was $4.85 per share as reported by the NYSE. Since many of our shares of common stock are held by brokers and other institutions on behalf of stockholders, we are unable to estimate the total number of stockholders represented by these record holders.

Dividend Policy

We currently expect to retain all future earnings, if any, for use in the operation and expansion of our business and paying any debts. We have never declared nor paid any dividends on our common stock and do not anticipate paying cash dividends to holders of our common stock in the foreseeable future. See "Risk Factors—Because we do not anticipate paying any cash dividends on our common stock in the foreseeable future, capital appreciation, if any, will be your sole source of gain." Any determination to pay dividends on our common stock in the future will be at the discretion of our board of directors and will depend upon, among other factors, our results of operations, financial condition, capital requirements and covenants in our existing financing arrangements and any future financing arrangements. Holders of the convertible preferred stock are entitled to receive a cumulative dividend at an annual rate of 5% of the original purchase price per share of convertible preferred stock, when and as declared by our board of directors and to the extent of funds legally available for the payment of dividends. Holders of the convertible preferred stock are also entitled to participate in all dividends declared and paid to holders of our common stock on an "as if" converted basis.

Purchases of Equity Securities by the Issuer of Affiliated Purchasers

None.

Securities Authorized for issuance under our Equity Compensation Plans

The information called for by this item will be included in our definitive proxy statement with respect to our 2020 Annual Meeting of Shareholders to be filed with the SEC, and is incorporated herein by reference.

Item 6. Selected Financial Data.

As a "smaller reporting company", we are not required to provide the information required by this item.

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

In this Annual Report on Form 10-K, unless otherwise stated or the context otherwise requires, references to the "Company," "Kadmon," "we," "us" and "our" refer to Kadmon Holdings, Inc. and its consolidated subsidiaries. You should read the following discussion and analysis of our financial condition and results of operations together with our financial statements and related notes appearing in 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, including information with respect to our plans and strategy for our business and related financing, 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 that discovers, develops and delivers transformative therapies for unmet medical needs. Our team has a proven track record of successful drug development and commercialization. Our clinical pipeline includes treatments for immune and fibrotic diseases as well as immuno-oncology therapies. We expect to continue to progress our clinical candidates and have further clinical trial events to report in 2020.

KD025, our most advanced product candidate, is an orally administered, selective small molecule inhibitor of Rho-associated coiled-coil kinase 2 ("ROCK2"). A pivotal study of KD025 is ongoing in patients with chronic graft-versus-host disease ("cGVHD"), a complication that can occur following hematopoietic cell transplantation ("HCT") that results in multi-organ inflammation and fibrosis. The U.S. Food and Drug Administration ("FDA") has granted Breakthrough Therapy Designation to KD025 for the treatment of cGVHD after failure of two or more prior lines of systemic therapy. The FDA has also granted Orphan Drug Designation to KD025 for the treatment of cGVHD.

In November 2019, we announced positive topline results from the planned interim analysis of ROCKstar (KD025-213), our pivotal trial evaluating KD025 in patients with cGVHD who have received at least two prior lines of systemic therapy. The trial met the primary endpoint of Overall Response Rate ("ORR") at the interim analysis, which was conducted as scheduled two months after completion of enrollment. KD025 showed statistically significant ORRs of 64% with KD025 200 mg once daily (95% Confidence Interval ("CI"): 51%, 75%; $p<0.0001$) and 67% with KD025 200 mg twice daily (95% CI: 54%, 78%; $p<0.0001$). In February 2020, we announced expanded results of the interim analysis of KD025-213, showing that ORRs were consistent across key subgroups, including in patients with four or more organs affected by cGVHD (n=69; 64%) and patients who had no response to their last line of treatment (n=74; 68%). Responses were observed in all affected organ systems, including in organs with fibrotic disease. KD025 has been well tolerated and adverse events have been consistent with those expected in the patient population. Additional secondary endpoints, including duration of response, corticosteroid dose reductions, Failure-Free Survival, Overall Survival and Lee Symptom Scale reductions continue to mature and will be available later in 2020

Further, in December 2019, we presented two-year follow-up data from our ongoing Phase 2a clinical trial of KD025 in cGVHD (KD025-208). The data showed continued patient benefit, with an ORR of 65% across all three dose cohorts. Responses were observed in all affected organ systems, including organs with fibrotic disease. Kaplan-Meier median duration of response was 35 weeks. KD025 was well tolerated, with no increased risk of infection observed. Twenty-four percent of the patients in the trial had remained on KD025 therapy for more than one-and-a-half years as of June 30, 2019.

We also initiated a double-blind, placebo-controlled Phase 2 clinical trial of KD025 for the treatment of systemic sclerosis, a life-threatening autoimmune disease characterized by chronic inflammation, fibrosis and vascular damage, in 2019.

In October 2019, we entered into a transaction pursuant to which we sold approximately 1.4 million ordinary shares of MeiraGTx Holdings plc ("MeiraGTx") for gross proceeds of $22.0 million. After consummation of the transaction, we held approximately 5.7% of the outstanding ordinary shares of MeiraGTx with a fair value of $42.0 million recorded as a current investment in equity securities at December 31, 2019. The fair value of our investment in MeiraGTx is subject to market conditions and may be volatile and fluctuate substantially.

In November 2019, we raised $101.6 million ($95.0 million net of $6.6 million of underwriting discounts and other offering expenses payable by us) from the issuance of 29,900,000 shares of common stock at a price of $3.40 per share ("2019 Public Offering"). Additionally, in November 2019, the Company repaid in full all amounts outstanding under the 2015 Credit Agreement and the Company no longer maintains any outstanding debt.

Our operations to date have been focused on developing first-in-class innovative therapies for indications with significant unmet medical needs while leveraging our commercial infrastructure. We have never been profitable and had an accumulated deficit of $333.1 million at December 31, 2019. Our net losses were $61.4 million and $54.3 million for the years ended December 31, 2019 and 2018, respectively. Although our commercial business generates revenue, we expect to incur significant losses for the foreseeable future, and we expect these losses to increase as we continue our development of, and seek regulatory approvals for, our product candidates, hire additional personnel and initiate commercialization of any products that receive regulatory approval. We anticipate that our expenses will increase substantially if, or as, we:

- invest significantly to further develop our most advanced product candidates, including KD025;
- initiate clinical trials of KD033 and KD045 and preclinical studies for our other product candidates;
- seek regulatory approval for our product candidates that successfully complete clinical trials;
- continue to invest in our research platforms;
- seek to identify additional product candidates;
- scale up our sales, marketing and distribution infrastructure and product sourcing capabilities;
- acquire or in-license other product candidates and technologies;
- scale up our operational, financial and management information systems and personnel, including personnel to support our product development;
- make milestone or other payments under any in-license agreements; or
- maintain, expand and protect our intellectual property portfolio

Components of Statement of Operations

Revenue

Our revenue is derived from sales of our portfolio of products, including CLOVIQUE and products obtained from Camber Pharmaceuticals, Inc. (“Camber”) pursuant to our terminated supply and distribution agreement with Camber, licensing revenue related to our strategic partnerships, and service revenue from our sublease agreements with MeiraGTx. No meaningful revenue has been generated from sales of our other products. Revenue in 2019 primarily includes the recognition of licensing fees related to our strategic partnership with BioNova Pharmaceuticals Ltd. (“BioNova”), pursuant to which we and BioNova formed a joint venture to develop KD025 in China.

Cost of Sales

Cost of sales consists of product costs, including ingredient costs and costs of contract manufacturers for production, and shipping and handling of the products. Also included are costs related to quality release testing and stability testing of the products. Other costs included in cost of sales are packaging costs, warehousing costs and certain allocated costs related to management, facilities and other expenses associated with supply chain logistics.

Research and development expenses

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

- license fees related to our license and collaboration agreements;
- research and development-based employee-related expenses, including salaries, benefits, travel and other compensation expenses;
- expenses incurred under our agreements with contract research organizations that conduct nonclinical and preclinical studies, and clinical sites and consultants that conduct our clinical trials;
- costs associated with regulatory filings;
- costs of laboratory supplies and the acquisition, development and manufacture of preclinical and clinical study materials and study drugs; and
- allocated facility-related expenses.

Our research and development expenses may vary substantially from period to period based on the timing of our research and development activities, including timing of initiation of clinical trials and enrollment of patients in clinical trials.

We do not allocate personnel-related costs, including share-based compensation, costs associated with broad technology platform improvements and other indirect costs to specific product candidates. We do not allocate these costs to specific product candidates because they are deployed across multiple overlapping projects under development, making it difficult to specifically and accurately allocate such costs to a particular product candidate.

The successful development of our product candidates is highly uncertain and subject to numerous risks including, but not limited to:

- the scope, rate of progress and expense of our research and development activities;
- clinical trial results;
- the scope, terms and timing of regulatory approvals;
- the expense of filing, prosecuting, defending and enforcing patent claims and other intellectual property rights;
- the cost, timing and our ability to acquire sufficient clinical and commercial supplies for any product candidates and products that we may develop; and
- the risks disclosed in the section entitled “Risk Factors” in this Annual Report on Form 10-K.

A change in the outcome of any of these variables could mean a significant change in the expenses and timing associated with the development of any product candidate.

Selling, general and administrative expenses

Selling, general and administrative expenses consist primarily of salaries and related costs for non-research personnel, including stock-based compensation and travel expenses for our employees in executive, operational, finance, legal, commercial, regulatory, pharmacovigilance and human resource functions. Other selling, general and administrative expenses include facility-related costs, lease expense, business insurance, director compensation, accounting and legal services, consulting costs and programs and marketing costs to support the commercial business.

Other income (expense)

Other income (expense) is comprised of interest income earned on cash and cash equivalents and restricted cash, interest expense on our outstanding indebtedness, and amortization of debt discount, debt premium and deferred financing costs associated with our indebtedness through November 2019 when the indebtedness was repaid in full.

Other income (expense) includes realized and unrealized gains on equity securities. The realized gains represent the total gains on our investment in MeiraGTx sold since MeiraGTx’s IPO in June 2018. The unrealized gains on equity securities consists of two components: (i) the reversal of the gain or loss recognized in previous periods on equity securities sold and (ii) the change in unrealized gain or loss resulting from mark-to-market adjustments on equity securities still held.

Other income also includes gains and losses arising from changes in fair value of our financial instruments are recognized in other income in the consolidated statements of operations. Such financial instruments include warrant liabilities which are exercisable and redeemable at the option of the holder upon the occurrence of, and during the continuance of, an event of default under the warrant agreement. The change in fair value is based upon the fair value of the underlying security at the end of each reporting period, as calculated using the Black-Scholes option pricing model.

In addition, we operate in currencies other than the U.S. dollar to fund research and development and commercial activities performed by various third-party vendors. The translation of these currencies into U.S. dollars results in foreign currency gains or losses, depending on the change in value of these currencies against the U.S. dollar, which are included in other income.

Income taxes

At December 31, 2019 and 2018, we had a deferred tax liability of $0.5 million and $0.4 million, respectively, and a full valuation allowance for our deferred tax assets. As of December 31, 2019, we have unused federal and state net operating loss (“NOL”) carryforwards of $371.1 million and $307.2 million, respectively, that may be applied against and reduce future taxable income. We recorded a valuation allowance to fully offset the gross deferred tax asset, because it is more likely than not that we will not realize future benefits associated with these deferred tax assets at December 31, 2019 and 2018. These NOL carryforwards expire at various dates through December 31, 2037, with the exception of approximately $79.9 million of federal net operating loss carryforwards which will not expire.

The use of our NOL carryforwards may, however, be subject to limitations as a result of an ownership change. A corporation undergoes an “ownership change,” in general, if a greater than 50% change (by value) in its equity ownership by one or more five-percent stockholders (or certain groups of non-five-percent stockholders) over a three-year period occurs. After such an ownership change, the corporation’s use of its pre-change NOL carryforwards and other pre-change tax attributes to offset its post-change income is subject to an annual limitation determined by the equity value of the corporation

on the date the ownership change occurs multiplied by a rate determined monthly by the Internal Revenue Service. We experienced ownership changes under Section 382 of the Code in 2010, 2011 and 2016, but we did not reduce the gross deferred tax assets related to the NOL carryforwards because the limitations do not hinder our ability to potentially utilize all of the NOL carryforwards. We also experienced another ownership change in 2019, as a result of equity offerings. This ownership change reduced our ability to fully utilize all of our NOL carryforwards and, consequently, we reduced the gross deferred tax assets related to our NOL carryforwards by $125.2 million.

Critical Accounting Policies and Significant Judgments and Estimates

Management's discussion and analysis of our financial condition and results of operations is based on our financial statements, which have been prepared in accordance with accounting principles generally accepted in the United States of America ("GAAP"). The preparation of these consolidated financial statements requires us to make estimates and judgments that affect the reporting amounts of assets, liabilities and expenses and the disclosure of contingent assets and liabilities in our financial statements. On an ongoing basis, we evaluate our estimates and judgments, including those related to investments, goodwill, fair value of financial instruments, share-based compensation and accrued expenses. We base our estimates on historical experience, known trends and events and various other factors that we believe to be reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions. If actual results for the critical accounting estimates listed below varied from our estimates, it could significantly impact our financial results. We believe that full consideration has been given to all relevant circumstances that we may be subject to, and the consolidated financial statements accurately reflect our best estimate of the results of operations, financial position and cash flows for the periods presented. While our significant accounting policies are more fully described in Note 2, "Summary of Significant Accounting Policies" of the notes to our consolidated financial statements in Part IV, Item 15 of this Annual Report on Form 10-K, we believe the following accounting policies to be most critical to the judgements and estimates used in the preparation of our financial statements.

Revenue Recognition

We recognize revenue in accordance with the Financial Accounting Standards Board's, or FASB, Accounting Standards Codification, or ASC, Topic 606, *Revenue from Contracts with Customers*. Accordingly, revenue is recognized when the customer obtains control of promised goods or services, in an amount that reflects the consideration which we expect to receive in exchange for those goods or services. To determine revenue recognition for arrangements that we determine are within the scope of ASC Topic 606, we perform the following five steps:

i. identify the contract(s) with a customer;
ii. identify the performance obligations in the contract;
iii. determine the transaction price;
iv. allocate the transaction price to the performance obligations; and
v. recognize revenue when (or as) performance obligations are satisfied

We only apply the five-step model to contracts when we determine that it is probable we will collect the consideration to which we are entitled in exchange for the goods or services we transfer to the customer. At contract inception, once the contract is determined to be within the scope of ASC Topic 606, we assess the goods or services promised within each contract and determine those that are performance obligations, and assess whether each promised good or service is distinct. We then recognize as revenue the amount of the transaction price that is allocated to the respective performance obligation when (or as) the performance obligation is satisfied.

Any amounts received prior to satisfying the revenue recognition criteria are recognized as deferred revenue in our balance sheet. Any amounts which are deferred and are expected to be recognized as revenue within the 12 months following the balance sheet date are classified as current portion of deferred revenue. Amounts not expected to be recognized as revenue within the 12 months following the balance sheet date are classified as deferred revenue, net of current portion.

Our revenues have primarily been generated through product sales, collaborative research, development and commercialization license agreements and other service agreements. The terms of these license agreements typically may include payment to us of one or more of the following: nonrefundable, up-front license fees, research, development and commercial milestone payments; and other contingent payments due based on the activities of the counterparty or the reimbursement by licensees of costs associated with patent maintenance. Each of these types of revenue are recorded as license revenues in our statement of operations.

In determining the appropriate amount of revenue to be recognized as we fulfill our obligations under each arrangement, we perform the following steps:

i. identify the promised goods and services in the contract;
ii. determine whether the promised goods or services are performance obligations, including whether they are distinct within the context of the contract;
iii. measure the transaction price, including the constraint on variable consideration;
iv. allocate the transaction price to the performance obligations; and
v. recognize revenue when (or as) performance obligations are satisfied

See Note 11, “License Agreements” for additional details regarding our license arrangements.

As part of the accounting for these arrangements, we allocate the transaction price to each performance obligation on a relative stand-alone selling price basis. The stand-alone selling price may be, but is not presumed to be, the contract price. In determining the allocation, we maximize the use of observable inputs. When the stand-alone selling price of a good or service is not directly observable, we estimate the stand-alone selling price for each performance obligation using assumptions that require judgment. Acceptable estimation methods include, but are not limited to: (i) the adjusted market assessment approach, (ii) the expected cost plus margin approach, and (iii) the residual approach (when the stand-alone selling price is not directly observable and is either highly variable or uncertain). In order for the residual approach to be used, we must demonstrate that (a) there are observable stand-alone selling prices for one or more of the performance obligations and (b) one of the two criteria in ASC 606-10- 32-34(c)(1) and (2) is met. The residual approach cannot be used if it would result in a stand-alone selling price of zero for a performance obligation as a performance obligation, by definition, has value on a stand-alone basis.

An option in a contract to acquire additional goods or services gives rise to a performance obligation only if the option provides a material right to the customer that it would not receive without entering into that contract. Factors that we consider in evaluating whether an option represents a material right include, but are not limited to: (i) the overall objective of the arrangement, (ii) the benefit the collaborator might obtain from the arrangement without exercising the option, (iii) the cost to exercise the option (e.g. priced at a significant and incremental discount) and (iv) the likelihood that the option will be exercised. With respect to options determined to be performance obligations, we recognize revenue when those future goods or services are transferred or when the options expire.

Our revenue arrangements may include the following:

Up-front License Fees: If a license is determined to be distinct from the other performance obligations identified in the arrangement, we recognize revenues from nonrefundable, up-front fees allocated to the license when the license is transferred to the licensee and the licensee is able to use and benefit from the license. For licenses that are bundled with other promises, we utilize judgment to assess the nature of the combined performance obligation to determine whether the combined performance obligation is satisfied over time or at a point in time and, if over time, the appropriate method of measuring progress for purposes of recognizing revenue from non-refundable, up-front fees. We evaluate the measure of progress each reporting period and, if necessary, adjusts the measure of performance and related revenue recognition.

Milestone Payments: At the inception of an agreement that includes research and development milestone payments, we evaluate whether each milestone is considered probable of being achieved and estimate the amount to be included in the transaction price using the most likely amount method. If it is probable that a significant revenue reversal would not occur, the associated milestone value is included in the transaction price. Milestone payments that are not within our control or the licensee, such as regulatory approvals, are not considered probable of being achieved until those approvals are received. The transaction price is then allocated to each performance obligation on a relative stand-alone selling price basis, for which we recognize revenue as or when the performance obligations under the contract are satisfied. At the end of each subsequent reporting period, we re-evaluate the probability of achievement of such milestones and any related constraint, and if necessary, adjust our estimate of the overall transaction price. Any such adjustments are recorded on a cumulative catch-up basis, which would affect license revenues and earnings in the period of adjustment.

Research and Development Activities: If we are entitled to reimbursement from our collaborators for specified research and development activities or the reimbursement of costs associated with patent maintenance, we determine whether such funding would result in license revenues or an offset to research and development expenses.

Royalties: If we are entitled to receive sales-based royalties from our collaborator, including milestone payments based on the level of sales, and the license is deemed to be the predominant item to which the royalties relate, we recognize revenue at the later of (i) when the related sales occur, provided the reported sales are reliably measurable, or (ii) when the performance obligation to which some or all of the royalty has been allocated has been satisfied (or partially satisfied). To date, we have not recognized any royalty revenue resulting from any of our collaboration and license arrangements.

Manufacturing Supply and Research Services: Arrangements that include a promise for future supply of drug substance, drug product or research services at the licensee's discretion are generally considered as options. We assess if these options provide a material right to the licensee and if so, they are accounted for as separate performance obligations. If we are entitled to additional payments when the licensee exercises these options, any additional payments are recorded in license revenues when the licensee obtains control of the goods, which is upon delivery, or as the services are performed.

We receive payments from our licensees based on schedules established in each contract. Upfront payments and fees are recorded as deferred revenue upon receipt, and may require deferral of revenue recognition to a future period until we perform our obligations under these arrangements. Amounts are recorded as accounts receivable when our right to consideration is unconditional. We do not assess whether a contract has a significant financing component if the expectation at contract inception is such that the period between payment by the licensees and the transfer of the promised goods or services to the licensees will be one year or less.

Share-based compensation expense

We are required to estimate the grant-date fair value of stock options and stock appreciation rights issued to employees and recognize this cost over the period these awards vest. We estimate the fair value of each stock option granted using the Black-Scholes option pricing model. The Black-Scholes model requires the input of assumptions, including the expected stock price volatility, the calculation of expected term and the fair value of the underlying common stock on the date of grant, among other inputs. Generally, we have issued stock options that vest over time. For these awards, we record compensation cost on a straight-line basis over the vesting period. We calculate the fair value of the award on the grant date, which is the date the award is authorized by the board of directors and the employee has an understanding of the terms of the award.

The assumptions relating to the valuation of our options granted for the years ended December 31, 2019 and 2018 are shown below. The table does not include Performance Options.

	Years Ended	
	December 31, 2019	December 31, 2018
Weighted average fair value of grants	$2.07	$1.69
Expected volatility	75.96% - 77.73%	72.94% - 75.92%
Risk-free interest rate	1.41% - 2.61%	2.44% - 2.90%
Expected life	5.5 - 6.0 years	5.5 - 6.0 years
Expected dividend yield	0%	0%

The following table summarizes by grant date the number of shares subject to options granted since January 1, 2018, the per share exercise price of the options, the fair value of common stock underlying the options on date of grant and the per share estimated fair value of the options:

Grant Date	Number of Shares Subject to Options Granted	Per Share Exercise Price of Options	Fair Value of Common Stock per Share on Date of Option Grant	Per Share Estimated Fair Value of Options
February 19, 2018	2,000	$ 3.63	$ 3.63	$ 2.42
March 16, 2018	3,077	$ 4.48	$ 4.48	$ 2.05
April 3, 2018	1,597,500	$ 4.06	$ 4.06	$ 2.71
April 10, 2018	40,000	$ 4.22	$ 4.22	$ 2.83
May 9, 2018	2,000	$ 3.96	$ 3.96	$ 2.66
June 25, 2018	33,847	$ 3.88	$ 3.88	$ 1.67
July 27, 2018	200,000	$ 3.35	$ 3.35	$ 2.16
December 14, 2018	1,829,500	$ 2.47	$ 2.47	$ 1.62
January 22, 2019	64,935	$ 2.27	$ 2.27	$ 1.54
February 6, 2019	64,935	$ 2.27	$ 2.27	$ 1.54
February 8, 2019	400,000	$ 2.17	$ 2.17	$ 1.48
May 15, 2019	664,103	$ 2.29	$ 2.29	$ 1.51
May 22, 2019	100,000	$ 2.25	$ 2.25	$ 1.48
August 30, 2019	300,000	$ 2.14	$ 2.14	$ 1.42
November 19, 2019	1,350,000	$ 4.15	$ 4.15	$ 2.76

Expenses Accrued Under Contractual Arrangements with Third Parties; Accrued Clinical Expenses

As part of the process of preparing our financial statements, we are required to estimate our accrued 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 cost. The majority of our service providers invoice us monthly in arrears for services performed. We make estimates of our accrued expenses as of each balance sheet date in our financial statements based on facts and circumstances known to us at that time. We periodically confirm the accuracy of our estimates with the service providers and make adjustments if necessary.

We base our expenses related to clinical trials on our estimates of the services received and efforts expended pursuant to contracts with multiple research institutions and contract research organizations that conduct and manage 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. Payments under some of these contracts depend on factors such as the successful enrollment of patients and the completion of clinical trial milestones. In accruing expenses, we estimate the time period over which services will be performed and the level of effort to be expended in each period, which is based on an established protocol specific to each clinical trial. If the actual timing of the performance of services or the level of effort varies from our estimate, we adjust the accrual 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 us reporting amounts that are too high or too low in any particular period.

To date, we have not experienced significant changes in our estimates of accrued research and development expenses following each applicable reporting period. However, due to the nature of estimates, we cannot assure you that we will not make changes to our estimates in the future as we become aware of additional information regarding the status or conduct of our clinical studies and other research activities.

Investment in Equity Securities

Equity securities consist of investments in common stock of companies traded on public markets (see Note 10 of the notes to our audited consolidated financial statements in Part IV, Item 15 of this Annual Report on Form 10-K). These shares are carried on our balance sheet at fair value based on the closing price of the shares owned on the last trading day before the balance sheet of this report. Fluctuations in the underlying bid price of the shares result in unrealized gains or losses. In accordance with FASB ASC 321, Investments – Equity Securities (“ASC 321”), we recognize these fluctuations in value as other expense (income). For investments sold, we recognize the gains and losses attributable to these investments as realized gains or losses in other expense (income).

Our total investment balance in equity securities totaled $42.0 million at December 31, 2019, all of which related to our investment in MeiraGTx’s common stock.

Recent Accounting Pronouncements

See Note 2 “Summary of Significant Accounting Policies,” of the notes to our audited consolidated financial statements included elsewhere in this Annual Report on Form 10-K for a summary of recently issued and adopted accounting pronouncements.

Results of Operations

	Years Ended December 31,	
	2019	2018
	(in thousands)	
Revenues		
Net sales	$ 420	$ 691
License and other revenue	4,675	705
Total revenue	5,095	1,396
Cost of sales	377	412
Write-down of inventory	912	270
Gross profit	3,806	714
Operating expenses:		
Research and development	56,461	48,966
Selling, general and administrative	36,425	37,644
Total operating expenses	92,886	86,610
Loss from operations	(89,080)	(85,896)
Other income	27,758	31,120
Income tax expense (benefit)	46	(524)
Net loss	$ (61,368)	$ (54,252)
Deemed dividend on convertible preferred stock	2,058	2,011
Net loss attributable to common stockholders	$ (63,426)	$ (56,263)

Comparison of the years ended December 31, 2019 and 2018

Revenues

Total revenue for 2019 increased by 264.3% as compared to 2018 primarily due to a $4.0 million milestone payment earned pursuant to a joint venture and license agreement entered into with BioNova and BK Pharmaceuticals Limited to develop KD025 in China (see Note 11 of the notes to our audited consolidated financial statements in Part IV, Item 15 of this Annual Report on Form 10-K).

Cost of sales and write-down of inventory

The decrease in cost of sales was primarily attributable to the decline in net sales revenue for the year ended December 31, 2019 as compared to the year ended December 31, 2018.

The increase in inventory write-downs during the year ended December 31, 2019 as compared to the year ended December 31, 2018 was related to write-downs of our CLOVIQUE inventory based on our expectation that such inventory will not be sold prior to reaching its product expiration date.

Research and development expenses

Research and development expenses increased by 15.3% for the year ended December 31, 2019 as compared the year ended December 31, 2018. The increase of approximately $7.5 million in research and development expense for 2019 was primarily related to direct external costs of developing our lead product candidate, KD025. For the years ended December 31, 2019 and 2018, we recognized expense of $18.3 million and $10.1 million, respectively, for KD025. There was no significant change in our expense related to developing our other product candidates across multiple projects in 2019 as compared to 2018.

Other income

The following table provides components of other income (expense):

	Years Ended December 31,	
	2019	2018
	(in thousands)	
Interest expense	$ (2,816)	$ (3,565)
Amortization of deferred financing costs, debt discount and debt premium	(565)	(1,054)
Change in fair value of financial instruments	(961)	1,525
Unrealized gain on equity securities	7,922	34,075
Realized gain on equity securities	22,000	—
Loss on equity method investment	—	(1,242)
Interest income	2,067	1,307
Other income	111	74
Other income	**$ 27,758**	**$ 31,120**

For the years ended December 31, 2019 and 2018, other income consisted primarily of unrealized and realized gains related to our investment in MeiraGTx ordinary shares as well as interest income. Interest expense and other costs related to our debt decreased in 2019 as the Company repaid its debt in full in November 2019. See Note 10 “Investment in MeiraGTx,” of the notes to our audited consolidated financial statements included elsewhere in this Annual Report on Form 10-K for details related to the Company’s investment in equity securities.

Income taxes

For the year ended December 31, 2019, we recorded an income tax expense of less than $0.1 million related to an adjustment to the deferred tax liability.

The Tax Cuts and Jobs Act (the “TCJA”) was enacted in December 2017. In accordance with the TCJA, the Company determined it necessary to reduce the recorded deferred tax liability by $0.6 million during the second quarter of 2018 to allow naked credit deferred tax liabilities to be used as a source of taxable income in the future. The change in deferred tax liability has been recognized as income tax benefit in the consolidated financial statements of operations for the year ended December 31, 2018.

Deemed Dividend

We have 28,708 shares of 5% convertible preferred stock outstanding, which accrue dividends at a rate of 5% and convert into shares of our common stock at $9.60 per share. In May 2019, the holders of 1,292 shares of 5% convertible preferred stock exercised their right to convert their convertible preferred shares into 154,645 shares of the Company’s common stock. We accrued dividends, inclusive of a beneficial conversion feature on the accrued dividends, on the 5% convertible preferred stock during each of the years ended December 31, 2019 and 2018.

Liquidity and Capital Resources

Overview

We had an accumulated deficit of $333.1 million, working capital of $155.9 million, and cash and cash equivalents of $139.6 million at December 31, 2019. Net cash used in operating activities was $80.1 million and $71.2 million for the years ended December 31, 2019 and 2018, respectively.

In November 2019, we raised $101.6 million ($95.0 million net of $6.6 million of underwriting discounts and other offering expenses payable by us) from the issuance of 29,900,000 shares of common stock at a price of $3.40 per share. Additionally, in November 2019, we repaid in full all amounts outstanding under our credit agreement with Perceptive Credit Opportunities Fund, L.P., as amended, and we no longer maintain any outstanding debt.

In October 2019, we entered into a transaction pursuant to which we sold approximately 1.4 million ordinary shares of MeiraGTx for gross proceeds of $22.0 million. After consummation of the transaction, we held approximately 5.7% of the outstanding ordinary shares of MeiraGTx with a fair value of $42.0 million recorded as a current investment in equity securities at December 31, 2019.

We filed a shelf registration statement on Form S-3 (File No. 333-233766) on September 13, 2019, which was declared effective by the Securities Exchange Commission ("SEC") on September 24, 2019. Under this registration statement, we may sell, in one or more transactions, up to $200.0 million of common stock, preferred stock, debt securities, warrants, purchase contracts and units, an amount which includes $50.0 million of shares of its common stock that may be issued in one or more "at-the-market" placements at prevailing market prices under our Controlled Equity Offering℠ Sales Agreement (the "Sales Agreement") with Cantor Fitzgerald & Co. ("Cantor Fitzgerald"). We had sold securities totaling an aggregate of $101.6 million pursuant to this registration statement as of December 31, 2019.

In April 2019, we sold 2,538,100 shares of common stock at a price of $2.70 per share and received total gross proceeds of $6.9 million ($6.7 million net of $0.2 million of commissions payable by us) and in January 2019, we sold 13,778,705 shares of common stock at a weighted average price of $2.17 per share and received total gross proceeds of $29.9 million ($29.0 million net of $0.9 million of commissions payable by us). These sales were effected pursuant to our registration statement on Form S-3 (File No. 333-222364), which was declared effective by the SEC on January 10, 2018, under the Sales Agreement.

Going Concern

The accompanying financial statements have been prepared in conformity with accounting principles generally accepted in the United States of America, which contemplate our continuation as a going concern. We have not established a source of revenues sufficient to cover our operating costs, and as such, have been dependent on funding operations through the issuance of debt and sale of equity securities. Since inception, we have experienced significant loses and incurred negative cash flows from operations. We expect to incur further losses over the next several years as we develop our business. We have spent, and expect to continue to spend, a substantial amount of funds in connection with implementing our business strategy, including our planned product development efforts, preparation for our planned clinical trials, performance of clinical trials and our research and discovery efforts.

Our cash and cash equivalents available at December 31, 2019 was $139.6 million, which is expected to enable us to advance our planned Phase 2 clinical studies for KD025, advance certain of our other pipeline product candidates, including KD033 and KD045, and provide for other working capital purposes. Although cash and cash equivalents will be sufficient to fund the foregoing, cash and cash equivalents will not be sufficient to enable us to meet our long-term expected plans, including commercialization of clinical pipeline products, if approved, or initiation or completion of future registrational studies. Following the completion of our ongoing and planned clinical trials, we will likely need to raise additional capital within one year of the issuance of this report to fund continued operations. We have no commitments for any additional financing and may not be successful in our efforts to raise additional funds or achieve profitable operations. Any amounts raised will be used for further development of our product candidates, to provide financing for marketing and promotion, to secure additional property and equipment, and for other working capital purposes.

If we are unable to obtain additional capital (which is not assured at this time), our long-term business plan may not be accomplished and we may be forced to curtail or cease operations. These factors individually and collectively raise substantial doubt about our ability to continue as a going concern. The accompanying financial statements do not include any adjustments or classifications that may result from our possible inability to continue as a going concern.

Sources of Liquidity

Since our inception through December 31, 2019, we have raised net proceeds from the issuance of equity and debt.

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

	Year Ended December 31,	
	2019	2018
	(in thousands)	
Net cash provided by (used in):		
Operating activities	$ (80,067)	$ (71,227)
Investing activities	21,485	(864)
Financing activities	103,439	99,314
Net increase in cash, cash equivalents and restricted cash	$ 44,857	$ 27,223

Operating Activities

The net cash used in operating activities was $80.1 million for the year ended December 31, 2019, and consisted primarily of a net loss of $61.4 million adjusted for $15.1 million in non-cash items and a net decrease in operating assets and liabilities of $3.7 million. The net loss, adjusted for non-cash items, was primarily driven by selling, general and administrative expenses of $26.3 million, research and development expense related to the advancement of our clinical product candidates of $54.2 million and interest paid on our debt of $2.7 million.

The net cash used in operating activities was $71.2 million for the year ended December 31, 2018, and consisted primarily of a net loss of $54.3 million adjusted for $21.6 million in non-cash items, as well as, a net increase in operating assets and liabilities of $4.7 million. The net loss, adjusted for non-cash items, was primarily driven by selling, general and administrative expenses of $28.8 million, research and development expense related to the advancement of our clinical product candidates of $45.9 million and interest paid on our debt of $3.6 million, partially offset by the net sales less cost of sales primarily from Camber products of $0.3 million.

Investing Activities

Net cash provided by (used in) investing activities was $21.5 million and ($0.9) million for the years ended December 31, 2019 and 2018, consisting of proceeds from the sale of a portion of our investment in MeiraGTx of $22.0 million and for 2018 primarily consist of costs related to the purchase lab equipment and in-house software purchased to support our clinical and research operations.

Financing Activities

Net cash provided by financing activities for the year ended December 31, 2019 was $103.4 million, consisting primarily of net proceeds from the issuance of common stock in our 2019 Public Offering and the 2019 placements under our Sales Agreement, together totaling $130.8 million, partially offset by principal payments on our secured term debt of $28.0 million.

Net cash provided by financing activities for the year ended December 31, 2018 was $99.3 million, consisting primarily of net proceeds from the issuance of common stock in a public offering of $105.8 million, partially offset by principal payments on our secured term debt of $6.6 million.

Future Funding Requirements

We expect our expenses to increase compared to prior periods in connection with our ongoing activities, particularly as we continue research and development, continue and initiate clinical trials and seek regulatory approvals for our product candidates. In anticipation of regulatory approval for any of our product candidates, we expect to incur significant pre-commercialization expenses related to product sales, marketing, distribution and manufacturing.

The expected use of our cash and cash equivalents at December 31, 2019 represents our intentions based upon our current plans and business conditions, which could change in the future as our plans and business conditions evolve. The amounts and timing of our actual expenditures may vary significantly depending on numerous factors, including the progress of our development, the status of, and results from, clinical trials, the potential need to conduct additional clinical trials to obtain approval of our product candidates for all intended indications, as well as any additional collaborations that we may

enter into with third parties for our product candidates and any unforeseen cash needs. As a result, our management will retain broad discretion over the allocation of our existing cash and cash equivalents. In addition, we anticipate the need to raise additional funds from the issuance of additional equity, and our management will retain broad discretion over the allocation of those funds as well.

Contractual Obligations and Commitments

The following table summarizes our contractual obligations at December 31, 2019:

	Payments due by period (in thousands)				
	Total	Less than 1 year	1 - 3 years	3 - 5 years	More than 5 years
Operating leases[1]	26,543	4,833	9,805	8,332	3,573
Purchase commitments[2]	1,566	522	783	261	—
Total[3]	$ 28,109	$ 5,355	$ 10,588	$ 8,593	$ 3,573

(1) Operating lease obligations primarily reflect our obligation to make payments in connection with leases for our corporate and clinical headquarters and commercial headquarters distribution center. See Note 8 "Leases" of the notes to our audited consolidated financial statements included elsewhere in this Annual Report on Form 10-K for information on our contractual obligations and commitments related to leases.

(2) The Company has certain non-cancellable minimum batch commitments to purchase CLOVIQUE inventory through 2023.

(3) This table does not include: (a) milestone payments totaling $225.9 million which may become payable to third parties under license agreements as the timing and likelihood of such payments are not known with certainty; (b) any royalty payments to third parties as the amounts, timing and likelihood of such payments are not known with certainty; and (c) contracts that are entered into in the ordinary course of business which are not material in the aggregate in any period presented above.

Off-balance Sheet Arrangements

During the periods presented we did not have, and we do not currently have, any off-balance sheet arrangements, as defined under the SEC rules other than future payments under license agreements as discussed in Note 15 "Commitments and Contingencies" of the notes to our audited consolidated financial statements included elsewhere in this Annual Report on Form 10-K.

Item 7A. Quantitative and Qualitative Disclosures about Market Risk

As a "smaller reporting company," we are not required to provide the information required by this item.

Item 8. Financial Statements and Supplementary Data

Our financial statements, together with the report of our independent registered public accounting firm, appear beginning on page 73 of this Annual Report on Form 10-K.

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

None.

Item 9A. Controls and Procedures

Management's Evaluation of our Disclosure Controls and Procedures

We maintain disclosure controls and procedures that are designed to ensure that information required to be disclosed in the reports that we file or submit under the Securities Exchange Act of 1934, as amended, is (1) recorded, processed, summarized and reported within the time periods specified in the SEC's rules and forms and (2) accumulated and communicated to our management, including our principal executive officer and principal financial officer, to allow timely decisions regarding required disclosure.

At December 31, 2019, our management, with the participation of our principal executive officer and principal financial officer, evaluated the effectiveness of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended). Our management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives, and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Our principal executive officer and principal financial officer have concluded based upon the evaluation described above that, at December 31, 2019, our disclosure controls and procedures were effective at the reasonable assurance level.

Management's Annual Report on Internal Control over Financial Reporting

Our management is responsible for establishing and maintaining adequate internal control over financial reporting (as defined in Rule 13a-15(f) and 15d-15(f) of the Exchange Act). Internal control over financial reporting is a process designed by, or under the supervision of, our Chief Executive Officer and Chief Financial Officer, and effected by our board of directors, management and other personnel, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles and includes those policies and procedures that:

- Pertain to the maintenance of records that accurately and fairly reflect in reasonable detail the transactions and dispositions of the assets of our company;
- Provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that our receipts and expenditures are being made only in accordance with authorizations of our management and directors; and
- Provide reasonable assurances regarding prevention or timely detection of unauthorized acquisition, use or disposition of our assets that could have a material adverse effect on our financial statements.

Under the supervision and with the participation of our management, including our Chief Executive Officer and Chief Financial Officer, we conducted an evaluation of the effectiveness of our internal control over financial reporting as of December 31, 2019 based on the criteria established in Internal Control - Integrated Framework issued by the Committee of Sponsoring Organizations of the Treadway Commission, or COSO 2013. Based on our evaluation under the criteria set forth in Internal Control - Integrated Framework issued by the COSO, our management concluded our internal control over financial reporting was effective as of December 31, 2019.

Attestation Report of the Registered Public Accounting Firm

This Annual Report on Form 10-K does not include an attestation report of our independent registered public accounting firm due to an exemption established by the JOBS Act for "emerging growth 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 fiscal quarter ended December 31, 2019 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 (other than the information set forth in the next paragraph in this Item 10) will be included in our definitive proxy statement with respect to our 2020 Annual Meeting of Shareholders to be filed with the SEC, and is incorporated herein by reference.

We have adopted a written code of business conduct and ethics that applies to our directors, officers and employees, including our principal executive officer, principal financial officer, principal accounting officer, or persons performing similar functions, and third-party consultants. We have posted a current copy of the code on our website, *www.kadmon.com*. In addition, we intend to post on our website all disclosures that are required by law or the NYSE listing standards concerning any amendments to, or waivers from, any provision of the code. The reference to our website does not constitute incorporation by reference of the information contained at or available through our website.

Item 11. Executive Compensation

The information called for by this item will be included in our definitive proxy statement with respect to our 2020 Annual Meeting of Shareholders to be filed with the SEC, and is incorporated herein by reference.

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

The information called for by this item will be included in our definitive proxy statement with respect to our 2020 Annual Meeting of Shareholders to be filed with the SEC, and is incorporated herein by reference.

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

The information required by this item will be included in our definitive proxy statement with respect to our 2020 Annual Meeting of Shareholders to be filed with the SEC, and is incorporated herein by reference.

Item 14. Principal Accounting Fees and Services.

The information required by this item will be included in our definitive proxy statement with respect to our 2020 Annual Meeting of Shareholders to be filed with the SEC, and is incorporated herein by reference.

PART IV

Item 15. Exhibits, Financial Statement Schedules.

The financial statements listed in the Index to Financial Statements beginning on page 73 are filed as part of this Annual Report on Form 10-K.

No financial statement schedules have been filed as part of this Annual Report on Form 10-K because they are not applicable, not required or because the information is otherwise included in our financial statements or notes thereto.

The exhibits filed as part of this Annual Report on Form 10-K are set forth on the Exhibit Index immediately following our financial statements. The Exhibit Index is incorporated herein by reference.

Item 16. Form 10-K Summary

None.

Kadmon Holdings, Inc.

Index to Financial Statements

Report of Independent Registered Public Accounting Firm

The Board of Directors and Stockholders
Kadmon Holdings, Inc.
New York, New York

Opinion on the Consolidated Financial Statements

We have audited the accompanying consolidated balance sheets of Kadmon Holdings, Inc. (the "Company") and subsidiaries as of December 31, 2019 and 2018, the related consolidated statements of operations, stockholders' equity, and cash flows for each of the two years in the period ended December 31, 2019, and the related notes (collectively referred to as the "consolidated financial statements"). In our opinion, the consolidated financial statements present fairly, in all material respects, the financial position of the Company at December 31, 2019 and 2018, and the results of its operations and its cash flows for each of the two years in the period ended December 31, 2019, in conformity with accounting principles generally accepted in the United States of America.

Change in Accounting Principle

As discussed in Note 8 to the consolidated financial statements, effective on January 1, 2019, the Company changed its method of accounting for leases due to the adoption of Accounting Standards Codification Topic 842, *Leases.*

Going Concern Uncertainty

The accompanying consolidated financial statements have been prepared assuming that the Company will continue as a going concern. As discussed in Note 1 to the consolidated financial statements, the Company has incurred recurring losses from operations and expects such losses to continue in the future. These factors raise substantial doubt about the Company's ability to continue as a going concern. Management's plans in regard to these matters are also described in Note 1. The consolidated financial statements do not include any adjustments that might result from the outcome of this uncertainty.

Basis for Opinion

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

We conducted our audits in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the consolidated financial statements are free of material misstatement, whether due to error or fraud. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. As part of our audits we are required to obtain an understanding of internal control over financial reporting but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion.

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

/s/ BDO USA, LLP

We have served as the Company's auditor since 2010.

New York, New York
March 5, 2020

Kadmon Holdings, Inc.
Consolidated balance sheets
(in thousands, except share and per share amounts)

	December 31,	
	2019	2018
Assets		
Current assets:		
Cash and cash equivalents	$ 139,597	$ 94,740
Accounts receivable, net	954	1,690
Inventories, net	640	925
Investment, equity securities	41,997	—
Prepaid expenses and other current assets	1,416	1,581
Total current assets	184,604	98,936
Fixed assets, net	2,444	3,654
Right of use lease asset	19,651	—
Goodwill	3,580	3,580
Restricted cash	2,116	2,116
Investment, equity securities	—	34,075
Investment, at cost	2,300	2,300
Other noncurrent assets	103	—
Total assets	$ 214,798	$ 144,661
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 9,043	$ 9,986
Accrued expenses	14,248	13,508
Lease liability - current	3,966	—
Warrant liabilities	1,485	524
Total current liabilities	28,742	24,018
Lease liability - noncurrent	19,759	—
Deferred rent	—	4,290
Deferred tax liability	461	415
Other long term liabilities	101	47
Secured term debt – net of current portion and discount	—	27,480
Total liabilities	49,063	56,250
Commitments and contingencies (Note 15)		
Stockholders' equity:		
Convertible preferred stock, $0.001 par value; 10,000,000 shares authorized at December 31, 2019 and December 31, 2018; 28,708 and 30,000 shares issued and outstanding at December 31, 2019 and December 31, 2018, respectively	42,433	42,231
Common stock, $0.001 par value; 400,000,000 and 200,000,000 shares authorized at December 31, 2019 and December 31, 2018, respectively; 159,759,996 and 113,130,817 shares issued and outstanding at December 31, 2019 and December 31, 2018, respectively	160	113
Additional paid-in capital	456,211	315,710
Accumulated deficit	(333,069)	(269,643)
Total stockholders' equity	165,735	88,411
Total liabilities and stockholders' equity	$ 214,798	$ 144,661

See accompanying notes to consolidated financial statements

Kadmon Holdings, Inc.
Consolidated statements of operations
(in thousands, except share and per share amounts)

	Years Ended December 31,	
	2019	2018
Revenues		
Net sales	$ 420	$ 691
License and other revenue	4,675	705
Total revenue	5,095	1,396
Cost of sales	377	412
Write-down of inventory	912	270
Gross profit	3,806	714
Operating expenses:		
Research and development	56,461	48,966
Selling, general and administrative	36,425	37,644
Total operating expenses	92,886	86,610
Loss from operations	(89,080)	(85,896)
Other income:		
Interest income	2,067	1,307
Interest expense	(3,381)	(4,619)
Change in fair value of financial instruments	(961)	1,525
Loss on equity method investment	—	(1,242)
Realized gain on equity securities	22,000	—
Unrealized gain on equity securities	7,922	34,075
Other income	111	74
Total other income	27,758	31,120
Loss before income tax expense	(61,322)	(54,776)
Income tax expense (benefit)	46	(524)
Net loss	$ (61,368)	$ (54,252)
Deemed dividend on convertible preferred stock	2,058	2,011
Net loss attributable to common stockholders	$ (63,426)	$ (56,263)
Basic and diluted net loss per share of common stock	$ (0.48)	$ (0.58)
Weighted average basic and diluted shares of common stock outstanding	132,308,548	97,609,000

See accompanying notes to consolidated financial statements

Kadmon Holdings, Inc.
Consolidated statements of stockholders' equity
(in thousands, except share amounts)

	Preferred stock		Common stock		Additional paid-in	Accumulated	
	Shares	Amount	Shares	Amount	capital	Deficit	Total
Balance, January 1, 2018	30,000	$ 40,220	78,643,954	$ 79	$ 198,856	$ (237,397)	$ 1,758
Share-based compensation expense	—	—	—	—	10,391	—	10,391
Common stock issued in public offering, net	—	—	34,303,030	34	105,727	—	105,761
Common stock issued under ESPP plan	—	—	51,999	—	148	—	148
Common stock issued for warrant exercises	—	—	131,834	—	588	—	588
Cumulative effect of change in accounting principle - ASC 606 adoption	—	—	—	—	—	24,017	24,017
Beneficial conversion feature on convertible preferred stock	—	402	—	—	—	(402)	—
Accretion of dividends on convertible preferred stock	—	1,609	—	—	—	(1,609)	—
Net loss	—	—	—	—	—	(54,252)	(54,252)
Balance, December 31, 2018	30,000	$ 42,231	113,130,817	$ 113	$ 315,710	$ (269,643)	$ 88,411
Share-based compensation expense	—	—	—	—	7,208	—	7,208
Common stock issued in public offering, net	—	—	46,216,805	46	130,722	—	130,768
Common stock issued under ESPP plan	—	—	88,619	1	194	—	195
Common stock issued for warrant exercises	—	—	76,776	—	256	—	256
Common stock issued for stock option exercises	—	—	92,334	—	265	—	265
Beneficial conversion feature on convertible preferred stock	—	412	—	—	—	(412)	—
Accretion of dividends on convertible preferred stock	—	1,646	—	—	—	(1,646)	—
Common stock issued upon conversion of convertible preferred stock	(1,292)	(1,856)	154,645	—	1,856	—	—
Net loss	—	—	—	—	—	(61,368)	(61,368)
Balance, December 31, 2019	28,708	$ 42,433	159,759,996	$ 160	$ 456,211	$ (333,069)	$ 165,735

See accompanying notes to consolidated financial statements

Kadmon Holdings, Inc.
Consolidated statements of cash flows
(in thousands)

	Years Ended December 31,	
	2019	**2018**
Cash flows from operating activities:		
Net loss	$ (61,368)	$ (54,252)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization of fixed assets	1,784	1,534
Non-cash operating lease cost	3,354	—
Write-down of inventory	912	270
Amortization of deferred financing costs	—	228
Amortization of debt discount	565	1,170
Amortization of debt premium	—	(344)
Share-based compensation	7,208	10,391
Change in fair value of financial instruments	961	(1,525)
Loss on equity method investment	—	1,242
Realized and unrealized gain on equity securities	(29,922)	(34,075)
Deferred taxes	46	(524)
Changes in operating assets and liabilities:		
Accounts receivable, net	736	(504)
Inventories, net	(627)	(994)
Prepaid expenses and other assets	62	(463)
Accounts payable	(902)	1,967
Lease liability	(3,717)	—
Accrued expenses and other liabilities	841	4,652
Net cash used in operating activities	(80,067)	(71,227)
Cash flows from investing activities:		
Purchases of fixed assets	(515)	(864)
Proceeds from sale of equity securities	22,000	—
Net cash provided by (used in) investing activities	21,485	(864)
Cash flows from financing activities:		
Proceeds from issuance of common stock, net	130,768	105,761
Payment of financing costs	—	(596)
Principal payments on secured term debt	(28,045)	(6,574)
Proceeds from issuance of ESPP shares	195	148
Proceeds from exercise of options	265	—
Proceeds from exercise of warrants	256	575
Net cash provided by financing activities	103,439	99,314
Net increase in cash, cash equivalents and restricted cash	44,857	27,223
Cash, cash equivalents and restricted cash, beginning of period	96,856	69,633
Cash, cash equivalents and restricted cash, end of period	$ 141,713	$ 96,856
Components of cash, cash equivalents, and restricted cash		
Cash and cash equivalents	139,597	94,740
Restricted cash	2,116	2,116
Total cash, cash equivalents, and restricted cash	141,713	96,856
Supplemental cash flow disclosures:		
Cash paid for interest	$ 2,841	$ 3,591
Cash paid for taxes	—	—
Non-cash investing and financing activities:		
Operating lease liabilities arising from obtaining right-of-use assets	212	—
Unpaid fixed asset additions	59	—
Beneficial conversion feature on convertible preferred stock	412	402
Accretion of dividends on convertible preferred stock	1,646	1,609
Common stock issued upon conversion of convertible preferred stock	1,856	—
Increase in lease liabilities from obtaining right-of-use assets – ASC 842 adoption	27,083	—
Cumulative effect of change in accounting principle - ASC 606 adoption	—	24,017
Fair value of modification to lender warrants	—	111

See accompanying notes to consolidated financial statements

Kadmon Holdings, Inc. and Subsidiaries

Notes to consolidated financial statements

1. Organization

Nature of Business

Kadmon Holdings, Inc. (together with its subsidiaries, "Kadmon" or "Company") is a biopharmaceutical company engaged in the discovery, development and commercialization of small molecules and biologics to address significant unmet medical needs, with a near-term clinical focus on immune and fibrotic diseases as well as immuno-oncology. The Company leverages its multi-disciplinary research and clinical development team members to identify and pursue a diverse portfolio of novel product candidates, both through in-licensing products and employing its small molecule and biologics platforms.

Liquidity

The Company had an accumulated deficit of $333.1 million, working capital of $155.9 million, and cash and cash equivalents of $139.6 million at December 31, 2019. Net cash used in operating activities was $80.1 million and $71.2 million for the years ended December 31, 2019 and 2018, respectively. In November 2019, the Company raised $101.6 million ($95.0 million net of $6.6 million of underwriting discounts and other offering expenses payable by the Company) from the issuance of 29,900,000 shares of common stock at a price of $3.40 per share ("2019 Public Offering"). Additionally, in November 2019, the Company repaid in full all amounts outstanding under the 2015 Credit Agreement and the Company no longer maintains any outstanding debt. In October 2019, the Company entered into a transaction pursuant to which it sold approximately 1.4 million ordinary shares of MeiraGTx Holdings plc ("MeiraGTx") for gross proceeds of $22.0 million. After consummation of the transaction, the Company held approximately 5.7% of the outstanding ordinary shares of MeiraGTx with a fair value of $42.0 million at December 31, 2019. The Company expects that its cash and cash equivalents will enable it to advance its clinical studies of KD025 and advance certain of its other pipeline product candidates and provide for other working capital purposes.

Management's plans include continuing to finance operations through the issuance of additional equity securities, monetization of assets and expanding the Company's commercial portfolio through the development of its current pipeline or through strategic collaborations. Any transactions that occur may contain covenants that restrict the ability of management to operate the business or may have rights, preferences or privileges senior to the Company's common stock and may dilute current stockholders of the Company.

The Company filed a shelf registration statement on Form S-3 (File No. 333-233766) on September 13, 2019, which was declared effective by the Securities Exchange Commission ("SEC") on September 24, 2019. Under this registration statement, the Company may sell, in one or more transactions, up to $200.0 million of common stock, preferred stock, debt securities, warrants, purchase contracts and units, an amount which includes $50.0 million of shares of its common stock that may be issued in one or more "at-the-market" placements at prevailing market prices under the Company's Controlled Equity Offering℠ Sales Agreement (the "Sales Agreement") with Cantor Fitzgerald & Co. ("Cantor Fitzgerald"). The Company had sold securities totaling an aggregate of $101.6 million pursuant to this registration statement as of December 31, 2019.

In April 2019, the Company sold 2,538,100 shares of common stock at a price of $2.70 per share and received total gross proceeds of $6.9 million ($6.7 million net of $0.2 million of commissions payable by the Company) and in January 2019, the Company sold 13,778,705 shares of common stock at a weighted average price of $2.17 per share and received total gross proceeds of $29.9 million ($29.0 million net of $0.9 million of commissions payable by the Company). These sales were effected pursuant to the Company's registration statement on Form S-3 (File No. 333-222364), which was declared effective by the SEC on January 10, 2018, under the Sales Agreement.

Going Concern

The accompanying financial statements have been prepared in conformity with accounting principles generally accepted in the United States of America ("GAAP"), which contemplate continuation of the Company as a going concern. The Company has not established a source of revenues sufficient to cover its operating costs, and as such, have been dependent on funding operations through the issuance of debt and sale of equity securities. Since inception, the Company has experienced significant loses and incurred negative cash flows from operations. The Company expects to incur further losses over the next several years as it develops its business. The Company has spent, and expects to continue to spend, a substantial amount of funds in connection with implementing its business strategy, including its planned product development efforts, preparation for its planned clinical trials, performance of clinical trials and its research and discovery efforts.

The Company's cash and cash equivalents at December 31, 2019 was $139.6 million, which is expected to enable the Company to advance its ongoing clinical studies for KD025, advance certain of its other pipeline product candidates,

including KD033 and KD045, and provide for other working capital purposes. Although cash and cash equivalents will be sufficient to fund the foregoing, cash and cash equivalents will not be sufficient to enable the Company to meet its long-term expected plans, including commercialization of clinical pipeline products, if approved, or initiation or completion of future registration studies. Following the completion of its ongoing and planned clinical trials, the Company will likely need to raise additional capital within one year of the issuance of this report to fund continued operations. The Company has no commitments for any additional financing and may not be successful in its efforts to raise additional funds or achieve profitable operations. Any amounts raised will be used for further development of the Company's product candidates, to provide financing for marketing and promotion, to secure additional property and equipment, and for other working capital purposes.

If the Company is unable to obtain additional capital (which is not assured at this time), its long-term business plan may not be accomplished and the Company may be forced to curtail or cease operations. These factors individually and collectively raise substantial doubt about the Company's ability to continue as a going concern. The accompanying financial statements do not include any adjustments or classifications that may result from the possible inability of the Company to continue as a going concern.

2. Summary of Significant Accounting Policies

Basis of Presentation

The Company operates in one segment considering the nature of the Company's products and services, class of customers, methods used to distribute the products and the regulatory environment in which the Company operates. The accompanying consolidated financial statements, which include the accounts of Kadmon Holdings, Inc. and its domestic and international subsidiaries, all of which are wholly owned by Kadmon Holdings, Inc., have been prepared in conformity with GAAP and pursuant to the rules and regulations of the SEC. In the Company's opinion, the financial statements include all adjustments (consisting of normal recurring adjustments) and disclosures considered necessary in order to make the financial statements not misleading.

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 and disclosure of contingent assets and liabilities at the date of the consolidated financial statements. Actual results could differ from those estimates.

Revenue Recognition

The Company adopted FASB ASC 606, Revenue from Contracts with Customers ("ASC 606"), on January 1, 2018 using the modified retrospective method for all contracts not completed as of the date of adoption – i.e., by recognizing the cumulative effect of initially applying ASC 606 as an adjustment to the opening balance of stockholders' equity at January 1, 2018. For contracts that were modified before the effective date, the Company reflected the aggregate effect of all modifications when identifying performance obligations and allocating transaction price in accordance with practical expedient ASC 606-10-65-1-(f)-4.

The Company recognizes revenue in accordance with ASC 606, the core principle of which is that an entity should recognize revenue to depict the transfer of promised goods or services to customers in an amount that reflects the consideration to which the entity expects to be entitled to receive in exchange for those goods or services. To achieve this core principle, five basic criteria must be met before revenue can be recognized: (1) identify the contract with a customer; (2) identify the performance obligations in the contract; (3) determine the transaction price; (4) allocate the transaction price to performance obligations in the contract; and (5) recognize revenue when or as the Company satisfies a performance obligation. The Company only applies the five-step model to contracts when it determines that it is probable it will collect the consideration to which it is entitled in exchange for the goods or services it transfers to the customer. At contract inception, once the contract is determined to be within the scope of ASC 606, the Company assesses the goods or services promised within each contract and determines those that are performance obligations, and assesses whether each promised good or service is distinct. The Company then recognizes as revenue the amount of the transaction price that is allocated to the respective performance obligation when (or as) the performance obligation is satisfied.

Amounts received prior to satisfying the revenue recognition criteria are recognized as deferred revenue in the Company's balance sheet. Amounts expected to be recognized as revenue within the 12 months following the balance sheet date are classified as current portion of deferred revenue. Amounts not expected to be recognized as revenue within the 12 months following the balance sheet date are classified as deferred revenue, net of current portion.

Disaggregation of Revenue

The Company's revenues have primarily been generated through product sales, collaborative research, development and commercialization license agreements, and other service agreements. The following table summarizes revenue from contracts with customers for the year ended December 31, 2019 (in thousands):

	Years Ended December 31, 2019
Product sales	$ 420
License revenue	4,000
Other revenue	675
Total revenue	$ 5,095

Product Sales

The Company markets and distributes products in a variety of therapeutic areas, including CLOVIQUE for the treatment of Wilson's Disease. These contracts typically include a single promise to deliver a fixed amount of product to the customer with payment due within 30 days of shipment. Revenues are recognized when control of the promised goods is transferred to the customer, in an amount that reflects the consideration the Company expects to be entitled to in exchange for those goods. The timing of revenue recognition may differ from the timing of invoicing to customers. The Company has not recognized any assets for costs to obtain or fulfill a contract with a customer as of December 31, 2019.

Sales Returns Reserve, Reserve for Wholesaler Chargebacks and Rebates, and Rebates Payable

As is typical in the pharmaceutical industry, gross product sales are subject to a variety of deductions, primarily representing rebates, chargebacks, returns, and discounts to government agencies, wholesalers, and managed care organizations. These deductions represent management's best estimates of the related reserves and, as such, judgment is required when estimating the impact of these sales deductions on gross sales for a reporting period. If estimates are not representative of the actual future settlement, results could be materially affected. The Company did not have any significant expense related to these sales deductions during 2019 or 2018.

License Revenue

The terms of these license agreements typically may include payment to the Company of one or more of the following: nonrefundable, up-front license fees, research, development and commercial milestone payments; and other contingent payments due based on the activities of the counterparty or the reimbursement by licensees of costs associated with patent maintenance. Each of these types of revenue are recorded as license revenues in the Company's statement of operations.

In determining the appropriate amount of revenue to be recognized as the Company fulfills its obligations under each arrangement, the Company performs the following steps:

i. identify the promised goods and services in the contract;
ii. determine whether the promised goods or services are performance obligations, including whether they are distinct within the context of the contract;
iii. measure the transaction price, including the constraint on variable consideration;
iv. allocate the transaction price to the performance obligations; and
v. recognize revenue when (or as) performance obligations are satisfied

See Note 11, "License Agreements" for additional details regarding the Company's license arrangements.

As part of the accounting for these arrangements, the Company allocates the transaction price to each performance obligation on a relative stand-alone selling price basis. The stand-alone selling price may be, but is not presumed to be, the contract price. In determining the allocation, the Company maximizes the use of observable inputs. When the stand-alone selling price of a good or service is not directly observable, the Company estimates the stand-alone selling price for each performance obligation using assumptions that require judgment. Acceptable estimation methods include, but are not limited to: (i) the adjusted market assessment approach, (ii) the expected cost plus margin approach, and (iii) the residual approach (when the stand-alone selling price is not directly observable and is either highly variable or uncertain). In order for the residual approach to be used, the Company must demonstrate that (a) there are observable stand-alone selling prices for one or more of the performance obligations and (b) one of the two criteria in ASC 606-10- 32-34(c)(1) and (2) is met. The

residual approach cannot be used if it would result in a stand-alone selling price of zero for a performance obligation as a performance obligation, by definition, has value on a stand-alone basis.

An option in a contract to acquire additional goods or services gives rise to a performance obligation only if the option provides a material right to the customer that it would not receive without entering into that contract. Factors that the Company considers in evaluating whether an option represents a material right include, but are not limited to: (i) the overall objective of the arrangement, (ii) the benefit the collaborator might obtain from the arrangement without exercising the option, (iii) the cost to exercise the option (e.g. priced at a significant and incremental discount) and (iv) the likelihood that the option will be exercised. With respect to options determined to be performance obligations, the Company recognizes revenue when those future goods or services are transferred or when the options expire.

The Company's revenue arrangements may include the following:

Up-front License Fees: If a license is determined to be distinct from the other performance obligations identified in the arrangement, the Company recognizes revenues from nonrefundable, up-front fees allocated to the license when the license is transferred to the licensee and the licensee is able to use and benefit from the license. For licenses that are bundled with other promises, the Company utilizes judgment to assess the nature of the combined performance obligation to determine whether the combined performance obligation is satisfied over time or at a point in time and, if over time, the appropriate method of measuring progress for purposes of recognizing revenue from non-refundable, up-front fees. The Company evaluates the measure of progress each reporting period and, if necessary, adjusts the measure of performance and related revenue recognition.

Milestone Payments: At the inception of an agreement that includes research and development milestone payments, the Company evaluates whether each milestone is considered probable of being achieved and estimate the amount to be included in the transaction price using the most likely amount method. If it is probable that a significant revenue reversal would not occur, the associated milestone value is included in the transaction price. Milestone payments that are not within the Company's control or the licensee, such as regulatory approvals, are not considered probable of being achieved until those approvals are received. The transaction price is then allocated to each performance obligation on a relative stand-alone selling price basis, for which the Company recognizes revenue as or when the performance obligations under the contract are satisfied. At the end of each subsequent reporting period, the Company re-evaluates the probability of achievement of such milestones and any related constraint, and if necessary, adjust the Company's estimate of the overall transaction price. Any such adjustments are recorded on a cumulative catch-up basis, which would affect license revenues and earnings in the period of adjustment.

Research and Development Activities: If the Company is entitled to reimbursement from its collaborators for specified research and development activities or the reimbursement of costs associated with patent maintenance, the Company determines whether such funding would result in license revenues or an offset to research and development expenses.

Royalties: If the Company is entitled to receive sales-based royalties from its collaborators, including milestone payments based on the level of sales, and the license is deemed to be the predominant item to which the royalties relate, the Company recognizes revenue at the later of (i) when the related sales occur, provided the reported sales are reliably measurable, or (ii) when the performance obligation to which some or all of the royalty has been allocated has been satisfied (or partially satisfied). To date, the Company has not recognized any royalty revenue resulting from any of its collaboration and license arrangements.

Supply Services: Arrangements that include a promise for future supply of drug substance or drug product or research services at the licensee's discretion are generally considered as options. The Company assesses if these options provide a material right to the licensee and if so, they are accounted for as separate performance obligations. If the Company is entitled to additional payments when the licensee exercises these options, any additional payments are recorded in license revenues when the licensee obtains control of the goods, which is upon delivery, or as the services are performed.

The Company receives payments from its licensees based on schedules established in each contract. Upfront payments and fees are recorded as deferred revenue upon receipt, and may require deferral of revenue recognition to a future period until the Company performs its obligations under these arrangements. Amounts are recorded as accounts receivable when the Company's right to consideration is unconditional. The Company does not assess whether a contract has a significant financing component if the expectation at contract inception is such that the period between payment by the licensees and the transfer of the promised goods or services to the licensees will be one year or less.

Transaction Price Allocated to Future Performance Obligations

ASC 606 requires that the Company disclose the aggregate amount of transaction price that is allocated to performance obligations that have not yet been satisfied as of December 31, 2019. The guidance provides certain practical expedients that limit this requirement. The Company has various contracts that meet the following practical expedients provided by ASC 606:

1. The performance obligation is part of a contract that has an original expected duration of one year or less.

2. Revenue is recognized from the satisfaction of the performance obligations in the amount billable to the customer.

3. The variable consideration is allocated entirely to a wholly unsatisfied performance obligation or to a wholly unsatisfied promise to transfer a distinct good or service that forms part of a single performance obligation.

As of December 31, 2019, the Company had one performance obligation related to a license agreement with Meiji Seika Pharma Co., Ltd that had not yet been satisfied and for which the upfront cash payment had not been received (Note 11). The transaction price of $6.0 million is allocated to the single combined performance obligation under the contract. There are no other performance obligations that have not yet been satisfied as of December 31, 2019 and therefore there is no other transaction price allocated to future performance obligations under ASC 606.

Other Revenue

The other revenue generated by the Company is primarily related to a sublease agreement with MeiraGTx (Note 10). The Company recognizes revenue related to sublease agreements as they are performed.

Share-based Compensation Expense

The Company's accounting policy for share-based compensation is disclosed in Note 12 "Share-based Compensation".

Research and Development Expenses

Costs incurred for research and development are expensed as incurred. Included in research and development expense are personnel related costs, expenditures for laboratory equipment and consumables, payments made pursuant to licensing and acquisition agreements, and the cost of conducting clinical trials. Expenses incurred associated with conducting clinical trials include, but are not limited to, drug development trials and studies, drug manufacturing, laboratory supplies, external research, payroll including stock-based compensation and overhead.

The Company has entered into agreements with third parties to acquire technologies and pharmaceutical product candidates for development (Note 11). Such agreements generally require an initial payment by the Company when the contract is executed, and additional payments upon the achievement of certain milestones. Additionally, the Company may be obligated to make future royalty payments in the event the Company commercializes the pharmaceutical product candidate and achieves a certain sales volume. In accordance with FASB ASC Topic 730-10-55, "Research and Development", expenditures for research and development, including upfront licensing fees and milestone payments associated with products that have not yet been approved by the FDA, are charged to research and development expense as incurred. Future contract milestone payments will be recognized as expense when achievement of the milestone is determined to be probable. Once a product candidate receives regulatory approval, subsequent license payments are recorded as an intangible asset.

Research and development expense was $56.5 million and $49.0 million during the years ended December 31, 2019 and 2018, respectively.

Accruals for Research and Development Expenses and Clinical Trials

As part of the process of preparing its financial statements, the Company is required to recognize its expenses resulting from its obligations under contracts with vendors, clinical research organizations and consultants and under clinical site agreements in connection with conducting clinical trials. This process involves reviewing open contracts and purchase orders, communicating with the applicable personnel to identify services that have been performed on behalf of the Company and estimating the level of service performed and the associated cost incurred for the service when the Company has not yet been invoiced or otherwise notified of actual cost. The majority of service providers invoice the Company monthly in arrears for services performed. The Company makes estimates of accrued expenses as of each balance sheet date in the financial statements based on facts and circumstances known to the Company at that time. The Company's clinical trial accruals are dependent upon the timely and accurate reporting of contract research organizations and other third-party vendors. Although the Company does not expect its estimates to be materially different from amounts actually incurred, its 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 it reporting amounts that are too high or too low for any particular period. For the years ended December 31, 2019 and 2018, there were no material adjustments to the Company's prior period estimates of accrued expenses for clinical trials. The Company periodically confirms the accuracy of its estimates with the service providers and make adjustments if necessary.

Income Taxes

The Company's accounting policy for income taxes is disclosed in Note 17 "Income Taxes".

Cash, Cash Equivalents and Restricted Cash

Cash and cash equivalents are comprised of deposits at major financial banking institutions and highly liquid investments with an original maturity of three months or less at the date of purchase. At December 31, 2019 and 2018, cash equivalents were comprised primarily of money market funds.

The Company has a lease agreement for the premises it occupies in New York. A secured letter of credit in lieu of a lease deposit totaling $2.0 million is secured by restricted cash in the same amount at December 31, 2019 and 2018. The secured letter of credit will remain in place for the life of the related lease, expiring in October 2025 (Note 8). The Company also has a lease agreement for the premises it occupies in Massachusetts. A secured letter of credit in lieu of a lease deposit totaling approximately $0.1 million is secured by restricted cash in the same amount at December 31, 2019 and 2018. The secured letter of credit will remain in place for the life of the related lease, expiring in April 2023 (Note 8).

Concentration of Credit Risk

The Company may from time to time have cash in banks in excess of Federal Deposit Insurance Corporation insurance limits. However, the Company regularly monitors the financial condition of the institutions in which it has depository accounts and believes the risk of loss is minimal as these banks are large financial institutions.

The Company has no off-balance-sheet concentration of credit risk such as foreign exchange contracts, option contracts or other hedging arrangements.

Accounts Receivable and Allowance for Doubtful Accounts

Accounts receivable are recorded at the invoiced amount, net of an allowance for doubtful accounts. A receivable is recognized in the period the Company deliver goods or provide services or when the Company's right to consideration is unconditional. The Company reviews the collectability of accounts receivable based on an assessment of historical experience, current economic conditions, and other collection indicators. The Company had no significant allowance for doubtful accounts at December 31, 2019 or December 31, 2018 and adjustments to the allowance for doubtful accounts amounted to less than $0.1 million for each of the years ended December 31, 2019 and 2018. When accounts are determined to be uncollectible they are written off against the reserve balance and the reserve is reassessed. When payments are received on reserved accounts they are applied to the customer's account and the reserve is reassessed. At December 31, 2019, accounts receivable consist primarily of amounts due from a collaboration agreement. The Company's management believes these receivables are fully collectible.

Inventories

The Company's accounting policy for inventories is disclosed in Note 7 "Inventories".

Investment in Equity Securities

Equity securities consist of investments in common stock of companies traded on public markets (Note 10). These shares are carried on the Company's balance sheet at fair value based on the closing price of the shares owned on the last trading day before the balance sheet of this report. Fluctuations in the underlying bid price of the shares result in unrealized gains or losses. In accordance with FASB ASC 321, Investments – Equity Securities ("ASC 321"), the Company recognizes these fluctuations in value as other expense (income). For investments sold, the Company recognizes the gains and losses attributable to these investments as realized gains or losses in other expense (income).

The Company's total investment balance in equity securities totaled $42.0 million and $34.1 million at December 31, 2019 and 2018, respectively.

Investments

The Company follows FASB ASC Topic 323, "Investments—Equity Method and Joint Ventures" ("ASC 323"), in accounting for its investment in a joint venture. In the event the Company's share of the joint venture's net losses reduces the Company's investment to zero, the Company will discontinue applying the equity method and will not provide for additional losses unless the Company has guaranteed obligations of the joint venture or is otherwise committed to provide further financial support for the joint venture. If the joint venture subsequently reports net income, the Company will resume applying the equity method only after its share of that net income equals the share of net losses not recognized during the period the equity method was suspended.

The Company follows FASB ASC Topic 325, "Investments—Other" ("ASC 325"), in accounting for its investment in the stock of another company accounted for as cost method investments. The Company currently only has one such investment, which is measured in accordance with the "practicability election" allowable for investments without a readily determinable fair value that do not qualify for the NAV practical expedient under ASC 820, "Fair Value Measurement". This requires investments to be measured at cost minus impairment, plus or minus changes resulting from observable price changes in orderly transactions for an identical or similar investment of the same issuer. In the event further contributions or additional shares are purchased, the Company will increase the basis in the investment. In the event distributions are made or indications exist that the fair value of the investment has decreased below the carrying amount, the Company will decrease the value of the investment as considered appropriate. The Company's cost method investment balance totaled $2.3 million at both December 31, 2019 and 2018, respectively.

For all non-consolidated investments, the Company will continually assess the applicability of FASB ASC Topic 810, "Consolidation" ("ASC 810"), to determine if the investments qualify for consolidation. At December 31, 2019 and 2018, no such investments qualified for consolidation.

Fixed Assets

The Company's accounting policy for fixed assets is disclosed in Note 9 "Fixed Assets".

Goodwill

The Company's goodwill relates to the 2010 acquisition of Kadmon Pharmaceuticals, a Pennsylvania limited liability company that was formed in April 2000. Goodwill is not amortized, but rather is assessed for impairment annually or upon the occurrence of an event that indicates impairment may have occurred, in accordance with FASB ASC Topic 350 "Intangibles—Goodwill and Other". The Company maintains a goodwill balance of $3.6 million at both December 31, 2019 and 2018. There were no changes in the carrying amount of goodwill and no impairment to goodwill was recorded for the years ended December 31, 2019 and 2018.

Impairment of Long-Lived Assets

Long-lived assets, including fixed assets and definite-lived intangible assets, are evaluated for impairment periodically, or when events or changes in circumstances indicate that the carrying amount of the assets may not be recoverable. When any such impairment exists, a charge is recorded in the statement of operations to adjust the carrying value of the related assets.

The Company performed a trigger analysis over all other long-lived assets at the lowest identifiable level of cash flows and determined that no impairment triggers existed during the years ended December 31, 2019 and 2018.

Accounting for Leases

The Company's accounting policy for leases is disclosed in Note 8 "Leases".

Accounting for Contingencies

The Company follows the guidance of FASB ASC Topic 450, "Contingencies" ("ASC 450"), in accounting for contingencies. If some amount within a range of loss is probable and appears at the time to be a better estimate than any other amount within the range, that amount shall be expensed. If a loss is probable, and no amount within the range is a better estimate than any other amount, the estimated minimum amount in the range shall be expensed.

Fair Value of Financial Instruments

The Company follows the provisions of FASB ASC Topic 820, "Fair Value Measurements and Disclosures" ("ASC 820"). This pronouncement defines fair value, establishes a framework for measuring fair value under GAAP and requires expanded disclosures about fair value measurements. ASC 820 emphasizes that fair value is a market-based measurement, not an entity-specific measurement, and defines fair value as the price to sell an asset or transfer a liability in an orderly transaction between market participants at the measurement date. ASC 820 discusses valuation techniques, such as the market approach (comparable market prices), the income approach (present value of future income or cash flow) and the cost approach (cost to replace the service capacity of an asset or replacement cost). These valuation techniques are based upon observable and unobservable inputs. Observable inputs reflect market data obtained from independent sources, while unobservable inputs reflect the Company's market assumptions. ASC 820 utilizes a fair value hierarchy that prioritizes inputs to fair value measurement techniques into three broad levels. The following is a brief description of those three levels:

- Level 1: Observable inputs such as quoted prices for identical assets or liabilities in active markets.
- Level 2: Observable inputs other than quoted prices that are directly or indirectly observable for the asset or liability, including quoted prices for similar assets or liabilities in active markets; quoted prices for similar or identical assets or liabilities in markets that are not active; and model-derived valuations whose inputs are observable or whose significant value drivers are observable.
- Level 3: Unobservable inputs that reflect the reporting entity's own assumptions.

The carrying amounts reported in the consolidated balance sheet for cash and cash equivalents, receivables, accounts payable and accrued expenses approximate their fair value based on the short-term nature of these instruments. The carrying amount reported in the consolidated balance sheet for investment in equity securities approximates fair value as the asset has a readily determinable market value (Note 10).

Warrants and Derivative Liabilities

The Company accounts for its derivative financial instruments in accordance with FASB ASC Topic 815, "Derivatives and Hedging" ("ASC 815"). The Company does not have derivative financial instruments that are hedges. ASC 815 establishes accounting and reporting standards requiring that derivative instruments, both freestanding and embedded in other contracts, be recorded on the balance sheet as either an asset or liability measured at its fair value each reporting period. ASC 815 also requires that changes in the fair value of derivative instruments be recognized currently in the results of operations unless specific criteria are met. For embedded features that are not clearly and closely related to the host instrument, are not carried at fair value, and are derivatives, the feature will be bifurcated and recorded as an asset or liability as noted above, unless the exceptions below are not met. Freestanding instruments that do not meet these exceptions will be accounted for in the same manner.

ASC 815 provides an exception—if an embedded derivative or freestanding instrument is both indexed to the company's own stock and classified in stockholders' equity, it can be accounted for in stockholders' equity. If at least one of the criteria is not met, the embedded derivative or warrant is classified as an asset or liability and recorded to fair value each reporting period through the income statement.

The Company has historically issued warrants in connection with debt and equity issuances. The Company assesses classification of its warrants and embedded features at each reporting date to determine whether a change in classification is required. The Company's accounting for its embedded warrants are explained further in Note 6.

Recent Accounting Pronouncements

In December 2019, the Financial Accounting Standards Board ("FASB") issued Accounting Standards Update ("ASU") No. 2019-12, *"Income Taxes: Simplifying the Accounting for Income Taxes"*, which removes certain exceptions for recognizing deferred taxes for investments, performing intraperiod allocation and calculating income taxes in interim periods. The ASU also adds guidance to reduce complexity in certain areas, including recognizing deferred taxes for tax goodwill and allocating taxes to members of a consolidated group. The ASU is effective for annual or interim periods beginning after December 15, 2020. Early adoption is permitted for periods for which financial statements have not been issued. The Company does not expect the standard to have a significant impact on its consolidated financial statements.

In November 2018, the FASB issued ASU No. 2018-18, *"Collaborative Arrangements (Topic 808): Clarifying the Interaction Between Topic 808 and Topic 606"*, which requires transactions in collaborative arrangements to be accounted for under ASC 606 if the counterparty is a customer for a good or service (or bundle of goods and services) that is a distinct unit of account. The amendments also preclude entities from presenting consideration from transactions with a collaborator

that is not a customer together with revenue recognized from contracts with customers. The ASU is effective for annual or interim periods beginning after December 15, 2019. Early adoption is permitted for entities that have adopted ASC 606. The Company does not expect the standard to have a significant impact on its consolidated financial statements.

In August 2018, the FASB issued ASU No. 2018-15, "*Intangibles—Goodwill and Other—Internal-Use Software (Subtopic 350-40): Customer's Accounting for Implementation Costs Incurred in a Cloud Computing Arrangement That Is a Service Contract (a consensus of the FASB Emerging Issues Task Force)*", which requires customers in a cloud computing arrangement that is a service contract to follow the internal-use software guidance in Accounting Standards Codification 350-40 to determine which implementation costs to capitalize as assets. This ASU is effective for annual or any interim periods beginning after December 15, 2019. The Company does not expect the standard to have a significant impact on its consolidated financial statements, as the Company's cloud computing contracts are not material.

In June 2018, the FASB issued ASU No. 2018-07, "*Compensation – Stock Compensation*", which expands the scope of Topic 718 to include share-based payment transactions for acquiring goods and services from nonemployees, except for specific exceptions. This ASU is effective for annual or any interim periods beginning after December 15, 2018. The Company adopted this standard on January 1, 2019, and the standard did not have a significant impact on its consolidated financial statements as the fair value of the Company's awards to non-employees is not material.

In January 2017, the FASB issued ASU No. 2017-04, "*Intangibles – Goodwill and Other*", which simplifies the subsequent measurement of goodwill by eliminating "Step 2" from the goodwill impairment test. Instead of performing Step 2 to determine the amount of an impairment charge, the fair value of a reporting unit will be compared with its carrying amount and an impairment charge will be recognized for the value by which the carrying amount exceeds the reporting unit's fair value. For smaller reporting companies, ASU 2017-04 is effective for annual or any interim goodwill impairment tests in fiscal years beginning after December 15, 2022. Early adoption is permitted for interim or annual goodwill impairment tests performed on testing dates after January 1, 2017. The Company does not expect the standard to have a significant impact on its consolidated financial statements.

In June 2016, the FASB issued ASU No. 2016-13, "*Measurement of Credit Losses on Financial Instruments*", to require financial assets carried at amortized cost to be presented at the net amount expected to be collected based on historical experience, current conditions and forecasts. For smaller reporting companies, the ASU is effective for interim and annual periods beginning after December 15, 2022, with early adoption permitted. Adoption of the ASU is on a modified retrospective basis. The Company does not expect this guidance to have a material impact on its financial statements

3. Stockholders' Equity

5% Convertible Preferred Stock

The Company's certificate of incorporation permitted the Company's board of directors to issue up to 10,000,000 shares of preferred stock from time to time in one or more classes or series. Concurrently with the closing of the Company's initial public offering (the "IPO") in 2016 and pursuant to the terms of the exchange agreement entered into with the holders of the Company's Senior Convertible Term Loan, the Company issued to such holders 30,000 shares of 5% convertible preferred stock, designated as the convertible preferred stock. Each share of convertible preferred stock was issued for an amount equal to $1,000 per share, which is referred to as the original purchase price. Shares of convertible preferred stock with an aggregate original purchase price and initial liquidation preference of $30.0 million were issued to the holders of the Senior Convertible Term Loan in exchange for an equivalent principal amount of the Senior Convertible Term Loan pursuant to the terms of an exchange agreement dated as of June 8, 2016, between the Company and those holders, which is referred to as the exchange agreement.

The shares of 5% convertible preferred stock are entitled to receive dividends, when and as declared by the board of directors and to the extent of funds legally available for the payment of dividends, at an annual rate of 5% of the sum of the original purchase price per share of 5% convertible preferred stock plus any dividend arrearages. Dividends on the 5% convertible preferred stock shall, at the Company's option, either be paid in cash or added to the stated liquidation preference amount for purposes of calculating dividends at the 5% annual rate (until such time as the Company declares and pays the missed dividend in full and in cash, at which time that dividend will no longer be part of the stated liquidation preference amount). Dividends shall be payable annually on June 30 of each year and shall be cumulative from the most recent dividend payment date on which the dividend has been paid or, if no dividend has ever been paid, from the original date of issuance of the 5% convertible preferred stock and shall accumulate from day to day whether or not declared until paid.

The Company had 28,708 shares of 5% convertible preferred stock outstanding at December 31, 2019, which shares convert into shares of the Company's common stock at a 20% discount to the initial public offering price per share of common stock in the Company's IPO of $12.00 per share, or $9.60 per share. In May 2019, a holder of 1,292 shares of 5% convertible preferred stock exercised its right to convert such shares into 154,645 shares of the Company's common stock.

The 5% convertible preferred stock, inclusive of accrued and unpaid dividends, is convertible into 3,536,125 and 3,519,303 shares of common stock at December 31, 2019 and 2018, respectively.

The Company accrued dividends on the 5% convertible preferred stock of $1.6 million for each of the years ended December 31, 2019 and 2018. The Company also calculated a deemed dividend of $0.4 million on the $1.6 million of accrued dividends for each of the years ended December 31, 2019 and 2018, which equals the 20% discount to the IPO price of the Company's common stock of $12.00 per share, a beneficial conversion feature. Approximately $1.6 million of accrued dividends that were payable on both June 30, 2019 and June 30, 2018, were added to the stated liquidation preference amount of the 5% convertible preferred stock on those respective dates. The stated liquidation preference amount on the 5% convertible preferred stock totaled $33.1 million and $33.0 million at December 31, 2019 and December 31, 2018, respectively.

Common Stock

On May 15, 2019, the Company's stockholders approved an amendment to the Company's certificate of incorporation to increase the number of shares of common stock, par value $0.001 per share, that the Company is authorized to issue from 200,000,000 to 400,000,000.

For the year ended December 31, 2019, the Company raised an aggregate of $138.5 million, $130.8 million net of $7.7 million of underwriting discounts and other offering costs and expenses, from the issuance of 46,216,805 shares of common stock at a weighted average issuance price of $3.00 per share.

For the year ended December 31, 2018, the Company raised $113.2 million, $105.8 million net of $7.4 million of underwriting discounts and other offering costs and expenses, from the issuance of 34,303,030 shares of common stock at a price of $3.30 per share ("2018 Public Offering").

4. Net Loss per Share Attributable to Common Stockholders

Basic net loss per share attributable to common stockholders is computed by dividing the net loss attributable to common stockholders by the weighted-average number of common stock outstanding for the period. Because the Company has reported a net loss for all periods presented, diluted net loss per common share is the same as basic net loss per common share for those periods. The following table summarizes the computation of basic and diluted net loss per share attributable to common stockholders of the Company (in thousands, except share and per share amounts):

	Years Ended December 31,	
	2019	2018
Numerator – basic and diluted:		
Net loss attributable to common stockholders	$ (63,426)	$ (56,263)
Denominator – basic and diluted:		
Weighted average common stock outstanding used to compute basic and diluted net loss per share	132,308,548	97,609,000
Net loss per share, basic and diluted	**$ (0.48)**	**$ (0.58)**

The amounts in the table below were excluded from the calculation of diluted net loss per share, due to their anti-dilutive effect:

	Years Ended December 31,	
	2019	2018
Options to purchase common stock	13,092,601	11,054,539
Warrants to purchase common stock	11,921,452	11,999,852
Convertible preferred stock	3,536,125	3,519,303
Total shares of common stock equivalents	**28,550,178**	**26,573,694**

5. Debt

Secured Term Debt

August 2015 Secured Term Debt

In August 2015, the Company entered into a secured term loan in the amount of $35.0 million with two lenders ("2015 Credit Agreement"). The interest rate on the loan was LIBOR plus 9.375% with a 1% floor. At December 31, 2018, the Company maintained an outstanding principal balance of $28.0 million under the 2015 Credit Agreement.

In October 2019, the Company entered into a transaction pursuant to which it sold approximately 1.4 million ordinary shares of MeiraGTx for gross proceeds of $22.0 million (Note 10). Pursuant to the 2015 Credit Agreement, half of the proceeds received from the sale, or $11.0 million, were used to pay down part of the outstanding amounts owed under the 2015 Credit Agreement. After this repayment, approximately $17.0 million of principal remained outstanding under the 2015 Credit Agreement. In November 2019, the Company repaid the remaining $17.0 million of principal outstanding under the credit agreement with Perceptive Credit Opportunities Fund, L.P., as amended. As such, the Company has no further payment obligations under the 2015 Credit Agreement at December 31, 2019.

The following table provides components of interest expense and other related financing costs (in thousands):

	Years Ended December 31,	
	2019	2018
Interest expense	$ 2,816	$ 3,565
Amortization of deferred financing costs, debt discount and debt premium	565	1,054
Interest expense	**$ 3,381**	**$ 4,619**

6. Financial Instruments

Equity issued pursuant to Credit Agreements

In connection with the 2015 Credit Agreement (Note 5), as fees to the lenders thereunder, the Company issued warrants to purchase an aggregate of $6.3 million of the Company's Class A units with an expiration date of August 2022, which were exchanged for 617,651 warrants with a strike price of $10.20 per share to purchase the same number of shares of the Company's common stock upon consummation of the Company's IPO in August 2016 (the "2015 Warrants").

As of December 31, 2019, the exercise price of a portion of the 2015 Warrants to purchase an aggregate of 529,413 shares of the Company's common stock was $3.30 per warrant share and the exercise price of the remaining 2015 Warrants to purchase an aggregate of 88,238 shares of the Company's common stock was $4.50 per warrant share. Since these warrants are exercisable and are redeemable at the option of the holder upon the occurrence of, and during the continuance of, an event of default under the warrant agreement, the fair value of the 2015 Warrants was recorded as a short-term liability of approximately $1.5 million at December, 31 2019 and approximately $0.5 million at December 31, 2018.

The Company used the Black-Scholes pricing model to value the liability related to the 2015 Warrants at December 31, 2019 and 2018 with the following assumptions:

	December 31, 2019	December 31, 2018
Stock Price	$4.53	$2.08
Strike price	$3.30 - $4.50	$3.30 - $4.50
Expected Volatility	72.20%	72.44%
Risk-free interest rate	1.62%	2.47%
Expected term	2.7 years	3.7 years
Expected dividend yield	0%	0%

The change in fair value of the 2015 Warrants was $1.0 million and $(0.8) million for the years ended December 31, 2019 and 2018, respectively. None of these instruments have been exercised at December 31, 2019 or December 31, 2018.

Other Warrants

In connection with the incurrence of the Senior Convertible Term Loan in 2015, the Company issued three tranches of warrants as fees to the lenders that were redeemable for Class A units. Upon consummation of the Company's IPO in

2016, the warrants to purchase Class A units issued to lenders in the Senior Convertible Term Loan were exchanged for 351,992 warrants with a strike price of $10.20 per share to purchase the same number of shares of the Company's common stock. None of these warrants have been exercised at December 31, 2019.

On April 16, 2013, the Company issued warrants with an estimated fair value of $1.4 million for the purchase of 30,000 Class A units at a strike price of $21.24 as consideration for fundraising efforts performed. Upon consummation of the Company's IPO in 2016, these warrants to purchase Class A units were exchanged for 46,163 warrants to purchase the same number of shares of the Company's common stock at a strike price of $138.06. None of these warrants have been exercised at December 31, 2019.

In connection with the sale of common stock in March 2017, warrants to purchase 2,707,138 shares of common stock were issued at an exercise price of $4.50 per share. During April 2018, warrants to purchase 119,047 shares of common stock were exercised for which the Company received proceeds of $0.5 million. The remaining 2,588,091 warrants expired in April 2018. These warrants included a cash settlement option requiring the Company to record a liability for the fair value of the warrants at the time of issuance and at each reporting period with any change in the fair value reported as other income or expense. At the time of issuance, approximately $1.6 million was recorded as warrant liability. The decline in the fair value of these warrants was ($0.7) million for the year ended December 31, 2018. As these warrants expired in April 2018, no change in fair value was recorded for these warrants after April 2018.

In connection with the 2017 Public Offering, the Company issued warrants to purchase 10,710,000 shares of common stock at an initial exercise price of $3.35 per share for a term of 5 years from the date of issuance. As of December 31, 2019, warrants to purchase 10,593,000 shares of common stock were outstanding. During 2019, the Company received proceeds of $0.3 million related to exercises of these warrants.

Fair Value Classification

The Company held certain liabilities that are required to be measured at fair value on a recurring basis. Fair value guidance establishes a three-tier fair value hierarchy, which prioritizes the inputs used in measuring fair value (Note 2).

The table below represents the values of the Company's financial instruments at December 31, 2019 and December 31, 2018 (in thousands):

	Fair Value Measurement Using Significant Other Observable Inputs (Level 2)	
Description	December 31, 2019	December 31, 2018
Warrants	$ 1,485	$ 524
Total	**$ 1,485**	**$ 524**

The table below represents a rollforward of the Level 2 financial instruments from January 1, 2018 to December 31, 2019 (in thousands).

	Significant Other Observable Inputs (Level 2)
Balance as of January 1, 2018	**$ 1,952**
Change in fair value of financial instruments	(1,525)
Fair value of modification to warrants issued to lenders	111
Exercise of warrants recorded as liability	(14)
Balance as of December 31, 2018	**$ 524**
Change in fair value of financial instruments	961
Balance as of December 31, 2019	**$ 1,485**

The Level 2 inputs used to value the Company's financial instruments were determined using prices that can be directly observed or corroborated in active markets. Although the fair value of this obligation is calculated using the observable market price of Kadmon Holdings Inc. common stock, an active market for this financial instrument does not exist and therefore the Company has classified the fair value of this liability as a Level 2 liability in the table above.

Warrants Outstanding

The following table summarizes information about warrants outstanding at December 31, 2019 and 2018:

	Warrants	Weighted Average Exercise Price
Balance, January 1, 2018	14,722,790	$ 5.94
Exercised	(134,847)	4.21
Forfeited	(2,588,091)	4.50
Balance, December 31, 2018	11,999,852	$ 5.95
Exercised	(78,400)	3.35
Balance, December 31, 2019	11,921,452	$ 5.97

7. Inventories

Inventories are stated at the lower of cost or net realizable value (on a first-in, first-out basis) using standard costs. Standard costs include an allocation of overhead rates, which include those costs attributable to managing the supply chain and are evaluated regularly. Variances are expensed as incurred.

The Company regularly reviews the expiration date of its inventories and maintains a reserve for inventories that are probable to expire before shipment. Inventories recorded on the Company's consolidated balance sheets are net of a reserve for expirable inventory of $3.0 million and $2.2 million at December 31, 2019 and 2018, respectively. The Company expensed inventory that it believes will not be sold prior to reaching its product expiration date totaling $0.9 million and $0.3 million during the years ended December 31, 2019 and 2018, respectively. If the amount and timing of future sales differ from management's assumptions, adjustments to the estimated inventory reserves may be required.

Inventories Produced in Preparation for Product Launches

The Company capitalizes inventories produced in preparation for product launches sufficient to support estimated initial market demand. Typically, capitalization of such inventory begins when positive results have been obtained for the clinical trials that the Company believes are necessary to support regulatory approval, uncertainties regarding ultimate regulatory approval have been significantly reduced and the Company has determined it is probable that these capitalized costs will provide some future economic benefit in excess of capitalized costs. The material factors considered by the Company in evaluating these uncertainties include the receipt and analysis of positive clinical trial results for the underlying product candidate, results from meetings with the relevant regulatory authorities prior to the filing of regulatory applications, and the compilation of the regulatory application. The Company closely monitors the status of each respective product within the regulatory approval process, including all relevant communication with regulatory authorities. If the Company is aware of any specific material risks or contingencies other than the normal regulatory review and approval process or if there are any specific issues identified relating to safety, efficacy, manufacturing, marketing or labeling, the related inventory would generally not be capitalized.

For inventories that are capitalized in preparation of product launch, anticipated future sales, expected approval date and shelf lives are evaluated in assessing realizability. The shelf life of a product is determined as part of the regulatory approval process; however, in evaluating whether to capitalize pre-launch inventory production costs, the Company considers the product stability data of all of the pre-approval production to date to determine whether there is adequate expected shelf life for the capitalized pre-launch production costs.

In September 2019, the U.S. Food and Drug Administration ("FDA") approved the Company's generic trientine hydrochloride capsules USP, 250 mg and in October 2019, the FDA approved CLOVIQUE™ (trientine hydrochloride capsules, USP), the Company's room-temperature stable, branded generic product (together, "CLOVIQUE"). Trientine hydrochloride is used for the treatment of Wilson's disease in patients who are intolerant of penicillamine. CLOVIQUE™ is the first FDA-approved trientine product in a portable blister pack that offers room temperature stability for up to 30 days, potentially providing patients more convenience. Accordingly, the pre-launch costs of these products are realizable as the Company expects the inventory will be sold or used prior to expiration. The Company maintained $0.6 million and $0.9 million of trientine hydrochloride inventory at December 31, 2019 and December 31, 2018, respectively.

Inventories are comprised of the following (in thousands):

	December 31, 2019	December 31, 2018
Raw materials	$ 371	$ —
Work-in-process	—	886
Finished goods, net	269	39
Total inventories	**$ 640**	**$ 925**

8. Leases

In February 2016, the FASB issued ASU No. 2016 02, Leases (“ASC 842”), to enhance the transparency and comparability of financial reporting related to leasing arrangements. Under this new lease standard, most leases are required to be recognized on the balance sheet as right-of-use (“ROU”) assets and lease liabilities. Disclosure requirements have been enhanced with the objective of enabling financial statement users to assess the amount, timing and uncertainty of cash flows arising from leases. Prior to January 1, 2019, GAAP did not require lessees to recognize assets and liabilities related to operating leases on the balance sheet. The new standard establishes a ROU model that requires a lessee to recognize a ROU asset and corresponding lease liability on the balance sheet for all leases with a term longer than 12 months. Leases will be classified as finance or operating, with classification affecting the pattern and classification of expense recognition in the statement of operations as well as the reduction of the ROU asset.

The Company has adopted the standard effective January 1, 2019 using the modified retrospective transition approach allowed under ASU 2018-11, Leases (Topic 842: Targeted Improvements), which releases companies from presenting comparative periods and related disclosures under ASC 842 and requires a cumulative-effect adjustment to the opening balance of retained earnings in the period of adoption. Consequently, financial information will not be updated and the disclosures required under the new standard will not be provided for dates and periods prior to January 1, 2019. The new standard provides a number of optional practical expedients in transition. The Company has elected to apply the ‘package of practical expedients’, which allow it to not reassess (i) whether existing or expired arrangements contain a lease, (ii) the lease classification of existing or expired leases, or (iii) whether previous initial direct costs would qualify for capitalization under the new lease standard. The Company has also elected to apply (i) the practical expedient, which allows it to not separate lease and non-lease components, for new leases entered into after adoption and (ii) the short-term lease exemption for all leases with an original term of less than 12 months, for purposes of applying the recognition and measurements requirements in the new standard. As of December 31, 2019, the short-term lease exemption applied to two operating leases for office space which are for a term of one year.

At the inception of an arrangement, the Company determines whether the arrangement is or contains a lease based on specific facts and circumstances, the existence of an identified asset(s), if any, and the Company’s control over the use of the identified asset(s), if applicable. Operating lease liabilities and their corresponding ROU assets are recorded based on the present value of future lease payments over the expected lease term. The interest rate implicit in lease contracts is typically not readily determinable. As such, the Company will utilize the incremental borrowing rate, which is the rate incurred to borrow on a collateralized basis over a similar term an amount equal to the lease payments in a similar economic environment. As of the ASC 842 effective date, the Company’s incremental borrowing rate ranged from approximately 4.0%-5.6% based on the remaining lease term of the applicable leases.

Operating leases are recognized on the balance sheet as ROU lease assets, lease liabilities current and lease liabilities non-current. Fixed rents are included in the calculation of the lease balances while variable costs paid for certain operating and pass-through costs are excluded. Lease expense is recognized over the expected term on a straight-line basis.

The Company is party to six operating leases for office or laboratory space and three finance leases for office IT equipment. The Company’s finance leases are immaterial both individually and in the aggregate. The Company has applied the guidance in ASC 842 to its corporate office and laboratory leases and has determined that these should be classified as operating leases. Consequently, as a result of the adoption of ASC 842, the Company recognized a ROU lease asset of approximately $22.7 million with a corresponding lease liability of approximately $27.0 million based on the present value of the minimum rental payments of such leases. In accordance with ASC 842, the beginning balance of the ROU lease asset was reduced by the existing deferred rent liability at inception of approximately $4.3 million. In the consolidated balance sheets at December 31, 2019, the Company has a ROU asset balance of $19.7 million and a current and non-current lease liability of $4.0 million and $19.8 million, respectively, relating to the ROU lease asset. The balance of both the ROU lease asset and the lease liabilities primarily consists of future payments under the Company’s office lease in New York, New York.

The Company is party to an operating lease in New York, New York for office and laboratory space for its headquarters. The lease commenced in October 2010, its initial term is set to expire in February 2021, and the Company opened a secured letter of credit with a third party financial institution in lieu of providing a security deposit of $2.0 million,

which letter of credit is included in restricted cash at December 31, 2019. As of December 31, 2019, there were six amendments to this lease agreement, which altered office and laboratory capacity and extended the lease term through October 2025, with total lease cost of $4.7 million for the year ended December 31, 2019. This office lease contains the ability to extend portions of the lease at fair market value but does not have any renewal options.

The Company is party to an operating lease in Warrendale, Pennsylvania for the Company's specialty-focused commercial operation. In March 2019, the Company entered into an amendment to this lease, which extended the lease term to September 30, 2022 with two five-year renewal options, which would extend the term to September 30, 2032, if exercised. Rental payments under the renewal period would be at market rates determined from the average rentals of similar tenants in the same industrial park. The option to renew this office lease was not considered when assessing the value of the ROU asset because the Company was not reasonably certain that it would assert its option to renew the lease. Total lease cost for this lease was $0.7 million for the year ended December 31, 2019.

In August 2015, the Company entered into an operating office lease agreement in Cambridge, Massachusetts for the Company's clinical office effective January 2016 and expiring in April 2023. The Company opened a secured letter of credit with a third party financial institution in lieu of providing a security deposit of $0.1 million, which letter of credit is included in restricted cash at 2019. The Company is also party to an operating lease for laboratory space in Princeton, New Jersey, which expires in February 2021. Neither of these office leases contain any renewal options. Total lease cost for these leases was $0.4 million for the year ended December 31, 2019.

Quantitative information regarding the Company's leases for the year ended December 31, 2019 is as follows (in thousands):

Lease Cost	**Classification**		**Year Ended December 31, 2019**
Operating lease cost [a]	SG&A expenses	$	4,632
Variable lease cost	SG&A expenses		1,346
Sublease income [b]	Other revenue		(674)
Net Lease Cost		$	5,304
Other Information			
Operating cash flows paid for amounts included in the measurement of lease liabilities		$	4,679
Operating lease liabilities arising from obtaining ROU assets			212
Weighted average remaining lease term (years)			5.5
Weighted average discount rate			4.1%

(a) Includes short-term lease costs and finance leases costs, which are immaterial.
(b) Includes sublease income related to MeiraGTx (Note 10).

Future lease payments under noncancellable leases are as follows (in thousands) at December 31, 2019:

Year ending December 31,	**Operating Leases**	**Finance Leases**
2020	$ 4,833	$ 48
2021	4,937	6
2022	4,868	—
2023	4,174	—
2024	4,158	—
Thereafter	3,573	—
Total Lease Payments	**$ 26,543**	**$ 54**
Less: Imputed Interest	(2,866)	(6)
Total Lease Liabilities	**$ 23,677**	**$ 48**

Note: As most of the Company's leases do not provide an implicit rate, the Company uses its incremental borrowing rate based on the information available at commencement date in determining the present value of lease payments. The Company used the incremental borrowing rate on January 1, 2019 for operating leases that commenced prior to that date.

Future minimum rental payments under noncancellable leases are as follows (in thousands) at December 31, 2018:

Year ending December 31,	Amount
2019	$ 4,672
2020	4,204
2021	4,177
2022	4,286
2023	4,153
Thereafter	7,731
Total	**$ 29,223**

9. Fixed Assets

Fixed assets are carried at cost less accumulated depreciation and amortization. Depreciated and amortization of fixed assets is calculated using the straight-line method over their estimated useful lives. Leasehold improvements are amortized over the shorter of their estimated useful lives or the lease term, using the straight-line method. Construction-in-progress and software under development are stated at cost and not depreciated. These items are transferred to fixed assets when the assets are placed into service.

When assets are retired or otherwise disposed of, the cost and accumulated depreciation are removed from the balance sheet and any resulting gain or loss is reflected in operations in the period realized. Expenditures for repairs and maintenance, which do not improve or extend the life of the assets, are expensed as incurred.

Fixed assets consisted of the following (in thousands):

	Useful Lives (Years)	December 31, 2019	December 31, 2018
Leasehold improvements	4-8	$ 10,397	$ 10,187
Office equipment and furniture	3-15	1,234	1,529
Machinery and laboratory equipment	3-15	3,599	3,247
Software	1-5	3,971	3,831
Construction-in-progress	—	45	45
		19,246	18,839
Less accumulated depreciation and amortization		(16,802)	(15,185)
Fixed assets, net		$ 2,444	$ 3,654

Depreciation and amortization of fixed assets totaled $1.8 million and $1.5 million in the years ended December 31, 2019 and 2018, respectively. The construction-in-progress balance was related to costs of unimplemented software still under development. Unamortized computer software costs were $0.3 million and $0.7 million at December 31, 2019 and 2018, respectively. The amortization of computer software costs amounted to $0.4 million and $0.2 million during the years ended December 31, 2019 and 2018, respectively.

10. Investment in MeiraGTx

In April 2015, the Company executed several agreements which transferred its ownership of Kadmon Gene Therapy, LLC to MeiraGTx. As part of these agreements, the Company also transferred various property rights, employees and management tied to the intellectual property and contracts identified in the agreements to MeiraGTx. At a later date, MeiraGTx ratified its shareholder agreement and accepted the pending equity subscription agreements, which provided equity ownership to various parties. The execution of these agreements resulted in a 48% ownership in MeiraGTx by the Company. On June 12, 2018, MeiraGTx completed its initial public offering (the “MeiraGTx IPO”) whereby it sold 5,000,000 ordinary shares at $15.00 per share. The shares began trading on the Nasdaq Global Select Market on June 7, 2018 under the symbol “MGTX.”

Prior to the MeiraGTx IPO, the Company had no remaining basis in any of the investments held in MeiraGTx. Upon completion of the MeiraGTx IPO, the Company’s investment was diluted to a 13.0% ownership in MeiraGTx ordinary shares and the Company no longer had the ability to exert significant influence over MeiraGTx. For the period beginning January 1, 2018 through June 12, 2018, the Company recorded its share of MeiraGTx’s net loss under the equity method of accounting amounting to $1.2 million. The Company discontinued the equity method of accounting for the investment in MeiraGTx on

June 12, 2018 and determined the remaining investment to be an equity security accounted for in accordance with ASC 321 at the date the investment no longer qualifies for the equity method of accounting. ASC 321 requires the investments to be recorded at cost, less any impairment, plus or minus changes resulting from observable price changes in orderly transactions for the identical or similar investment of the same issuer. As the Company's investment in MeiraGTx ordinary shares had a readily determinable market value, the Company recorded an unrealized gain of $40.5 million in June 2018 related to the fair value of its ownership of ordinary shares of MeiraGTx.

In October 2019, the Company entered into a transaction pursuant to which it sold approximately 1.4 million ordinary shares of MeiraGTx for gross proceeds of $22.0 million, which was recorded as a realized gain on equity securities. The Company has recorded a net unrealized gain on its MeiraGTx ordinary share investment of $7.9 million and $34.1 million for the years ended December 31, 2019 and 2018, respectively. The realized gains represent the total gains on shares sold since the MeiraGTx IPO. The unrealized gains on equity securities consists of two components: (i) the reversal of the gain or loss recognized in previous periods on equity securities sold and (ii) the change in unrealized gain or loss resulting from mark-to-market adjustments on equity securities still held. The table below represents a rollforward of the MeiraGTx investment from January 1, 2018 to December 31, 2019 (in thousands).

	Significant Observable Inputs (Level 1)
Balance as of January 1, 2018	**$ —**
Unrealized gain on investment at completion of MeirGTx IPO	40,508
Unrealized loss on investment	(6,433)
Balance as of December 31, 2018	**$ 34,075**
Unrealized gain on investment sold during year	8,148
Realized gain on sale of investment	(22,000)
Unrealized gain on remaining investment	21,774
Balance as of December 31, 2019	**$ 41,997**

As of December 31, 2019 and 2018, the Company maintained a 5.7% and 12.9% ownership in the ordinary shares of MeiraGTx with a fair value of $42.0 million and $34.1 million, respectively. As of December 31, 2018, the investment was recorded as a noncurrent investment in equity securities since depending on certain circumstances, the Company could, at times, have been deemed to be an affiliate of MeiraGTx. During the third quarter of 2019 the affiliate restrictions on the resale of these securities were removed and, accordingly, the Company's investment in MeiraGTx has been recorded as a current investment in equity securities at December 31, 2019. The investment in MeiraGTx is valued using Level 1 inputs which includes quoted prices in active markets for identical assets in accordance with the fair value hierarchy (Note 2).

As part of the agreements executed with MeiraGTx in April 2015, the Company entered into a transition services agreement ("TSA") with MeiraGTx which expired in April 2018. Upon expiration of the TSA, the Company continued to provide office space to MeiraGTx. On October 1, 2018, the Company and MeiraGTx entered into a sublease agreement which is effective from October 1, 2018 for a period of two months and will automatically be renewed on a monthly basis unless MeiraGTx provides 30 days prior written notice. The monthly sublease amount is approximately $49 thousand. As part of the TSA and sublease agreement with MeiraGTx, the Company recognized $0.6 million and $0.6 million to license and other revenue during the years ended December 31, 2019 and 2018, respectively. The Company received cash payments of $0.6 million and $1.4 million from MeiraGTx during 2019 and 2018, respectively, and the Company has no amounts receivable from MeiraGTx at either December 31, 2019 or 2018.

11. License Agreements

Nano Terra, Inc.

On April 8, 2011, the Company entered into a series of transactions with Nano Terra, Inc. ("Nano Terra"), pursuant to which the Company (i) paid $2.3 million for Nano Terra's Series B Preferred Stock, (ii) entered into a joint venture with Surface Logix, Inc. ("Surface Logix") (Nano Terra's wholly-owned subsidiary) through the formation of NT Life Sciences, LLC ("NT Life"), whereby the Company contributed $0.9 million at the date of formation in exchange for a 50% interest in NT Life and (iii) entered into a sub-licensing arrangement with NT Life and Surface Logix. Pursuant to the sub-licensing arrangement, the Company was granted a worldwide, exclusive license under certain intellectual property owned by Surface Logix to three clinical-stage product candidates, as well as rights to Surface Logix's drug discovery platform, Pharmacomer™ Technology, each of which were licensed by Surface Logix to NT Life. In December 2014, the Company received one share of Nano Terra's Common Stock for every 100 shares of Series B Preferred Stock held by the Company, resulting in approximately a 1% holding in Nano Terra as of December 31, 2019 and 2018. In accordance with ASC 325, "Investments—Other", the Company continues to account for the investment under the cost method (Note 2).

The primary product candidates are currently in early to mid-stage clinical development for a variety of diseases and target several novel pathways of disease by inhibiting the activity of specific enzymes.

Nano Terra and NT Life are research and development companies, each of which independently maintains intellectual property for the purpose of pursuing medical discoveries. The Company is a minority shareholder of Nano Terra and thereby is unable to exercise significant influence with regard to the entity's daily operations. The Company is represented on the board of managers of NT Life and is a party to decisions which influence the direction of the organization.

Since inception, the Company has continuously assessed the applicability of ASC 810, based on the corporate structure, voting rights and contributions of the various parties in connection with these agreements, and determined that Nano Terra and NT Life are not variable interest entities ("VIE") and not subject to consolidation. On April 8, 2011 the Company recorded its $2.3 million investment in Nano Terra in accordance with ASC 325 and the Company has assessed the recoverability of the investment in Nano Terra as of December 31, 2019 and 2018 and identified no events or changes in circumstances that may have a significant adverse impact on the fair value of this investment. No impairment or changes resulting from observable price changes occurred during the year ended December 31, 2019. There was no activity of the joint venture during the years ended December 31, 2019 and 2018 which resulted in income or loss to the Company. The Company's maximum exposure associated with Nano Terra and NT Life is limited to cash contributions made.

Additionally, subject to certain exceptions, the Company must pay a percentage that ranges between a low twenty percent and a low forty percent of all sublicensing revenue the Company receives in the event the Company further assigns or sublicenses its rights under the sub-licensing arrangement to certain third parties. In addition, the Company must pay to the previous shareholders of Surface Logix from which Nano Terra acquired Surface Logix and NT Life royalties on net sales in the amount of 5% and 10%, respectively. As the Company owns 50% of NT Life, the cumulative results of these obligations is that the Company will owe an aggregate royalty a 9.75% on net sales of licensed products. No sublicensing revenue or sales were achieved as of December 31, 2019 and 2018.

Dyax Corp. (acquired by Shire Plc in January 2016, acquired by Takeda Pharmaceuticals Co., Ltd. in 2018)

On July 22, 2011 the Company entered into a license agreement with Dyax Corp. ("Dyax") for the rights to use the Dyax Antibody Libraries, Dyax Materials and Dyax Know-How (collectively "Dyax Property"). The agreement terminated on September 22, 2015, but the Company had a right to a commercial license of any research target within two years of expiration of the agreement. The Company exercised its right to a commercial license of two targets in September 2017, resulting in a license fee payable to Shire Plc of $1.5 million which was recorded to research and development expense for the year ended December 31, 2017. Under the terms of the agreement, the Company recorded $1.5 million in development milestone expense during the fourth quarter of 2019 related to development milestones reached by the Company.

BioNova Pharmaceuticals Ltd.

In November 2019, the Company entered into a strategic partnership with BioNova Pharmaceuticals Ltd. ("BioNova") to form a joint venture to exclusively develop and commercialize KD025, the Company's lead product candidate, for the treatment of graft-versus-host-disease ("GVHD") in the People's Republic of China. The joint venture was entered into through the creation of BK Pharmaceuticals Limited ("BK Pharma"), whereby the Company entered into a royalty-bearing exclusive license agreement with BK Pharma and BioNova in exchange for a 20% interest in BK Pharma. BK Pharma is domiciled in Hong Kong with shared oversight between the Company and BioNova.

Under the terms of the license agreement, the Company received an upfront payment of $4.0 million in December 2019 and is eligible to receive an additional $41.0 million in development, regulatory and commercial milestone payments upon the occurrence of specified events over the term of the agreement. In addition, the Company is eligible to receive double-digit percentage royalty payments on sales of KD025 for GVHD in China.

The Company assessed the applicability of ASC 810 to the aforementioned agreements and based on the corporate structure, voting rights and contributions of the various parties in connection with these agreements, determined that BK Pharma was a VIE, however consolidation was not required as the Company was not the primary beneficiary based upon the voting and managerial structure of the entity. The purpose of the VIE is to develop and commercialize KD025 in China and the operations of BK Pharma will be financed and guaranteed entirely by BioNova. The Company has not and is not required to provide financial support under the agreements and has no exposure to loss as a result of its involvement in the VIE. The Company's investment in BK Pharma was accounted for under the equity method as the Company has the ability to exercise significant influence over BK Pharma. The equity method investment was recorded at immaterial cost representing the Company's initial capital contribution for its ownership. This value was determined based upon the corporate structure which does not allocate profits or losses to the Company. An adjustment to this recorded investment will only occur upon a sales transaction or liquidation event, as defined in the agreement.

The Company evaluated the arrangement under ASC 808, *Collaborative Arrangements* ("ASC 808"), and determined that the license agreement and related joint venture with BioNova is not within the scope of ASC 808, and that the license agreement represents a contract with a customer under ASC 606. The Company has determined that the license agreement contains a single performance obligation that consists of the exclusive license to Kadmon's intellectual property and related initial technology transfer. All other promises included in the license agreement were deemed to be immaterial in the context of the contract including clinical supply, participation in a joint steering committee ("JSC"), and limited technical assistance as requested by BK Pharma and BioNova.

The Company determined that the $4.0 million non-refundable, upfront payment under the license agreement constituted the entire consideration to be included in the transaction price at the inception of the arrangement. As such, this amount was allocated to the single performance obligation. The potential development, regulatory and commercial milestone payments and sales-based royalties that the Company is eligible to receive represent variable consideration under the license agreement. The development and regulatory milestone amounts were excluded from the transaction price and were fully constrained based on their probability of achievement and the fact that Company cannot reasonably conclude that a significant reversal of revenue related to these milestones would not occur. Any future sales-based royalties, including commercial milestone payments based on the level of sales, will be included in the transaction price and recognized as revenue when the related sales occur and the milestones are achieved. The Company will reevaluate the transaction price at the end of each reporting period as uncertain events are resolved or other changes in circumstances occur, and, if necessary, adjust its estimate of the transaction price.

The single performance obligation represents a license of functional intellectual property and the associated revenue was recognized upon completion of the initial technology transfer in December 2019. The Company recognized $4.0 million in license revenue for the year ended December 31, 2019. No other milestone or royalty revenues have been earned as of December 31, 2019.

Meiji Seika Pharma Co., Ltd

In December 2019, the Company entered into a collaboration agreement with Meiji Seika Pharma Co., Ltd ("Meiji"), a Tokyo-based wholly owned subsidiary of Meiji Holdings Co., Ltd., to form a joint venture to exclusively develop and commercialize KD025 in Japan and certain other Asian countries. The joint venture was entered into through the creation of Romeck Pharma, LLC ("Romeck"), whereby the Company entered into a royalty-bearing exclusive license agreement with Romeck and Meiji in exchange for a 50% interest in Romeck. Romeck is domiciled in Japan with shared oversight between the Company and Meiji.

Under the terms of the license agreement, the Company received an upfront payment of $6.0 million in January 2020 and is eligible to receive an additional $23.0 million in development, regulatory and commercial milestone payments upon the occurrence of specified events over the term of the agreement. In addition, the Company is eligible to receive double-digit percentage royalty payments on sales of KD025 for GVHD in Japan.

The Company assessed the applicability of ASC 810 to the aforementioned agreements and based on the corporate structure, voting rights and contributions of the various parties in connection with these agreements, determined that BK Pharma was a VIE, however consolidation was not required as the Company was not the primary beneficiary based upon the voting and managerial structure of the entity. The purpose of the VIE is to develop and commercialize KD025 in Japan and the operations of Romeck will be financed entirely by Meiji. The Company has not and is not required to provide financial support under the agreements and has no exposure to loss as a result of its involvement in the VIE. The Company's investment in Romeck was accounted for under the equity method as the Company has the ability to exercise significant influence over Romeck. The equity method investment was recorded at immaterial cost representing the Company's initial capital contribution for its ownership. This value was determined based upon the corporate structure which does not allocate profits or losses to the Company. An adjustment to this recorded investment will only occur upon a sales transaction or liquidation event, as defined in the agreement.

The Company evaluated the arrangement under ASC 808 and determined that the license agreement and related joint venture with Romeck is not within the scope of ASC 808, and that the license agreement represents a contract with a customer under ASC 606. The Company has determined that the license agreement contains a single performance obligation that consists of the exclusive license to Kadmon's intellectual property and related initial technology transfer. All other promises included in the license agreement were deemed to be immaterial in the context of the contract including clinical supply, participation in a JSC, and limited technical assistance as requested by Romeck and Meiji.

The Company determined that the $6.0 million non-refundable, upfront payment under the license agreement constituted the entire consideration to be included in the transaction price at the inception of the arrangement. As such, this amount was allocated to the single performance obligation. The potential development, regulatory and commercial milestone payments and sales-based royalties that the Company is eligible to receive represent variable consideration under the license

agreement. The development and regulatory milestone amounts were excluded from the transaction price and were fully constrained based on their probability of achievement and the fact that Company cannot reasonably conclude that a significant reversal of revenue related to these milestones would not occur. Any future sales-based royalties, including commercial milestone payments based on the level of sales, will be included in the transaction price and recognized as revenue when the related sales occur and the milestones are achieved. The Company will reevaluate the transaction price at the end of each reporting period as uncertain events are resolved or other changes in circumstances occur, and, if necessary, adjust its estimate of the transaction price.

The single performance obligation represents a license of functional intellectual property and the associated revenue was recognized upon completion of the initial technology transfer in the first quarter of 2020. The Company had not received the upfront payment or completed the single combined performance obligation as of December 31, 2019. As such, the Company recognized $6.0 million in license revenue in the first quarter of 2020 upon completion of the single combined performance obligation. No other milestone or royalty revenues have been earned as of December 31, 2019.

12. Share-based Compensation

Accounting for Share-based Compensation

The Company recognizes share-based compensation expense in accordance with FASB ASC Topic 718, “Stock Compensation” (“ASC 718”), for all share-based awards made to employees, non-employees and board members based on estimated fair values. ASC 718 requires companies to measure the cost of employee services incurred in exchange for the award of equity instruments based on the estimated fair value of the share-based award on the grant date. The expense is recognized over the requisite service period. The Company recognizes share-based award forfeitures only as they occur rather than an estimate by applying a forfeiture rate.

The Company uses a Black-Scholes option-pricing model to value the Company’s option awards. Using this option-pricing model, the fair value of each employee and board member award is estimated on the grant date. The fair value is expensed on a straight-line basis over the vesting period. The option awards generally vest pro-rata annually. The expected volatility assumption is based on the volatility of the share price of comparable public companies. The expected life is determined using the “simplified method” permitted by Staff Accounting Bulletin Numbers 107 and 110 (the midpoint between the term of the agreement and the weighted average vesting term). The risk-free interest rate is based on the implied yield on a U.S. Treasury security at a constant maturity with a remaining term equal to the expected term of the option granted. The dividend yield is zero, as the Company has never declared a cash dividend.

As of December 31, 2019, the only equity compensation plans from which the Company may currently issue new awards are the Company’s 2016 Equity Incentive Plan (as amended and restated to date, the “2016 Equity Plan”) and 2016 Employee Stock Purchase Plan (as amended and restated to date, the “2016 ESPP”), each as more fully described below.

2016 Equity Incentive Plan

In July 2016, the Company adopted the 2016 Equity Plan. Pursuant to the 2016 Equity Plan, the Company’s Board of Directors may grant incentive and nonqualified stock options, stock appreciation rights, restricted stock, restricted stock units, performance shares and units and other cash-based or stock-based awards.

The 2016 Equity Plan provides for annual increases in the number of shares available for issuance under the 2016 Equity Plan on January 1 of each year by an amount equal to the smaller of (a) 4% of the number of shares of common stock issued and outstanding on the immediately preceding December 31, or (b) an amount determined by the board of directors. On January 1, 2019, pursuant to the evergreen provision contained in the 2016 Equity Plan, the number of shares reserved for future grants was increased by 4,525,233 shares, which was four percent (4%) of the outstanding shares of common stock on December 31, 2018. As of December 31, 2019, there was a total of 16,194,138 shares reserved for issuance under the 2016 Equity Plan and there were 2,937,486 shares available for future grants. Options issued under the 2016 Equity Plan generally vest over 3 years from the date of grant in equal annual tranches and are exercisable for up to 10 years from the date of issuance. Upon exercise of stock options granted under the 2016 Equity Plan, the Company issues new shares from the shares reserved for issuance. Shares subject to awards which expire or are cancelled or forfeited will again become available for issuance under the 2016 Equity Plan. The shares available will not be reduced by awards settled in cash or by shares withheld to satisfy tax withholding obligations. Only the net number of shares issued upon the exercise of stock appreciation rights or options exercised by means of a net exercise or by tender of previously owned shares will be deducted from the shares available under the 2016 Equity Plan.

In accordance with the terms of the 2016 Equity Plan, effective as of January 1, 2020, the number of shares of common stock available for issuance under the 2016 Plan increased by 5,591,600 shares, which was less than four percent

(4%) of the outstanding shares of common stock on December 31, 2019. As of January 1, 2020, the 2016 Equity Plan had a total reserve of 21,785,738 shares and there were 8,529,086 shares available for future grants.

The Company recorded share-based option compensation expense under the 2016 Equity Plan of $7.2 million and $10.4 million for the years ended December 31, 2019 and 2018, respectively. Total unrecognized compensation expense related to unvested options granted under the Company's share-based compensation plan was $6.5 million and $6.8 million at December 31, 2019 and 2018, respectively. That expense is expected to be recognized over a weighted average period of 2.2 years and 1.5 years as of December 31, 2019 and 2018, respectively.

The following table summarizes information about stock options outstanding, not including performance stock options, at December 31, 2019 and 2018:

	Options Outstanding			
	Number of Options	Weighted Average Exercise Price	Weighted Average Remaining Contractual Term (years)	Aggregate Intrinsic Value (in thousands)
Balance, January 1, 2018	8,496,872	$ 6.96	8.83	$ —
Granted	2,110,424	2.76		
Exercised	—	—		
Forfeited	(842,757)	4.82		
Balance, December 31, 2018	9,764,539	$ 6.24	7.84	$ —
Granted	2,943,973	3.11		
Exercised	(92,334)	2.87		
Forfeited	(813,577)	4.63		
Balance, December 31, 2019	11,802,601	$ 5.59	7.52	$ 9,520,190
Options vested and exercisable, December 31, 2019	8,171,838	$ 6.65	6.73	$ 4,726,277

The aggregate intrinsic value in the table above represents the total pre-tax intrinsic value calculated as the difference between the fair value of the Company's common stock at December 31, 2019 ($4.53 per share) and December 31, 2018 ($2.08 per share) and the exercise price, multiplied by the related in-the-money options that would have been received by the option holders had they exercised their options at the end of the fiscal year. This amount changes based on the fair value of the Company's common stock. There were 92,334 options exercised during the year ended December 31, 2019 with an aggregate intrinsic value of $0.2 million. There were no options exercised during the year ended December 31, 2018.

The weighted-average fair value of the stock option awards, not including performance stock options, granted to employees, officers, directors and advisors was $2.07 and $1.69 during the years ended December 31, 2019 and 2018, respectively, and was estimated at the date of grant using the Black-Scholes option-pricing model and the assumptions noted in the following table:

	Years Ended	
	December 31, 2019	December 31, 2018
Weighted average fair value of grants	$2.07	$1.69
Expected volatility	75.96% - 77.73%	72.94% - 75.92%
Risk-free interest rate	1.41% - 2.61%	2.44% - 2.90%
Expected life	5.5 - 6.0 years	5.5 - 6.0 years
Expected dividend yield	0%	0%

Performance Awards

On April 3, 2018 the Company granted 1,597,500 nonqualified performance-based stock options ("Performance Options") to certain executive officers under the 2016 Equity Plan, which represents the maximum number of Performance Options that may be earned if all three performance milestones (each, a "Performance Goal") are achieved during the three-year period following the Grant Date (the "Performance Period"). The Performance Options may be earned based on the achievement of three separate Performance Goals related to the Company's operating and research and development activities during the Performance Period, subject to employment through the achievement date. In addition to the achievement of the Performance Goals, the Performance Options are also subject to time-based vesting requirements. Each Performance Option was granted with an exercise price of $4.06 per share and does not contain any voting rights. No other Performance Options have been granted under the 2016 Equity Plan.

Any Performance Options earned upon the achievement of a Performance Goal will generally vest in three equal installments on specified vesting dates between the date of achievement of the Performance Goal and the third anniversary of the Grant Date based on continued employment; *provided*, that, if the relevant achievement date for a Performance Goal occurs after the second anniversary of the Grant Date, the full vesting of the Options earned will occur on the one year anniversary of the date of achievement of the applicable Performance Goal.

Compensation expense for the Performance Options is recognized on a straight-line basis over the awards' requisite service period. The Performance Options vest upon the satisfaction of both a service condition and the satisfaction of one or more performance conditions, therefore the Company initially determined which outcomes are probable of achievement. The Company believes that the three-year service condition (explicit service period) and all three performance conditions (implicit service periods) will be satisfied. The requisite service period would be three years as that is the longest period of both the explicit service period and the implicit service periods. The first two performance conditions were satisfied during 2018 and the third performance condition was satisfied in 2019.

The weighted-average fair value of the Performance Options granted was $2.71 and was estimated at the date of grant using the Black-Scholes option-pricing model with the following assumptions: risk-free interest rate of 2.67%, expected term of 6.0 years, expected volatility of 74.50%, and a dividend rate of 0%.

During the year ended December 31, 2018, 307,500 Performance Options were forfeited. At December 31, 2019, 1,290,000 Performance Options are outstanding with a weighted average remaining contractual life of 6.7 years. Total unrecognized compensation expense related to unvested Performance Options was $0.5 million at December 31, 2019. The unrecognized compensation cost is expected to be recognized over a weighted-average period of 1.2 years. No Performance Options were exercised during the years ended December 31, 2019 and 2018.

Stock Appreciation Rights

The Company granted 1,040,000 stock appreciation rights under the 2016 Equity Plan to three executive employees during the year ended December 31, 2017. No other stock appreciation rights have been granted under the 2016 Equity Plan. During the year ended December 31, 2018, 205,000 stock appreciation rights were forfeited. At December 31, 2019, 835,000 stock appreciation rights are outstanding with a weighted average remaining contractual life of 6.6 years.

Compensation expense for stock appreciation rights is recognized on a straight-line basis over the awards' requisite service period. At December 31, 2019, there was $0.5 million of total unrecognized compensation cost related to stock appreciation rights. The unrecognized compensation cost is expected to be recognized over a weighted-average period of 0.9 years. No stock appreciation rights were exercised during the years ended December 31, 2019 and 2018.

2014 Long-term Incentive Plan ("LTIP")

The LTIP was adopted in May 2014 and amended in December 2014. Under the LTIP, the Company's board of directors may grant up to 10% of the equity value of the Company in Equity Appreciation Rights Units ("EAR units") or Performance Awards. In 2015 and 2014, certain employees were granted a total of 9,750 EAR units with a base price of $6.00 per unit, expiring 10 years from the grant date. No other awards have been granted under the LTIP and no future awards are available to be granted under the LTIP.

The EAR units vested in 2016 and are payable upon the fair market value of the Company's common stock exceeding 333% of the $6.00 grant price ($20.00) per share prior to December 7, 2024. The EAR units are also payable upon a change in control where the acquisition price of the Company's common stock exceeds $6.00 per share. The payment amount with respect to the holder's EAR units will be determined using the fair market value of the common stock on the trading date immediately preceding the settlement date. Each payment under the Award will be made in a lump sum and is considered a separate payment. The holders of the LTIP have no right to demand a particular form of payment, and the Company reserves the right to make payment in the form of cash or common stock. Any settlement in the form of common stock will be limited to a maximum share allocation, which is 3,478,057 shares of the Company's common stock.

A total of 9,750 units were outstanding under the LTIP at December 31, 2019 and 2018. The compensation expense for this award was recognized upon consummation of the Company's IPO in 2016 and was recorded as additional paid in capital.

2016 Employee Stock Purchase Plan

In July 2016, the Company adopted the 2016 ESPP. The 2016 ESPP provides for annual increases in the number of shares available for sale under the 2016 ESPP on January 1 of each year by an amount equal to the smaller of (a) 750,000 shares, (b) one-and-a-half percent (1.5%) of the number of shares of common stock issued and outstanding on the immediately preceding December 31, or (c) an amount determined by the board of directors. On January 1, 2019 and January

1, 2020, pursuant to the evergreen provision contained in the 2016 ESPP, the number of shares reserved for sale was not increased by the Company's board of directors. As of December 31, 2019, there was a total of 2,551,180 shares reserved for sale under the 2016 ESPP and there were 2,399,968 shares available for future sales.

The Company issued 88,619 shares and 51,999 shares of common stock under the 2016 ESPP during the years ended December 31, 2019 and 2018, respectively. No significant compensation expense was recognized for the ESPP during the years ended December 31, 2019 and 2018.

13. Accrued Expenses

Short-term accrued expenses at December 31, 2019 and 2018 include the following (in thousands):

	December 31, 2019	December 31, 2018
Commission payable	$ 2,395	$ 2,395
Compensation, benefits and severance	4,668	3,848
Research and development	4,962	4,847
Other	2,223	2,418
Total Accrued Expenses	$ 14,248	$ 13,508

Commission payable

During 2014 and 2015, the Company raised $40.4 million in gross proceeds, $37.2 million net of $3.2 million in transaction costs. At December 31, 2019 and 2018, $2.4 million remains in accrued liabilities relating to commissions to third parties for these equity raises during 2015 and 2014.

Compensation, benefits and severance

Compensation, benefits and severance represent earned and unpaid employee wages and bonuses, as well as contractual severance to be paid to former employees. At December 31, 2019 and December 31, 2018, these accrued expenses totaled $4.7 million and $3.8 million, respectively.

In August 2019, the Company entered into a Separation Agreement and General Release (the "Separation Agreement") with Steven N. Gordon, Esq., Executive Vice President, General Counsel, Chief Administrative, Compliance and Legal Officer, and Corporate Secretary of the Company. The Separation Agreement provides that Mr. Gordon will receive, among other things, $0.9 million in aggregate cash payments (including reimbursement of certain of Mr. Gordon's expenses) over 18 months. At December 31, 2019, $0.6 million of severance payable to Mr. Gordon was recorded as accrued expenses while $0.1 million was recorded as other long-term liabilities. Further, the Company has recorded insurance receivable payments related to this matter in the amount of $0.4 million in accounts receivable and $0.1 million in other long-term assets.

Separately, a separation agreement with Dr. Samuel D. Waksal, which expired on February 8, 2019, contained severance payments and certain supplement conditional payments. The Company has not recorded any expense related to these conditional payments as none of the conditional payments were met as of the expiration of the agreement on February 8, 2019.

Research and development

The Company has contracts with third parties for the development of the Company's product candidates. The timing of the expenses varies depending upon the timing of initiation of clinical trials and enrollment of patients in clinical trials. At December 31, 2019 and 2018, accrued research and development expenses for which the Company has not yet been invoiced totaled $5.0 million and $4.8 million, respectively.

14. Employee Benefit Plan

In October 2011, the Company began sponsoring a qualified Tax Deferred Savings Plan (401(k)) for all eligible employees of the Company. Participation in the plan is voluntary. Participating employees may defer up to 75% of their compensation up to the maximum prescribed by the Internal Revenue Code. The Company has an obligation to match non-highly compensated employee contributions of up to 6% of deferrals and also has the option to make discretionary matching contributions and profit sharing contributions to the plan annually, as determined by the Company's board of directors.

The Company expensed employer matching contributions of $0.3 million and $0.2 million for the years ended December 31, 2019 and 2018, respectively. The Company made disbursements of $0.2 million in each of the years ended December 31, 2019 and 2018. The Company typically disburses employer matching contributions during the first quarter following the plan year.

15. Commitments and Contingencies

Lease Commitments

The Company's commitments related to lease agreements are disclosed in Note 8.

Purchase Commitments

The Company has certain non-cancellable minimum batch commitments to purchase CLOVIQUE inventory through 2023. These commitments include $0.5 million for 2020, $0.4 million for 2021 and $0.3 million for both 2022 and 2023. The Company recorded less than $0.1 million of expense related to the CLOVIQUE purchase commitment for the year ended December 31, 2019.

Employment Agreements

Certain employment agreements provide for severance compensation. The Company has recorded a liability for such agreements of $0.8 million and $1.0 million at December 31, 2019 and 2018, respectively (Note 13).

Licensing Contingencies

The Company has entered into several license agreements for products currently under development (Note 11). The Company may be obligated in future periods to make additional payments, which would become due and payable only upon the achievement of certain research and development, regulatory, and approval milestones. The specific timing of such milestones cannot be predicted and depends upon future discretionary clinical developments as well as regulatory agency actions which cannot be predicted with certainty (including action which may never occur). These additional contingent milestone payments aggregate to $225.9 million at December 31, 2019. Any payments made prior to FDA approval will be expensed as research and development. Payments made after FDA approval will be capitalized.

Further, under the terms of certain licensing agreements, the Company may be obligated to pay commercial milestones contingent upon the realization of sales revenues and sublicense revenues. Due to the long-range nature of such commercial milestones, they are neither probable at this time nor predictable, and consequently are not included in the additional contingent milestone payment amount.

Legal Contingencies

The Company has been subject to various legal proceedings that arise from time to time in the ordinary course of its business. Although the Company believes that the various proceedings brought against it have been without merit, and that it has adequate product liability and other insurance to cover any claims, litigation is subject to many factors which are difficult to predict and there can be no assurance that the Company will not incur material costs in the resolution of legal matters. Should the Company determine that any future obligations exist, the Company will record an expense equal to the amount which is deemed probable and estimable. The Company has no significant contingencies related to legal proceedings at December 31, 2019.

16. Related Party Transactions

Certain of the Company's existing institutional investors purchased an aggregate of 10,444,117 shares of the Company's common stock in the public offering that closed on November 18, 2019. Consonance Capital Management LP purchased 4,900,000 shares of the Company's common stock for $16.7 million, Perceptive Advisors LLC purchased 1,470,588 shares of the Company's common stock for $5.0 million, Vivo Capital VIII LLC purchased 1,323,529 shares of the Company's common stock for $4.5 million, Acuta Capital Partners LLC purchased 1,250,000 shares of the Company's

common stock for $4.3 million and Millenium Management LLC purchased 1,500,000 shares of the Company's common stock for $5.1 million.

17. Income Taxes

The Company accounts for income taxes in accordance with the asset and liability method of accounting for income taxes prescribed by FASB ASC Topic 740, "Accounting for Income Taxes" ("ASC 740"). Under the asset and liability method of ASC 740, deferred tax assets and liabilities are recognized for the future tax consequences attributable to differences between the financial statement carrying amounts of existing assets and liabilities and their respective tax basis and operating loss and tax credit carryforwards. Deferred tax assets and liabilities are measured using enacted tax rates expected to apply to the taxable income in the years in which those temporary differences are expected to be recovered or settled. Under ASC 740, the effect on deferred tax assets and liabilities of a change in tax rates is recognized in income in the period that includes the enactment dates.

The Company follows FASB ASC Topic 740-10, "Accounting for Uncertainty in Income Taxes", which prescribes a recognition threshold and measurement attribute for financial statement recognition and measurement of a tax position taken, or expected to be taken, in a tax return. For those benefits to be recognized, a tax position must be more likely than not to be sustained upon examination by taxing authorities. At December 31, 2019 and 2018, the Company had no material uncertain tax positions to be accounted for in the financial statements. The Company recognizes interest and penalties, if any, related to unrecognized tax benefits in interest expense. The Company is obligated to file income tax returns in the U.S. federal jurisdiction and several U.S. States. Since the Company had losses in the past, all prior years that generated net operating loss carryforwards are open and subject to audit examination in relation to the net operating loss generated from those years.

No provision or benefit for federal or state income taxes has been recorded, as the Company has incurred a net loss for all of the periods presented, and the Company has provided a full valuation allowance against its deferred tax assets.

The Company recorded an income tax expense of less than $0.1 million for the year ended December 31, 2019, related to an adjustment to the deferred tax liability. The Company recorded an income tax benefit of $0.5 million for the year ended December 31, 2018, primarily related to an adjustment to the deferred tax liability, as explained below.

During the year ended December 31, 2018, in accordance with the Tax Cuts and Jobs Act (the "Act"), the Company determined it necessary to reduce the recorded deferred tax liability by $0.6 million. The deferred tax liability was initially recorded to account for the book vs. tax basis difference related to the goodwill intangible asset, also known as a "naked credit". The deferred tax liability was excluded from sources of future taxable income, as the timing of its reversal cannot be predicted due to the indefinite life of the goodwill. As such, this deferred tax liability cannot be used to offset the valuation allowance. In accordance with the Act, losses generated beginning with the 2018 tax year may be carried forward indefinitely for U.S. federal tax purposes. However, such losses are limited to offset 80% of taxable income in future years. Thus, 80% of the U.S. federal deferred tax liability related to goodwill may be used to offset the valuation allowance, resulting in a reduction of the Company's deferred tax liability of $0.6 million.

The income tax expense (benefit) differs from the expense (benefit) that would result from applying federal statutory rates to loss before income taxes as follows (in thousands):

	For the Years Ended December 31,			
	2019		2018	
	Amount	Rate	Amount	Rate
Expected federal statutory income tax	$ (12,897)	21.0%	$ (11,283)	21.0%
State income taxes, net of federal benefits	1,700	-2.8%	(3,649)	6.8%
Adjustment to deferred tax assets related to ownership change	26,287	-42.8%	—	0.0%
Adjustment to deferred tax assets	469	-0.8%	8,578	-16.0%
Change in valuation allowance	(15,513)	25.3%	5,830	-10.8%
Income tax expense (benefit)	**$ 46**	**-0.1%**	**$ (524)**	**1.0%**

Deferred income tax expense (benefit) results primarily from the timing of temporary differences between the tax and financial statement carrying amounts of goodwill. The net deferred tax asset and liability in the accompanying consolidated balance sheets consists of the following components (in thousands):

	For the Years Ended December 31,	
	2019	2018
Deferred tax assets		
Net operating loss carryforward	$ 96,924	$ 123,562
Foreign tax credit carryforward	631	631
Capitalized research and development	92,265	73,893
Share-based compensation	22,846	21,319
Organization costs	24	27
Depreciation	1,039	838
Intangibles	24,647	27,769
163(j) interest limitations	—	1,252
Right of use lease liability	6,677	—
Other	226	1,335
Total deferred tax assets	245,279	250,626
Deferred tax liabilities		
Goodwill	(461)	(415)
Right of use lease asset	(5,531)	—
Unrealized gain on MeiraGTx equity securities investment	(7,604)	(2,393)
Change in fair value of financial instruments	(3,536)	(4,112)
Total deferred tax liabilities	(17,132)	(6,920)
Total deferred tax assets, net	228,147	243,706
Valuation allowance	(228,608)	(244,121)
Deferred tax liability	$ (461)	$ (415)

At December 31, 2019, the Company has unused federal and state net operating loss (“NOL”) carryforwards of $371.1 million and $307.2 million, respectively, that may be applied against future taxable income. These carryforwards expire at various dates through December 31, 2037, with the exception of approximately $79.9 million of federal net operating loss carryforwards, which will not expire. The 20-year limitation was eliminated for losses generated after January 1, 2018, giving the taxpayer the ability to carry forward losses indefinitely. However, NOL carry forward arising after January 1, 2018, will now be limited to 80 percent of taxable income.

The use of the Company’s NOL carryforwards may, however, be subject to limitations as a result of an ownership change. A corporation undergoes an “ownership change,” in general, if a greater than 50% change (by value) in its equity ownership by one or more five-percent stockholders (or certain groups of non-five-percent stockholders) over a three-year period occurs. After such an ownership change, the corporation’s use of its pre-change NOL carryforwards and other pre-change tax attributes to offset its post-change income is subject to an annual limitation determined by the equity value of the corporation on the date the ownership change occurs multiplied by a rate determined monthly by the Internal Revenue Service. The Company experienced ownership changes under Section 382 of the Code in 2010, 2011 and 2016, but the Company did not reduce the gross deferred tax assets related to the NOL carryforwards because the limitations do not hinder the Company’s ability to potentially utilize all of the NOL carryforwards. The Company also experienced an ownership change under Section 382 of the Code in 2019, as a result of equity offerings. This ownership change reduced the Company’s ability to potentially utilize all of the NOL carryforwards and, consequently, resulted in a reduction to the Company’s gross deferred tax assets and related valuation allowance of $125.2 million during November 2019.

If an additional ownership change occurred in the future and if the Company earned net taxable income, the Company’s ability to use its pre-change NOLs to offset U.S. federal taxable income would be subject to these limitations, which could potentially result in increased future tax liability compared to the tax liability the Company would incur if its use of NOL carryforwards were not so limited. In addition, for state income, franchise and similar tax purposes, there may be periods during which the use of NOL carryforwards is suspended or otherwise limited, which could accelerate or permanently increase the Company’s state income, franchise or similar taxes.

In accordance with ASC 740, “Income Taxes,” the Company recorded a valuation allowance to fully offset the gross deferred tax asset, because it is more likely than not that the Company will not realize future benefits associated with these deferred tax assets at December 31, 2019 and 2018. The change in deferred tax liability has been recognized as an income tax expense and benefit in the consolidated statements of operations for the years ended December 31, 2019 and 2018, respectively.

EXHIBIT INDEX

Exhibit Number	Description of Exhibit
3.1	Restated Certificate of Incorporation of Kadmon Holdings, Inc. (incorporated herein by reference to Exhibit 3.1 to the Registrant's Quarterly Report on Form 10-Q (File No. 001-37841), filed with the SEC on August 5, 2019).
3.2	Certificate of Designations of Kadmon Holdings, Inc. creating the 5% Convertible Preferred Stock (incorporated herein by reference to Exhibit 3.3 to the Registrant's Current Report on Form 8-K (File No. 001-37841), filed with the SEC on August 1, 2016).
3.3	Bylaws of Kadmon Holdings, Inc. (incorporated herein by reference to Exhibit 3.2 to the Registrant's Current Report on Form 8-K (File No. 001-37841), filed with the SEC on August 1, 2016).
4.1	Form of Kadmon Holdings, Inc.'s Common Stock Certificate (incorporated herein by reference to Exhibit 4.1 to the Registrant's Registration Statement on Form S-1/A (File No. 333-211949), filed with the SEC on July 14, 2016).
4.2*	Description of the Company's Common Stock
4.3	Form of Warrant Agreement dated September 28, 2017 (incorporated herein by reference to Exhibit 4.1 to the Registrant's Current Report on Form 8-K (File No. 001-37841), filed with the SEC on September 28, 2017).
4.4	Form of 2013 Warrant (incorporated by reference to Exhibit 10.46 to the Registrant's Registration Statement on Form S-1 (File No. 333-211949), filed with the SEC on June 10, 2016).
4.5	Form of 2013/2014 Warrant (incorporated by reference to Exhibit 10.47 to the Registrant's Registration Statement on Form S-1 (File No. 333-211949), filed with the SEC on June 10, 2016).
4.6	Form of 2015 Warrant (incorporated by reference to Exhibit 10.48 to the Registrant's Registration Statement on Form S-1 (File No. 333-211949), filed with the SEC on June 10, 2016).
10.1	Credit Agreement, dated August 28, 2015 between Kadmon Pharmaceuticals, LLC, the guarantors from time to time party thereto, the lenders from time to time party thereto and Perceptive Credit Opportunities Fund, L.P. (incorporated by reference to Exhibit 10.1 to the Registrant's Registration Statement on Form S-1 (File No. 333-211949), filed with the SEC on June 10, 2016).
10.2	Amendment to Credit Agreement, dated October 27, 2015, by and between Kadmon Pharmaceuticals, LLC, the guarantors from time to time party thereto, the lenders from time to time party thereto and Perceptive (incorporated by reference to Exhibit 10.2 to the Registrant's Registration Statement on Form S-1 (File No. 333-211949), filed with the SEC on June 10, 2016).
10.3	Amendment # 2 to Credit Agreement, dated November 4, 2016, by and among Kadmon Pharmaceuticals, LLC, the guarantors from time to time party thereto, the lenders from time to time party thereto and Perceptive Credit Holdings, L.P. (incorporated herein by reference to Exhibit 10.3 to the Registrant's Quarterly Report on Form 10-Q (File No. 001-37841), filed with the SEC on November 9, 2016).
10.4	Amendment #3 to Credit Agreement, dated March 31, 2017 by and among Kadmon Pharmaceuticals, LLC , the guarantors from time to time party thereto, the lenders from time to time party thereto and Perceptive Credit Holdings, L.P. (incorporated herein by reference to Exhibit 10.1 to the Registrant's Current Report on Form 8-K (File No. 001-37841), filed with the SEC on April 3, 2017).
10.5	Sub-license Agreement, dated April 8, 2011, by and among NT Life Sciences, LLC, Kadmon Pharmaceuticals, LLC and Surface Logix, Inc. (incorporated by reference to Exhibit 10.17 to the Registrant's Registration Statement on Form S-1 (File No. 333-211949), filed with the SEC on June 10, 2016).
10.6	Employment Agreement between Kadmon Corporation, LLC and Harlan W. Waksal, M.D., dated effective as of November 1, 2015 (incorporated by reference to Exhibit 10.33 to the Registrant's Registration Statement on Form S-1 (File No. 333-211949), filed with the SEC on June 10, 2016).
10.7	Employment Agreement between Kadmon Corporation, LLC and Steven N. Gordon, dated and effective as of July 1, 2015 (incorporated by reference to Exhibit 10.35 to the Registrant's Registration Statement on Form S-1 (File No. 333-211949), filed with the SEC on June 10, 2016).
10.8	Lease Agreement, dated October 28, 2010, by and between ARE-East River Science Park, LLC and Kadmon Pharmaceuticals, LLC (incorporated by reference to Exhibit 10.37 to the Registrant's Registration Statement on Form S-1 (File No. 333-211949), filed with the SEC on June 10, 2016).
10.9	First Amendment to Lease Agreement, dated July 1, 2011, by and between ARE-East River Science Park, LLC and Kadmon Pharmaceuticals, LLC (incorporated by reference to Exhibit 10.38 to the Registrant's Registration Statement on Form S-1 (File No. 333-211949), filed with the SEC on June 10, 2016).

10.10	Second Amendment to Lease Agreement, dated November 16, 2011, by and between ARE-East River Science Park, LLC and Kadmon Pharmaceuticals, LLC (incorporated by reference to Exhibit 10.39 to the Registrant's Registration Statement on Form S-1 (File No. 333-211949), filed with the SEC on June 10, 2016).
10.11	Third Amendment to Lease Agreement, dated January 4, 2013, by and between ARE-East River Science Park, LLC and Kadmon Pharmaceuticals, LLC (incorporated by reference to Exhibit 10.40 to the Registrant's Registration Statement on Form S-1 (File No. 333-211949), filed with the SEC on June 10, 2016).
10.12	Fourth Amendment to Lease Agreement, dated July 25, 2013, by and between ARE-East River Science Park, LLC and Kadmon Pharmaceuticals, LLC (incorporated by reference to Exhibit 10.41 to the Registrant's Registration Statement on Form S-1 (File No. 333-211949), filed with the SEC on June 10, 2016).
10.13	Fifth Amendment to Lease Agreement, dated August 11, 2017, by and between ARE-East River Science Park, LLC and Kadmon Corporation, LLC (incorporated by reference to Exhibit 10.1 to the Registrant's Current Report on Form 8-K (File No. 001-37841), filed with the SEC on August 14, 2017).
10.14	Sixth Amendment to Lease Agreement, dated August 11, 2017, by and between ARE-East River Science Park, LLC and Kadmon Corporation, LLC (incorporated by reference to Exhibit 10.2 to the Registrant's Current Report on Form 8-K (File No. 001-37841), filed with the SEC on August 14, 2017).
10.15	Kadmon Holdings, LLC 2014 Long-Term Incentive Plan, as amended (incorporated by reference to Exhibit 10.43 to the Registrant's Registration Statement on Form S-1 (File No. 333-211949), filed with the SEC on June 10, 2016).
10.16	Form of Indemnification to be entered into by Kadmon Holdings, Inc. and each of its directors, executive officers and certain key employees (incorporated by reference to Exhibit 10.55 to the Registrant's Registration Statement on Form S-1/A (File No. 333-211949), filed with the SEC on July 14, 2016).
10.17	Amended and Restated Kadmon Holdings, Inc. 2016 Equity Incentive Plan (incorporated herein by reference to Exhibit 10.57 to the Registrant's Annual Report on Form 10-K (File No. 001-37841), filed with the SEC on March 6, 2018).
10.18	Form of Stock Appreciation Right Agreement under Amended and Restated Kadmon Holdings, Inc. 2016 Equity Incentive Plan (incorporated herein by reference to Exhibit 10.58 to the Registrant's Annual Report on Form 10-K (File No. 001-37841), filed with the SEC on March 6, 2018).
10.19	Amended and Restated Kadmon Holdings, Inc. 2016 Employee Stock Purchase Plan (incorporated herein by reference to Exhibit 10.1 to the Registrant's Quarterly Report on Form 10-Q (File No. 001-37841), filed with the SEC on May 8, 2018).
10.20	Form of Subscription Agreement dated June 11, 2018 (incorporated herein by reference to Exhibit 10.1 to the Registrant's Current Report on Form 8-K (File No. 001-37841), filed with the SEC on June 14, 2018).
10.21	Amendment # 4 to Credit Agreement dated June 12, 2018, by and among Kadmon Pharmaceuticals, LLC, the guarantors from time to time party thereto, the lenders from time to time party thereto and Perceptive Credit Holdings, L.P. (incorporated herein by referend to Exhibit 10.1 to the Registrant's Current Report on Form 8-K (File No. 001-37841), filed with the SEC on June 13, 2018).
10.22	Amendment # 5 to Credit Agreement and Amendment to Warrant Certificate, dated August 15, 2018, by and among Kadmon Pharmaceuticals, LLC, the guarantors from time to time party thereto, and Perceptive Credit Holdings, L.P., as collateral representative and lender (incorporated herein by reference to Exhibit 10.1 to the Registrant's Current Report on Form 8-K (File No. 001-37841), filed with the SEC on August 16, 2018).
10.23	Controlled Equity Offering Sales Agreement, dated August 4, 2017, between the Registrant and Cantor Fitzgerald & Co. (incorporated herein by reference to Exhibit 1.2 to the Registrant's Registration Statement on Form S-3 (File No. 333-219712), filed with the SEC on August 4, 2017).
10.24	Form of Performance Stock Option Agreement under Amended and Restated Kadmon Holdings, Inc. 2016 Equity Incentive Plan (incorporated herein by reference to Exhibit 10.39 to the Registrant's Annual Report on Form 10-K (File No. 001-37841), filed with the SEC on March 7, 2019).
10.25	Separation and Release Agreement between Kadmon Corporation, LLC and Konstantin Poukalov, dated November 30, 2018 (incorporated herein by reference to Exhibit 10.40 to the Registrant's Annual Report on Form 10-K (File No. 001-37841), filed with the SEC on March 7, 2019).
10.26	Employment Agreement between Kadmon Corporation, LLC and Steven Meehan, dated effective as of February 8, 2019 (incorporated herein by reference to Exhibit 10.1 to the Registrant's Quarterly Report on Form 10-Q (File No. 001-37841), filed with the SEC on May 9, 2019).
10.27	Separation Agreement and General Release between Kadmon Corporation, LLC and Steven N. Gordon, effective as of August 30, 2019 (incorporated herein by reference to Exhibit 10.1 to the Registrant's Quarterly Report on Form 10-Q (File No. 001-37841), filed with the SEC on November 7, 2019).

10.28	Employment Agreement between Kadmon Corporation, LLC and Gregory S. Moss, effective as of August 30, 2019 (incorporated herein by reference to Exhibit 10.2 to the Registrant's Quarterly Report on Form 10-Q (File No. 001-37841), filed with the SEC on November 7, 2019).
10.29*	Employment Agreement between Kadmon Corporation, LLC and Harlan W. Waksal, M.D., dated effective as of January 1, 2020.
21.1*	List of subsidiaries.
23.1*	Consent of independent registered public accounting firm.
31.1*	Certification of Principal Executive Officer pursuant to Rules 13a-14(a) and 15d-14(a) promulgated under the Securities Exchange Act of 1934, as amended, as adopted pursuant to Section 302 of The Sarbanes-Oxley Act of 2002.
31.2*	Certification of Principal Financial Officer pursuant to Rules 13a-14(a) and 15d-14(a) promulgated under the Securities Exchange Act of 1934, as amended, as adopted pursuant to Section 302 of The Sarbanes-Oxley Act of 2002.
32.1**	Certification of Chief Executive Officer 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 pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
101*	The following materials from the Kadmon Holdings, Inc. Form 10-K for the year ended December 31, 2019, formatted in Extensible Business Reporting Language (XBRL): (i) Consolidated Balance Sheets at December 31, 2019 and 2018, (ii) Consolidated Statements of Operations for the years ended December 31, 2019 and 2018, (iii) Consolidated Statements of Stockholders' Equity for the years ended December 31, 2019 and 2018, (iv) Consolidated Statements of Cash Flows for the years ended December 31, 2019 and 2018, and (v) Notes to the Financial Statements.
*	Filed herewith.
**	Furnished herewith.

SIGNATURES

Pursuant to the requirements of Section 13 or 15(d) of the Securities Exchange Act of 1934, the registrant has duly caused this Annual Report on Form 10-K to be signed on its behalf by the undersigned, thereunto duly authorized.

Kadmon Holdings, Inc.

Date: March 5, 2020

By: /s/ Harlan W. Waksal
Harlan W. Waksal
President and Chief Executive Officer
(Principal Executive Officer)

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

Signature	Title	Date
/s/ Harlan W. Waksal Harlan W. Waksal	President and Chief Executive Officer (Principal Executive Officer)	March 5, 2020
/s/ Steven Meehan Steven Meehan	Executive Vice President, Chief Financial Officer (Principal Financial Officer)	March 5, 2020
/s/ Kyle Carver Kyle Carver	Principal Accounting Officer, Controller (Principal Accounting Officer)	March 5, 2020
/s/ Tasos G. Konidaris Tasos G. Konidaris	Director	March 5, 2020
/s/ Eugene Bauer Eugene Bauer	Director	March 5, 2020
/s/ D. Dixon Boardman D. Dixon Boardman	Director	March 5, 2020
/s/ David E. Cohen David E. Cohen	Director	March 5, 2020
/s/ Arthur Kirsch Arthur Kirsch	Director	March 5, 2020
/s/ Cynthia Schwalm Cynthia Schwalm	Director	March 5, 2020

DESCRIPTION OF CAPITAL STOCK

The following summary describes our capital stock and certain provisions of our restated certificate of incorporation, our bylaws, the registration rights agreements to which we and certain of our stockholders are parties and the General Corporation Law of the State of Delaware. Because the following is only a summary, it does not contain all of the information that may be important to you. For a complete description, you should refer to our restated certificate of incorporation, bylaws, and registration rights agreements, copies of which are filed with this Annual Report on Form 10-K.

General

We are a Delaware corporation. We completed transactions on July 26, 2016 pursuant to which we converted into a Delaware corporation and changed our name from Kadmon Holdings, LLC to Kadmon Holdings, Inc. Our authorized share capital consists of 400,000,000 shares of common stock, par value $0.001 per share, and 10,000,000 preferred shares, par value $0.001 per share.

The following descriptions are summaries of important terms contained in our restated certificate of incorporation and our bylaws (our "Certificate of Incorporation" and "Bylaws," respectively). Reference is made to the more detailed provisions of, and the descriptions are qualified in their entirety by reference to, our Certificate of Incorporation and Bylaws, copies of which are filed with the United States Securities and Exchange Commission ("SEC") as exhibits to this Annual Report on Form 10-K, and to relevant portions of the General Corporation Law of the State of Delaware ("DGCL").

Common Stock

General. As of December 31, 2019, there were 159,759,996 shares of common stock issued and outstanding. All outstanding shares of common stock are validly issued, fully paid and nonassessable.

Voting Rights. The holders of our common stock are entitled to one vote for each share held of record on all matters submitted to a vote of the stockholders, including the election of directors, and do not have cumulative voting rights. Unless otherwise required by law, matters submitted to a vote of our stockholders require the approval of a majority of votes cast by stockholders represented in person or by proxy and entitled to vote on such matter, except that directors will be elected by a plurality of votes cast. Accordingly, the holders of a majority of the shares of common stock entitled to vote in any election of directors are able to elect all of the directors standing for election, if they so choose.

Dividend Rights. Holders of shares of common stock are entitled to receive ratably dividends if, as and when dividends are declared from time to time by our board of directors out of funds legally available for that purpose, subject to any preferential dividend rights of any then outstanding preferred stock.

Liquidation. Upon our liquidation, dissolution or winding up, the holders of shares of common stock are entitled to share ratably in the net assets legally available for distribution to stockholders after the payment of all of our debts and other liabilities, subject to any liquidation preference granted to holders of any outstanding preferred stock.

Rights and Preferences. Holders of shares of common stock have no preemptive or conversion rights or other subscription rights, and no redemption or sinking fund provisions are applicable to our common stock. The rights, preferences and privileges of the holders of our common stock are subject to and may be adversely affected by the rights of the holders of shares of any series of our preferred stock that we may designate in the future.

Fully Paid and Nonassessable. All outstanding shares of common stock are, and the shares of common stock to be issued in this offering will be, fully paid and nonassessable.

Registration Rights. In connection with a private placement of our securities in March 2017, we entered into a Registration Rights Agreement dated March 8, 2017 with certain investors. Pursuant to the terms of the registration rights agreement, we were obligated to prepare, file and maintain with the SEC a registration statement to register

for resale certain shares purchased by investors in the private placement following the closing of the private placement, among other customary obligations for agreements of this type.

Listing. Our common stock is listed under the symbol "KDMN" on the New York Stock Exchange.

Transfer Agent and Registrar. The transfer agent and registrar for our common stock is American Stock Transfer & Trust Company, LLC.

Preferred Stock

As of December 31, 2019, no shares of our preferred stock were outstanding other than shares of our 5% convertible preferred stock, as described below under "—5% Convertible Preferred Stock." Our Certificate of Incorporation authorizes our board of directors, without further action by our stockholders, to issue up to 10,000,000 shares of preferred stock in one or more series and to fix the rights, preferences, privileges and restrictions thereof. These rights, preferences and privileges could include dividend rights, conversion rights, voting rights, redemption rights, terms of sinking funds, liquidation preferences, the number of shares constituting any class or series and the designation of the class or series. Terms selected by our board of directors in the future could decrease the amount of earnings and assets available for distribution to holders of shares of common stock or adversely affect the rights and powers, including voting rights, of the holders of shares of common stock without any further vote or action by the stockholders. As a result, the rights of holders of our common stock will be subject to, and may be adversely affected by, the rights of the holders of the 5% convertible preferred stock and any other preferred stock that may be issued by us in the future, which could have the effect of decreasing the market price of our common stock. The issuance of preferred stock could, among other things, have the effect of delaying, deferring or preventing a change in control of our company, which could depress the market price of our common stock.

The terms of any particular series of preferred stock will be described in the prospectus supplement relating to the offering of shares of such series of preferred stock. If we issue shares of preferred stock under this prospectus and any related prospectus supplement, the shares will be fully paid and nonassessable and will not have, or be subject to, any preemptive or similar rights.

5% Convertible Preferred Stock

As of December 31, 2019, we had 28,708 shares of convertible preferred stock issued and outstanding, designated as the 5% convertible preferred stock pursuant to the certificate of designations filed by us with the Secretary of State of the State of Delaware, with an aggregate original purchase price and initial liquidation preference of $30.0 million. As of December 31, 2019, the stated liquidation preference of the 5% convertible preferred stock was $33.1 million. Each share of convertible preferred stock was issued for an amount equal to $1,000 per share, which we refer to as the original purchase price.

The following description is a summary of the material provisions of the 5% convertible preferred stock and the certificate of designations and does not purport to be complete. This summary is subject to and is qualified by reference to all the provisions of the convertible preferred stock and certificate of designations, including the definitions of certain terms used in the certificate of designations. We urge you to read this document because it, and not this description, defines the rights of a holder of the 5% convertible preferred stock. A copy of the form of certificate of designations that we filed with the Secretary of State of the State of Delaware on July 26, 2016 has been incorporated by reference as an exhibit to this Annual Report on Form 10-K.

No Mandatory Redemption Date or Sinking Fund

The shares of 5% convertible preferred stock do not have a mandatory redemption date and are not subject to any sinking fund. The shares of convertible preferred stock will remain outstanding indefinitely unless we are required to redeem them under the circumstances described below in "—Redemption" or we otherwise repurchase them or they are converted into shares of our common stock as described below under "—Conversion Rights."

Dividends

The shares of 5% convertible preferred stock are entitled to receive dividends, when and as declared by our board of directors and to the extent of funds legally available for the payment of dividends, at an annual rate of 5% of the sum of the original purchase price per share of 5% convertible preferred stock plus any dividend arrearages. Dividends on the convertible preferred stock shall, at our option, either be paid in cash or added to the stated liquidation preference amount for purposes of calculating dividends at the 5% annual rate (until such time as we declare and pay the missed dividend in full and in cash, at which time that dividend will no longer be part of the stated liquidation preference amount). Dividends shall be payable annually on June 30 of each year and shall be cumulative from the most recent dividend payment date on which dividend has been paid or, if no dividend has ever been paid, from the original date of issuance of the 5% convertible preferred stock and shall accumulate from day to day whether or not declared until paid.

The shares of 5% convertible preferred stock are also entitled to participate in all dividends declared and paid on shares of company common stock on an “as if” converted basis.

Liquidation Preference

In the event of:

(A) a liquidation, dissolution or winding up of our company, whether voluntary or involuntary;

(B) certain changes of control;

(C) a sale or transfer of all, or substantially all, of our consolidated assets other than to a wholly-owned subsidiary of ours;

(D) any other event of discharge, retirement or cancellation of the 5% convertible preferred stock, in each case in this clause (D), that is not described in the foregoing clauses (A), (B) or (C) or a redemption pursuant to the certificate of designations;

(E) our company or one of our significant subsidiaries becoming the subject of certain bankruptcy events;

(F) a material breach of our obligations under the exchange agreement that is not cured within 15 days after we receive notice from a holder of the 5% convertible preferred stock; or

(G) upon our failure to make any payment of principal, interest, or other amount due and payable of any of our or our subsidiaries’ indebtedness after giving effect to any applicable cure period,

the holders of the 5% convertible preferred stock shall be entitled to receive for each share of 5% convertible preferred stock an amount equal to the greater of (i) (A) (I) the original purchase price per share of 5% convertible preferred stock plus dividend arrearages thereon in cash *plus* (II) any dividends accrued and unpaid thereon from the last dividend payment date to the date of the final distribution to such holder *plus* (B) solely in connection with an event specified in clauses (A), (D), (E), (F) or (G) above, a premium equal to 20.2% of the amount described in clause (i)(A) of this sentence at such time or (ii) an amount per share of 5% convertible preferred stock equal to the amount which would have been payable or distributable had each share of 5% convertible preferred stock been converted into shares of our common stock immediately before the event occurred under clause (A), (B), (C) or (D) above.

Subject to the rights of the holders of any parity shares, upon any of the events specified in clauses (A) through (D) above, after payment shall have been made in full to the holders of the convertible preferred stock and any parity securities, any other series or class or classes of junior securities shall be entitled to receive any and all assets remaining to be paid or distributed, and the holders of the convertible preferred stock and any parity securities as such shall not be entitled to share in that payment or distribution.

In the event that the event giving rise to the determination of the amount that holders of 5% convertible preferred stock shall be entitled to receive as their liquidation preference is a failure by us to make any payment of principal, interest, or other amount due and payable of any of our or our subsidiaries’ indebtedness after giving effect to any applicable cure period, that event shall be deemed never to have occurred if, subsequent to the expiration of the cure period, (i) that failure to make payment is cured in full, (ii) all other obligations to pay

principal, interest or other amounts due and payable of any of our or our subsidiaries' indebtedness have been paid at that time, and (iii) no bankruptcy event has occurred.

Ranking

The 5% convertible preferred stock ranks, with respect to rights to the payment of dividends and the distribution of assets in the event of any of the events specified in clauses (A) through (D) under "—Liquidation Preference" above,

(1) senior to all common stock and to all other equity securities of our company other than equity securities referred to in clauses (2) and (3) of this sentence ("junior securities");

(2) to the extent authorized under the certificate of designations, on a parity with all equity securities of our company the terms of which specifically provide that such equity securities rank on a parity with the 5% convertible preferred stock ("parity securities"); and

(3) to the extent authorized under the certificate of designations, junior to all equity securities of our company the terms of which specifically provide that such equity securities rank senior to the 5% convertible preferred stock ("senior securities").

See "Voting Rights—Matters Requiring Approval of Holders of 5% Convertible Preferred Stock" for a description of the types of issuances of equity securities and other securities of our company requiring approval of holders of a majority of shares of 5% convertible preferred stock then outstanding, voting together as a class.

Redemption

If:

(A) we or one of our significant subsidiaries becomes the subject of certain bankruptcy events;

(B) a material breach of our obligations under the exchange agreement occurs that is not cured within 15 days after we receive notice from a holder of the 5% convertible preferred stock; or

(C) we fail to make any payment of principal, interest, or other amount due and payable of any of our or our subsidiaries' indebtedness after giving effect to any applicable cure period,

each holder of 5% convertible preferred stock shall have the right to cause us to redeem all or part of the shares of 5% convertible preferred stock held by such holder for a redemption price per share equal to (i) the original purchase price *plus* any dividend arrearages *plus* any dividends accrued and unpaid thereon from the last dividend payment date to, but excluding, the redemption date *plus* (ii) a premium equal to 20.2% of the amount described in clause (i) of this sentence at such time.

We are required to mail notice of any redemption event to the holders of 5% convertible preferred stock not later than one business day after we acquire knowledge of that event. That notice must state, among other things, (1) the redemption price and the date of redemption, which shall be no sooner than 30 days and no later than 90 days from the date the notice is mailed and (2) any holder of 5% convertible preferred stock electing to have its shares redeemed shall be required to surrender its shares, with a properly completed redemption request, to us before the close of business on the fifth business day before the redemption date. If we fail to give notice of the redemption event within the time period specified above, then any holder of 5% convertible preferred stock may deliver that notice to us and the other holders, in which case the redemption date shall occur on the 45th day after the date of the notice and any holder electing to have any of its shares of 5% convertible preferred stock redeemed shall be required to surrender its shares, with a properly completed redemption request, to us before the close of business on the fifth business day preceding that redemption date.

Until the holders of the 5% convertible preferred stock who have delivered a notice to us requesting redemption have been paid the redemption price specified in the previous paragraph in full, no payment will be made to any holder of parity securities or junior securities.

Notwithstanding anything to the contrary, in the event that the event giving rise to the above redemption right is a failure by us to make any payment of principal, interest or other amount due and payable of any of our indebtedness after giving effect to any applicable cure period, that event shall be deemed never to have occurred and any request for redemption delivered by a holder of 5% convertible preferred stock in respect of that event shall be deemed automatically rescinded if, subsequent to the expiration of the cure period, (i) our failure to make payment is cured in full, (ii) all other obligations to pay principal, interest or other amounts due and payable of any of our or our subsidiaries' indebtedness have been paid at such time and (iii) no bankruptcy event has occurred.

Conversion Rights

Conversion at the Option of the Holder. The holders of shares of 5% convertible preferred stock will, at any time, be entitled to convert some or all of their 5% convertible preferred stock into the number of shares of our common stock obtained by dividing the aggregate original purchase price of the shares to be converted *plus* any dividend arrearages *plus* any dividends accrued and unpaid from the last dividend payment date to but excluding the conversion date by an amount equal to 80% of the initial public offering price per share in our initial public offering, which amount we refer to as the conversion price. The conversion price will be adjustable upon the occurrence of certain events and transactions to prevent dilution as described under "—Adjustments to Conversion Price to Prevent Dilution." Any shares of our common stock issued upon conversion of the shares of 5% convertible preferred stock shall be validly issued, fully paid and nonassessable. Cash shall be paid in lieu of fractional shares.

Conversion at our Option. At any time following the first anniversary of the issuance of the 5% convertible preferred stock, provided that (A) the volume-weighted average price of our common stock for the 30 consecutive trading days immediately preceding the date we elect for conversion is in excess of 150% of the initial public offering price per share in this offering (as adjusted for the events described below under "—Adjustments to Conversion Price to Prevent Dilution" and dividends paid in shares of our common stock) and (B) we have in place an effective resale shelf registration statement permitting the resale of all of the shares of common stock issuable upon conversion of the 5% convertible preferred stock, we have the right to require the conversion of any number of shares of 5% convertible preferred stock then outstanding into the number of shares of our common stock obtained by dividing the aggregate original purchase price of the shares to be converted *plus* any dividend arrearages *plus* any dividends accrued and unpaid from the last dividend payment date to but excluding the conversion date by the then applicable conversion price.

Adjustments to Conversion Price to Prevent Dilution

The 5% convertible preferred stock is subject to provisions that protect the holders against dilution by adjustment of the conversion price and/or number of shares of common stock issuable upon conversion in certain events such as a subdivision, combination or reclassification of our outstanding common stock.

Voting Rights—Matters Requiring Approval of Holders of 5% Convertible Preferred Stock

Holders of the 5% convertible preferred stock shall be entitled to vote on any and all matters on which holders of the company common stock are entitled to vote on an "as if" converted basis. Additionally, so long as any 5% convertible preferred stock remains outstanding, without the affirmative approval of the holders of at least a majority of the shares of 5% convertible preferred stock then outstanding, we shall not, directly or indirectly (including through merger or consolidation with any other corporation), and shall not permit any of our subsidiaries to:

(1) authorize or approve the issuance of any senior securities, 5% convertible preferred stock, or parity securities (or, in each case, any security convertible into, or convertible or exchangeable therefor or linked thereto) or authorize or create or increase the authorized amount of any senior securities, 5% convertible preferred stock or parity securities (or, in each case, any security convertible into, or convertible or exchangeable therefor or linked thereto);

(2) authorize or approve the purchase or redemption of any parity securities or junior securities;

(3) amend, alter or repeal any of the provisions of the certificate of designations, our Certificate of Incorporation or our Bylaws in a manner that would adversely affect the powers, designations, preferences and rights of the 5% convertible preferred stock;

(4) contract, create, incur, assume or suffer to exist any indebtedness or guarantee any such indebtedness with an aggregate value of more than $5,000,000 (subject to certain exceptions); or

(5) agree to take any of the foregoing actions.

The certificate of designations governing the 5% convertible preferred stock also provides that no amendment or waiver of any provision of the certificate of designations or our Certificate of Incorporation or Bylaws shall, without the prior written consent of all holders of the 5% convertible preferred stock who are known to us to hold, together with their affiliates, more than 5% of the 5% convertible preferred stock then outstanding, (i) reduce any amounts payable or that may become payable to holders of the 5% convertible preferred stock, (ii) postpone the payment date of any amount payable to holders of the 5% convertible preferred stock or waive or excuse any payment, (iii) modify or waive the conversion rights of the 5% convertible preferred stock in a manner that would adversely affect any holder of the 5% convertible preferred stock, or (iv) change any of the voting-related provisions or any other provision of the certificate of designations specifying the number or percentage of holders of the 5% convertible preferred stock which are required to waive, amend or modify any rights under the certificate of designations or make any determination or grant any consent under that document.

Registration Rights

The holders of the 5% convertible preferred stock were granted registration rights, subject to customary cutbacks, blackout periods and other exceptions, for all shares of our common stock issued or issuable upon conversion of the 5% convertible preferred stock, including (a) two demand registrations at any time after the expiration of 180 days from the closing of our initial public offering, (b) unlimited piggyback rights and (c) the right to require filing of a resale S-3 registration statement (once we became eligible to file on such form) and maintenance of its effectiveness on an "evergreen" basis until such time as there are no longer any registrable securities.

Anti-Takeover Effects of Provisions of Our Certificate of Incorporation and Bylaws and Delaware Law

The provisions of the DGCL and our Certificate of Incorporation and Bylaws could have the effect of discouraging others from attempting an unsolicited offer to acquire our company. Such provisions may also have the effect of preventing changes in our management. It is possible that these provisions could make it more difficult to accomplish transactions that stockholders may otherwise deem to be in their best interests.

Election and Removal of Directors. Our directors are elected until the expiration of the term for which they are elected and until their respective successors are elected. Our directors may be removed only by the affirmative vote of at least a majority of the holders of our then outstanding common stock. This system of electing and removing directors generally makes it more difficult for stockholders to replace a majority of our directors. For more information on our board of directors, see the section entitled "Corporate Governance" in our Definitive Proxy Statement dated as of April 2, 2019.

Authorized but Unissued Shares. The authorized but unissued shares of our common stock and our preferred stock are available for future issuance without any further vote or action by our stockholders. These additional shares may be utilized for a variety of corporate purposes, including future public offerings to raise additional capital, corporate acquisitions and employee benefit plans. The existence of authorized but unissued shares of our common stock and our preferred stock could render more difficult or discourage an attempt to obtain control over us by means of a proxy contest, changes in our management, tender offer, merger or otherwise.

Stockholder Action; Advance Notification of Stockholder Nominations and Proposals. Our Certificate of Incorporation and Bylaws require that any action required or permitted to be taken by our stockholders must be effected at a duly called annual or special meeting of stockholders and may not be effected by a consent in writing. Our Certificate of Incorporation and Bylaws also require that special meetings of stockholders be called only by our board of directors, the Chairman of our board of directors or our Chief Executive Officer. In addition, our Bylaws provide that candidates for director may be nominated and other business brought before an annual meeting only by the board of directors or by a stockholder who gives written notice to us no later than 90 days prior to nor earlier than 120 days prior to the first anniversary of the last annual meeting of stockholders. These provisions may have the effect of deterring unsolicited offers to acquire our company or delaying changes in our management, which could depress the market price of our common stock.

Delaware Anti-Takeover Law. Our Certificate of Incorporation provides that Section 203 of the DGCL, an anti-takeover law, applies to us. In general, Section 203 prohibits a publicly held Delaware corporation from engaging in a "business combination" with an "interested stockholder" for a period of three years following the date the person became an interested stockholder, unless the "business combination" or the transaction in which the person became an interested stockholder is approved in a prescribed manner. Generally, a "business combination" includes a merger, asset or stock sale, or other transaction resulting in a financial benefit to the interested stockholder. Generally, an "interested stockholder" is a person who, together with affiliates and associates, owns or, within three years prior to the determination of interested stockholder status, did own, 15% or more of a corporation's voting stock.

Limitation of Liability and Indemnification

Our Certificate of Incorporation provides that no director will be personally liable for monetary damages for breach of any fiduciary duty as a director, except with respect to liability:

- for any breach of the director's duty of loyalty to us or our stockholders;
- for acts or omissions not in good faith or that involve intentional misconduct or a knowing violation of law;
- under Section 174 of the DGCL (governing distributions to stockholders); or
- for any transaction from which the director derived any improper personal benefit.

If the DGCL is amended to authorize corporate action further eliminating or limiting the personal liability of directors, then the liability of our directors will be eliminated or limited to the fullest extent permitted by the DGCL, as so amended. The modification or repeal of this provision of our Certificate of Incorporation will not adversely affect any right or protection of a director existing at the time of such modification or repeal.

Our Bylaws also provide that we will, to the fullest extent permitted by law, indemnify our directors and officers against all liabilities and expenses in any suit or proceeding or arising out of their status as an officer or director or their activities in these capacities. We also indemnify any person who, at our request, is or was serving as a director, officer, employee, agent or trustee of another corporation or of a partnership, limited liability company, joint venture, trust or other enterprise. We may, by action of our board of directors, provide indemnification to our employees and agents within the same scope and effect as the foregoing indemnification of directors and officers.

Exclusive Forum

Our Certificate of Incorporation provides that the Court of Chancery of the State of Delaware shall, to the fullest extent permitted by law, be the sole and exclusive forum for any (i) derivative action or proceeding brought on behalf of our company, (ii) action asserting a claim of breach of a fiduciary duty owed by any director or officer of our company to our company or our company's stockholders, (iii) action asserting a claim against our company arising pursuant to any provision of the DGCL or our Certificate of Incorporation or our Bylaws or (iv) action asserting a claim against our company governed by the internal affairs doctrine. This exclusive forum provision would not apply to suits brought to enforce any liability or duty created by the Exchange Act or any other claim for which the federal courts have exclusive jurisdiction. Section 27 of the Exchange Act creates exclusive federal jurisdiction over all suits brought to enforce any duty or liability created by the Exchange Act or the rules and regulations thereunder. In addition, Section 22 of the Securities Act 1933, as amended (the "Securities Act"), creates concurrent jurisdiction for federal and state courts over all suits brought to enforce any duty or liability created by the Securities Act or the rules and regulations thereunder. As a result, there is uncertainty as to whether a court would enforce our forum selection clause in connection with claims arising under the Securities Act or the rules and regulations thereunder.

Any person or entity purchasing or otherwise acquiring any interest in shares of capital stock of our company shall be deemed to have notice of and consented to the forum provisions in our Certificate of Incorporation. However, the enforceability of similar forum provisions in other companies' certificates of incorporation has been challenged in legal proceedings, and it is possible that a court could find these types of provisions to be unenforceable.

EMPLOYMENT AGREEMENT

This **EMPLOYMENT AGREEMENT,** dated as of November 19, 2019 (the "Agreement"), is entered into between **Kadmon Corporation, LLC**, a Delaware limited liability company (the "Company"), and **Harlan W. Waksal, M.D.**, an individual with a place of domicile of [ADDRESS REDACTED] (the "Employee"). Each of Company and Employee a "Party" and collectively, the "Parties".

WHEREAS, the Parties have previously entered into that certain Employment Agreement dated as of November 1, 2015 (as amended, the "Previous Agreement")); and

WHEREAS, the Parties now wish to supersede in its entirety the Previous Agreement, effective as of January 1, 2020 (the "Effective Date"), by entering in this Agreement.

NOW, THEREFORE, in consideration of the Employee's employment by the Company, and for other good and valuable consideration set forth herein, the receipt and sufficiency of which are hereby acknowledged, the Parties hereby agree as follows:

1. **Employment**. The Employee shall be employed as President and Chief Executive Officer, and shall have the duties, responsibilities and authority as may from time to time be assigned to him by the Board of Directors (the "Board") of Kadmon Holdings, Inc., the Company's parent company, that are consistent with such positions in a company of the size and nature of the Company. The Employee will report to the Board and shall, separate and aside from his roles as President and Chief Executive Officer, serve as a member of the Board, which appointment has been previously voted upon and approved in accordance with the Company's organization documents. The Employee agrees while he is employed by the Company to devote his full business time and attention to the activities of the Company and to not engage in other employment without the prior written consent of the Board. The Employee agrees to perform his duties hereunder diligently and to use his best efforts, skill and ability to promote the interests of the Company and its affiliates.

2. **Term**. The term of the Employee's employment under this Agreement shall be effective as of the date hereof and shall continue until terminated by either party in accordance with Section 5 hereof (the "Term"). Upon termination for any reason, the Parties agree that the provisions of Sections 4 and 5 shall survive, and Employee's service on the Company's Board shall survive subject to and in accordance with the Company's organizational documents.

3. **Compensation and Benefits**
    a) **Base Salary**. Beginning as of the Effective Date, the Company shall pay the Employee a base salary at the rate of $600,000.00 per year (the "Base Salary"). All salary shall be paid in accordance with the Company's regular payroll schedule and subject to required withholdings.
    b) **Discretionary Bonus**. Employee will be eligible for a year-end target bonus of 60% of the Base Salary ("Target Bonus"), based on Company performance and Employee performance. The evaluation of both Company performance and Employee performance, and the amount of any bonus paid hereunder (the "Discretionary Bonus"), will be at the discretion of the Board's Compensation Committee, and no guarantees relating to such cash bonus are being made by the Company. Employee must be employed on the date the bonus is paid in order to receive any Discretionary Bonus described hereunder.
    c) **Incentive Compensation**. The Employee will be entitled to participate in the Company's annual, year-end incentive compensation plans, subject to the terms of such plans. The decision as to the amounts of any incentive compensation, including grants of equity, to be awarded shall be made by the Company, but in any event shall be consistent in type and amount as are given to other members of executive management generally.
    d) **Benefits**. The Employee will be entitled to coverage under or participation in all benefit plans provided to members of executive management of the Company. The Company may, in its sole discretion, at any time amend or terminate its benefit plans. The Employee shall be entitled to no

fewer than four weeks of paid vacation per calendar year, to be used in accordance with the Company's then-current vacation policies.

e) **Reimbursements**. In connection with your employment, the Company agrees to reimburse you for business-related expenses incurred in the ordinary course of business, and separately to reimburse you for travel expenses incurred during the Term for travel between the Company's office(s) and Employee's residence in Florida. All reimbursements under this paragraph shall be subject to the Company's travel and expense policy in effect at the time the expense is incurred. Employee shall be responsible for any income tax or other tax due in connection with any taxable reimbursement provided under this paragraph.

4. **Covenants**

a) **Return of Documents**. Immediately upon the Company's request or promptly upon the end of the Employee's employment, for whatever reason, the Employee shall deliver to the Company any property of the Company or any of its affiliates (including, but not limited to, documents prepared or made by the Employee) which may be in the Employee's possession, including, but not limited to, materials, memoranda, notes, records, reports, designs, sketches, plans, programs, printouts, or other documents as well as all copies thereof and files related thereto.

b) **Confidentiality**. The Employee agrees to hold all Proprietary Information (as defined below) in strict confidence during the term of and following the Employee's employment under this Agreement. "Proprietary Information" includes, by way of example but without limitation, the following information relating to the Company or any of its affiliates or any customer, client or business partner of the Company or any of its affiliates:

i. working methods and operations, methodologies, marketing plans and strategies (including internal and external growth strategies), sales and financial reports, customer lists, trade secrets, copyrightable materials, patentable materials, programs, processes, plans, product ideas, techniques, designs, models, formulas, data, know-how and other information used in research, developmental, marketing, sales, and operational activities; and

ii. any commercial or technical information, improvements, or things which may be communicated to the Employee or which the Employee may learn by virtue of his employment by the Company, or of which the Employee may have gained knowledge, or discovered, invented, or perfected while employed by the Company, including without limitation any ideas or processes relating to the development, operation, or improvement of any software or other program, product or proposed product, tool, article, or process sold, licensed, distributed, maintained or contemplated by the Company or any of its affiliates (or their respective customers).

Notwithstanding the foregoing, Proprietary Information shall not include information that (a) is publicly known as of the date of this Agreement or (b) becomes publicly known after the date of this Agreement other than by means in violation of this Agreement or another obligation of confidentiality.

The Employee agrees to never, directly or indirectly, disclose or otherwise communicate to any person, firm, corporation, or other entity or to use for himself (except while the Employee is employed by the Company, and solely in pursuit of his activities as an employee of the Company), any Proprietary Information.

c) **Developments**. The Employee agrees to disclose promptly to the Company any and all Developments (as defined below) which are made, invented, developed or discovered by the Employee, either singly or jointly with others, in the course of his employment by the Company, including upon termination of such employment. The Employee also agrees that such Developments are works made for hire and are or shall become the exclusive property of the Company, and that he hereby relinquishes and assigns any and all intellectual property rights and or other rights in the Developments to the Company, including, by way of example, but without limitation, rights of identification or authorship and rights of approval with respect to modifications and limitations on subsequent modifications. In order to effectuate ownership by the Company when necessary, the Employee agrees, without further consideration:

i. to immediately upon the Company's request execute all documents and make all assignments necessary to vest title to such Developments in the Company;
ii. to assist the Company in any reasonable manner to obtain for the benefit of the Company any patents or copyright applications on such Developments, in any and all countries; and
iii. to execute when requested any and all patent and copyright applications and any other lawful documents deemed necessary by the Company to carry out the purposes of this Agreement.

"Developments" include, by way of example but without limitation, the following: any and all inventions, improvements, discoveries, developments, results of research, or useful ideas, whether or not patentable, which relate in any manner to any products, work, or other business or proposed business of the Company or one of its affiliates or any customer, client or business partner of the Company or one of its affiliates, or to any process, apparatus, formulas, equipment, or article worked on in connection with the Employee's employment by the Company.

5. **Termination**

a) **Death or Disability**. The Employee's employment hereunder shall terminate immediately upon his death or upon 30 days written notice by the Company to the Employee that the Employee's employment has been terminated due to the Employee's Disability. For the purposes of this Agreement, "Disability" shall mean upon the earlier of: (i) the date Employee becomes entitled to receive disability benefits under the Company's long-term disability plan; or (ii) the determination by the Board that the Employee is physically or mentally incapacitated or impaired and has been unable, for a period of at least 90 consecutive days, to perform the duties and responsibilities contemplated under this Agreement, even with a reasonable accommodation.

b) **Termination for Cause**. Employment with the Company may be terminated by the Board immediately for Cause. In this context the term "Cause" shall mean: (i) the Employee's conviction of a felony; (ii) any material misconduct by the Employee with respect to the Company, any affiliate of the Company, or any of their respective employees, customers, clients, business partners or suppliers; (iii) in carrying out his duties and responsibilities set forth herein, refusal, neglect or failure by the Employee to carry out, in all material respects, the legal instructions of the Board; (iv) a material breach by the Employee of any term or provision of Section 4 of this Agreement; or (v) the Employee's failure to comply in all material with the internal policies or procedures of the Company or its affiliates, or any laws or regulations applicable to Employee's conduct as an employee of the Company; which in each case of clauses (ii) to (v) above, remains uncured by the Employee for 5 days following receipt by the Employee of written notice of same, which notice shall include reasonable detail as to the nature of the potential resulting Cause. However, no notice and opportunity to cure shall be required in the event of conduct by the Employee that the Company reasonably believes cannot be adequately cured.

c) **Termination Without Cause**. Employment may be terminated by the Board without Cause, at any time, without prior notice.

d) **Resignation by Employee for Good Reason**. Employee may resign from his employment hereunder at any time if Employee has Good Reason. For purposes of this Agreement, the term "Good Reason" shall mean: (i) any material diminution in Employee's duties or responsibilities hereunder (other than in connection with a termination of Employee's employment), which remains uncured by the Company for 5 days following receipt by the Company of written notice of same, which notice shall include reasonable detail as to the nature of the potential resulting Good Reason; (ii) a material diminution in Employee's Base Salary; or (iii) a relocation of the Company's principal place of business outside New York City.

e) **Resignation by Employee Without Good Reason**. Employee may resign from his employment hereunder without Good Reason at any time upon written notice to the Company. Following any such notice, the Company may reduce or remove any and all of Employee's duties, authority or responsibilities with the Company, and any such reduction or removal shall not constitute Good Reason.

f) **Effect of Termination**. In the event that the Employee's employment hereunder is terminated for Cause, or Employee resigns without Good Reason, the Company shall pay the Employee his Base Salary through the date of such termination and any unreimbursed business expense (in accordance

with Company policy). In the event that the Employee's employment hereunder is terminated without Cause, or Employee resigns with Good Reason, and provided that Employee first signs and does not revoke any portion of a comprehensive release of claims against the Company, and its current and former affiliated entities and individuals, in a customary form drafted by the Company, the Company shall pay the Employee severance in an amount equal to his Base Salary and an amount equal to the greater of his Target Bonus or the previous year's Discretionary Bonus (collectively, the "Severance"). This Severance will be combined together and paid in in equal installments, and in accordance with the Company's regular payroll schedule, and subject to required withholdings, over the one-year period following the expiration of a seven-day revocation period set forth in the comprehensive release of claims, provided, however, that in the event Employee becomes employed by another entity or individual (and not self-employed) during that one-year period, he will so notify the Company, and such employment will end the Company's obligation to make any further severance payments.

g) **Benefits**. Subject to Employee's timely election of continuation coverage under COBRA, the Company will continue payment of Employee's medical, dental and vision insurance coverage during the twelve (12) month period following the first day of the month following the date of termination or resignation (the "Coverage Period") to the same extent that the Company paid for such coverage immediately prior to the date of termination or resignation, in a manner intended to avoid any excise tax under Section 4980D of the Internal Revenue Code of 1986, as amended (the "Code"), subject to the eligibility requirements and other terms and conditions of such insurance coverage, provided that Employee first signs and does not revoke any portion of a comprehensive release of claims against the Company, and its current and former affiliated entities and individuals, in a form drafted by the Company, and provided further that in the event Employee becomes employed by another entity or individual (and not self-employed) during that one-year period, he will so notify the Company, and such employment will end the Company's obligation to continued payments for medical, dental, and vision insurance coverage. If Employee fails to sign or revokes any portion of a comprehensive release of claims against the Company, the Employee's accrual of or participation in plans providing for medical, dental and vision insurance benefits will cease at the end of the Term, unless Employee properly and timely elects to continue medical, dental and vision insurance coverage in accordance with the continuation requirements of COBRA and pays the applicable premiums for such coverage. The Employee will not receive, as part of his termination pay pursuant to this Section 5, any payment or other compensation for any sick leave or other leave unused on the date the notice of termination or resignation is given, (or on the date the termination or resignation is otherwise effective in the event no notice is required), under this Agreement.

6. **Miscellaneous**

a) **Governing Law**. This Agreement will be governed by the laws of the State of New York without regard to the conflict of laws principles.

b) **Arbitration**. The Parties agree that any dispute arising under or concerning this Agreement, the Employee's employment by the Company or any related entity, or any compensation or benefits claimed by the Employee, shall be resolved solely in a confidential proceeding before a single arbitrator in New York, New York. The arbitration will be conducted pursuant to the then current rules of the American Arbitration Association for the resolution of employment disputes. Neither Party will bring any publicity to the arbitration, including, without limitation, the existence of a dispute, any claims or defenses raised in arbitration, or the arbitration award. However, either Party may bring an action to enforce an arbitration award in the event the other Party refuses to comply with the arbitration award within thirty (30) days following its issuance.

c) **Notices**. All notices, consents, waivers and other communications under this Agreement must be in writing and will be deemed to have been duly given when (i) delivered by hand (with written confirmation of receipt), (ii) sent by email (with written confirmation of receipt), provided that a copy is mailed by registered mail, return receipt requested, or (iii) when received by the addressee, if sent by a nationally recognized overnight delivery service (receipt requested), in each case to the appropriate addresses and facsimile numbers of the Company and Employee as set forth in the records of the Company.

d) **Section Headings**: Construction. The headings of sections in this Agreement are provided for convenience only and will not affect its construction or interpretation. All references to "Section"

or “Sections” refer to the corresponding Section or Sections of this Agreement unless otherwise specified. All words used in this Agreement will be construed to be of such gender or number as the circumstances require. Unless otherwise expressly provided, the word -including” does not limit the preceding words or terms.

e) **Amendments; Entire Agreement; Successors and Assigns**. Neither this Agreement nor any term hereof may be changed, waived, discharged or terminated orally, but only by an instrument in writing signed by the Party against which enforcement of such change, waiver, discharge or termination is sought. This Agreement embodies the entire agreement and the understanding among the Parties, superseding all prior agreements and understandings relating to the subject matter hereof, except the confidentiality and invention assignment agreement between you and the Company, any equity or equity-based or linked award agreements outstanding as of the Effective Date, and any Company employee benefit plan outstanding as of the Effective Date. Employee understands and agrees that this Agreement shall govern his employment with the Company and its related entities, and shall supersede in its entirety any other form of agreement, written or oral, relating to Employee’s employment with the Company, except for the agreements and plans set forth in the preceding sentence . If any provision of this Agreement shall be held illegal, invalid or unenforceable, in whole or in part, such provision shall be modified to the minimum extent necessary to make it legal, valid and enforceable, and the legality, validity and enforceability of the remaining provisions shall not be affected thereby. This Agreement shall be binding upon the Company’s successors and assigns.

f) **Non-Disparagement**. The Company’s officers and directors and the Employee agree that, during the Term and thereafter (including following the end of Employee’s employment for any reason) neither Party will make any statements or representations, or otherwise communicate, directly or indirectly, in writing, orally, or otherwise, or take any action that may, directly or indirectly, disparage the other Party. Nothing in this paragraph shall prohibit the Employee or the Company, or its officers or directors, from bringing an action under Section 6(b) above and providing truthful information or making truthful statements in connection with such action, or with providing truthful information or making truthful statements in connection with a subpoena or other legal process.

g) **Representations**. The Employee represents and warrants to the Company that (i) the execution, delivery and performance of this Agreement by the Employee does not and will not conflict with, breach, violate or cause a default under any contract, agreement, instrument, order, judgment or decree to which the Employee is a party or by which the Employee is bound, (ii) the Employee has had the opportunity to review the covenants contained in Section 4 with counsel, that said covenants were the result of negotiation between the parties, and that he desires to be bound by the covenants in order to obtain the compensation provided by this Agreement and (iii) upon the execution and delivery of this Agreement by the Company, this Agreement shall be the valid and binding obligation of the Employee, enforceable in accordance with its terms. The Company represents and warrants to the Employee that (i) the execution, delivery and performance of this Agreement by the Company does not and will not conflict with, breach, violate or cause a default under any its organizational documents or any contract, agreement, instrument, order, judgment or decree to which the Company is a party or by which the Company is bound, (ii) this Agreement has been duly authorized by all requisite limited liability company action on the part of the Company and (iii) upon the execution and delivery of this Agreement by the Employee, this Agreement shall be the valid and binding obligation of the Company, enforceable in accordance with its terms.

h) **Confidentiality of this Agreement**. The Employee agrees to keep confidential the terms of this Agreement. This provision does not prohibit the Employee from providing this information to the Employee’s attorneys or accountants for purposes of obtaining legal or tax advice or as required by law; provided that such persons are informed of the confidential nature of such information and the Employee shall be responsible for breaches of the confidentiality restrictions contained herein by such persons as if the Employee had breached such restrictions. The Company shall not disclose the terms of this Agreement except as necessary in the ordinary course of its business, as required by law or as required by any governmental or quasi-governmental entity or any self-regulatory organization.

i) **Cooperation**. Following termination of employment with the Company for any reason, the Employee shall cooperate with the Company, as requested by the Company, to effect a transition of

the Employee's responsibilities and to ensure that the Company is aware of all matters being handled by the Employee.

j) **Counterparts**. This Agreement may be executed in separate counterparts, each of which shall be deemed to be an original and both of which taken together shall constitute one and the same agreement.

k) **Section 409A and Taxes**. All forms of compensation paid to you by the Company, including any payments made pursuant to this Agreement, are subject to reduction (or payment by you, to the extent that additional amounts are required) to reflect applicable withholding and payroll taxes and other applicable deductions. You agree that the Company does not have a duty to design its compensation policies in a manner that minimizes your tax liabilities, and you will not make any claim against the Company related to tax liabilities arising from your compensation. The payments and benefits under this Agreement are intended, and will be construed, to be exempt from or comply with Section 409A of the Internal Revenue Code of 1986, as amended ("Section 409A"); provided, however, that nothing in this Agreement shall be construed or interpreted to transfer any liability for any tax (including a tax or penalty due as a result of a failure to comply with Section 409A) from you to the Company or to any other entity or person. Any payment to you under this Agreement that is subject to Section 409A and that is contingent on a termination of employment is contingent on a "separation from service" within the meaning of Section 409A. If, upon separation from service, you are a "specified employee" within the meaning of Section 409A, any payment under this Agreement that is subject to Section 409A and triggered by a separation from service and would otherwise be paid within six months after your separation from service will instead be paid in the seventh month following your separation from service or, if earlier, upon your death (to the extent required by Section 409A(a)(2)(B)(i)). Any taxable reimbursement due under the terms of this Agreement shall be paid no later than December 31 of the year after the year in which the expense is incurred, and all taxable reimbursements and in-kind benefits shall be provided in accordance with Treas. Reg. § 1.409A-3(i)(1)(iv). The parties agree that if necessary to avoid non-compliance with Section 409A, they will cooperate in good faith to modify the terms of this Agreement or any applicable equity award, provided, that such modification shall endeavor to maintain the economic intent of this Agreement or any such equity award.

IN WITNESS WHEREOF the parties hereto have executed this Agreement as of the date first written above.

KADMON CORPORATION, LLC

By: /s/ Tasos G. Konidaris
Tasos G. Konidaris
Chairman of the Board

Date: 11/19/2019

/s/ Harlan W. Waksal
Harlan W. Waksal, M.D.

Date: 11/19/2019

EXHIBIT 21.1

List of Subsidiaries of the Registrant

Name of Subsidiary	Jurisdiction of Organization
Kadmon Corporation, LLC	Delaware
Kadmon Pharmaceuticals, LLC	Pennsylvania

EXHIBIT 23.1

Consent of Independent Registered Public Accounting Firm

Kadmon Holdings, Inc.
New York, New York

We hereby consent to the incorporation by reference in the Registration Statements on Form S3 (No. 333-233766) and Form S8 (No.333-233770) of Kadmon Holdings, Inc. of our report dated March 5, 2020, relating to the consolidated financial statements which appear in this Form 10-K. Our report contains an explanatory paragraph regarding Kadmon Holdings, Inc.'s ability to continue as a going concern.

/s/ BDO USA, LLP
New York, New York

March 5, 2020

EXHIBIT 31.1

CERTIFICATION OF CHIEF EXECUTIVE OFFICER PURSUANT TO EXCHANGE ACT RULES 13a-14 AND 15d-14, AS ADOPTED PURSUANT TO SECTION 302 OF SARBANES-OXLEY ACT OF 2002

I, Harlan W. Waksal, certify that:

1. I have reviewed this Annual Report on Form 10-K of Kadmon Holdings, 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)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f) for the registrant and have:

   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) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

   c) 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

   d) 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.

Dated: March 5, 2020

/s/ Harlan W. Waksal
Harlan W. Waksal
President and Chief Executive Officer

EXHIBIT 31.2

CERTIFICATION OF CHIEF FINANCIAL OFFICER PURSUANT TO EXCHANGE ACT RULES 13a-14 AND 15d-14, AS ADOPTED PURSUANT TO SECTION 302 OF SARBANES-OXLEY ACT OF 2002

I, Steven Meehan, certify that:

1. I have reviewed this Annual Report on Form 10-K of Kadmon Holdings, 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)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f) for the registrant and have:

   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) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

   c) 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

   d) 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.

Dated: March 5, 2020

/s/ Steven Meehan
Steven Meehan
Executive Vice President, Chief Financial Officer

EXHIBIT 32.1

CERTIFICATION OF CHIEF EXECUTIVE OFFICER 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 Kadmon Holdings, Inc. (the "Company") for the period ended December 31, 2019 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Harlan W. Waksal, 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 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.

A signed original of this written statement has been provided to Kadmon Holdings, Inc. and will be retained by Kadmon Holdings, Inc. and furnished to the Securities and Exchange Commission or its staff upon request.

Dated: March 5, 2020

/s/ Harlan W. Waksal
Harlan W. Waksal
President and Chief Executive Officer

EXHIBIT 32.2

CERTIFICATION OF CHIEF FINANCIAL OFFICER 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 Kadmon Holdings, Inc. (the "Company") for the period ended December 31, 2019 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Steven Meehan, Executive Vice President, 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 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.

A signed original of this written statement has been provided to Kadmon Holdings, Inc. and will be retained by Kadmon Holdings, Inc. and furnished to the Securities and Exchange Commission or its staff upon request.

Dated: March 5, 2020

/s/ Steven Meehan
Steven Meehan
Executive Vice President, Chief Financial Officer